UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2023

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to

Commission File Number 001-38360

Solid Biosciences Inc.

(Exact name of Registrant as specified in its Charter)

Delaware	**90-0943402**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
500 Rutherford Avenue, Third Floor Charlestown, MA	**02129**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 337-4680

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol**	**Name of exchange on which registered**
Common Stock $0.001 par value per share	SLDB	The Nasdaq Global Select Market

Securities registered pursuant to Section 12(g) of the Act:

None
(Title of class)

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. YES ☐ NO ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. YES ☐ NO ☒

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. YES ☒ NO ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). YES ☒ NO ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
Emerging growth company	☐		

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). YES ☐ NO ☒

As of June 30, 2023, the last business day of the registrant's most recently completed second fiscal quarter, the aggregate market value of the registrant's common stock held by non-affiliates was $51.5 million, based on the last reported sale price of such stock on the Nasdaq Global Select Market as of such date.

The number of shares of the registrant's common stock outstanding as of March 5, 2024 was 37,756,877.

DOCUMENTS INCORPORATED BY REFERENCE

The registrant intends to file a definitive proxy statement pursuant to Regulation 14A within 120 days of the end of the fiscal year ended December 31, 2023. Portions of such definitive proxy statement are incorporated by reference into Part III of this Annual Report on Form 10-K.

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS AND INDUSTRY DATA

This Annual Report on Form 10-K includes forward-looking statements, which involve risks and uncertainties. These forward-looking statements can be identified by the use of forward-looking terminology, including the terms "believe," "estimate," "project," "anticipate," "expect," "seek," "predict," "aim," "continue," "possible," "intend," "may," "might," "will," "could," "would" or "should" or, in each case, their negative, or other variations or comparable terminology. These forward-looking statements include all matters that are not historical facts. They appear in a number of places throughout this Annual Report on Form 10-K. We derive many of our forward-looking statements from our operating budgets and forecasts, which are based upon many detailed assumptions. While we believe that our assumptions are reasonable, we caution that it is very difficult to predict the impact of known factors, and, of course, it is impossible for us to anticipate all factors that could affect our actual results. All forward-looking statements are based upon information available to us on the date of this Annual Report on Form 10-K.

The forward-looking statements in this Annual Report on Form 10-K include, among other things, statements about:

- the timing, progress and results of ongoing and planned preclinical studies and clinical trials for our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) or other future candidates;
- our ability to establish or maintain collaborations or strategic relationships, including our collaboration with Ultragenyx Pharmaceutical Inc., or Ultragenyx;
- our ability to obtain and maintain U.S. and foreign regulatory approval of our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) or other future candidates, and the timing and scope thereof;
- the size of the patient populations and potential market opportunity for our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) or other future candidates, if approved for commercial use;
- our manufacturing capabilities and strategy, including the scalability and commercial viability of our manufacturing methods and processes;
- our plans to develop and commercialize our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) or other future candidates, if approved;
- the pricing and reimbursement of our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) or other future candidates we may develop, if approved;
- the establishment of sales, marketing and distribution capabilities and entry into agreements with third parties to market and sell our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) or other future candidates, if approved;
- the rate and degree of market acceptance and clinical utility of our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) or other future candidates if approved;
- our plans to develop our platform technologies;
- our expectations related to our use of capital resources;
- our estimates regarding expenses, ongoing losses, future revenue, capital requirements and need for and ability to obtain additional financing;
- our intellectual property position;
- our competitive and market position;
- developments relating to our competitors and our industry;
- our ability to continue as a going concern; and
- the impact of laws, regulations and global economic developments on our business, operations, strategy and goals.

By their nature, forward-looking statements involve risks and uncertainties because they relate to events and depend on circumstances that may or may not occur in the future. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition, business and prospects may differ materially from those made in or suggested by the forward-looking statements contained in this Annual Report on Form 10-K. In addition, even if our results of operations, financial condition, business and prospects are consistent with the forward-looking statements contained in this Annual Report on Form 10-K, those results may not be indicative of results in subsequent periods.

You should read this Annual Report on Form 10-K and the documents that we have filed as exhibits to this Annual Report on Form 10-K completely and with the understanding that our actual future results may be materially different from

what we expect. We qualify all of our forward-looking statements by these cautionary statements. The forward-looking statements contained in this Annual Report on Form 10-K are made as of the date of this Annual Report on Form 10-K, and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

This Annual Report on Form 10-K includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties as well as our own estimates of potential market opportunities based on our analysis of these data, research, surveys and studies. All of the market data used in this Annual Report on Form 10-K involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such data. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. Our estimates of the potential market opportunities for our candidates include a number of key assumptions based on our industry knowledge, industry publications and third-party research, surveys and studies, which may be based on a small sample size and fail to accurately reflect market opportunities. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions.

As used in this Annual Report on Form 10-K, the terms "Solid," "the Company," "we," "us" and "our" refer to Solid Biosciences Inc., and its consolidated subsidiaries, unless the context indicates otherwise.

RISK FACTOR SUMMARY

Our business is subject to a number of risks that if realized could materially affect our business, operating results and financial condition and the trading price of our common stock could decline. These risks are discussed more fully in the "Risk Factors" section of this Annual Report on Form 10-K. These risks include the following:

- We may fail to realize the anticipated benefits of our acquisition of AavantiBio, those benefits may take longer to realize than expected, and we may encounter significant integration difficulties.
- Our stockholders may not realize a benefit from the Acquisition and the related private placement commensurate with the ownership dilution they experienced in connection with the Acquisition and the related private placement.
- We have incurred significant net losses since inception and anticipate that we will continue to incur net losses for the foreseeable future and may never achieve or maintain profitability.
- We will need additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.
- We have never generated revenue from product sales and do not expect to do so for the foreseeable future, if ever.
- Our limited operating history may make it difficult for our stockholders to evaluate the success of our business to date and to assess our future viability.
- Unfavorable global economic conditions could harm our business, financial condition or results of operations.
- Our gene transfer candidates are based on novel technology, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. To our knowledge, only a limited number of gene transfer products have been approved for commercialization in the United States and the European Union.
- Our gene transfer candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.
- One of our clinical trials has been placed on clinical hold by the FDA in the past, and we cannot guarantee that similar events will not happen in future clinical trials for our Candidates.
- We have never completed a clinical trial and may be unable to do so for any product candidate, including SGT-003 and other Candidates.
- Success in preclinical studies or early clinical trials may not be indicative of results obtained in later trials.
- Preliminary or interim data that we announce or publish from time to time may change as more data becomes available and are subject to audit and verification procedures that could result in material changes in the final data.
- We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

- We may find it difficult to enroll patients in our clinical trials, which could delay or prevent us from proceeding with clinical trials of SGT-003 or our other Candidates.
- Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize our Candidates and the approval may be for a more narrow indication than we seek.
- We face significant competition and our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may adversely affect our ability to develop, successfully market or commercialize our Candidates. Changes within the competitive landscape could lead us to alter our clinical trial strategy, baseline eligibility criteria or make other modifications to clinical trial designs.
- We may not be successful in finding strategic collaborators for continuing development of our Candidates or platform technologies, or for successfully commercializing or competing in the market for certain indications.
- We have limited gene therapy manufacturing experience and could experience production problems and delays in obtaining regulatory approval of our manufacturing processes, which could result in delays in the development or commercialization of SGT-003, SGT-501, or other current and future candidates. In addition, changes to manufacturing sites or processes, or formulations for our product candidates may result in additional cost or delay.
- We expect to utilize third parties to conduct our product manufacturing for the foreseeable future. Therefore, we are subject to the risk that these third parties may not perform satisfactorily or meet regulatory requirements.
- Our gene transfer approach utilizes capsids derived from a virus, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of gene transfer product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our Candidates.
- We heavily rely on certain in-licensed patents and other intellectual property rights in connection with our development of our Candidates and may be required to acquire or license additional patents or other intellectual property rights to continue to develop and commercialize our Candidates.
- If we are unable to obtain and maintain patent protection for our Candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our Candidates may be adversely affected.
- Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.
- The price of our common stock has been, and in the future is likely to be, volatile and fluctuate substantially, which could result in substantial losses for holders of our common stock.

PART I

Item 1. Business.

Overview

We are a life sciences company focused on advancing a portfolio of current and future gene therapy candidates, which we refer to collectively as our Candidates, including SGT-003 for the treatment of Duchenne muscular dystrophy, or Duchenne, SGT-501 for the treatment of Catecholaminergic polymorphic ventricular tachycardia, or CPVT, and additional assets for the treatment of cardiac and other diseases, at different stages of development, with varying levels of investment. We are advancing our diverse pipeline across rare neuromuscular and cardiac diseases, bringing together experts in science, technology, disease management and care. Patient-focused and founded by those directly impacted by Duchenne, our mission is to improve the daily lives of patients living with these devastating diseases.

Solid was purpose-built to advance the best science and accelerate the discovery and development of treatments that may benefit all patients with Duchenne. As we expand to bring meaningful treatments to patients living with other neuromuscular and cardiac diseases, the values and guiding principles that drive us continue. Our corporate vision is to build an innovation platform enabling the discovery and development of high-value genetic medicines for neuromuscular and cardiac diseases by integrating internal capabilities, including a vector core, use of validated animal models, optimized expression cassettes, novel capsids and regulatory expertise, and collaborations with leaders in related clinical and research fields. Our mission, which guides our operations, is to treat and change the course of neuromuscular and cardiac diseases at all stages. Underscoring this mission, our disease-focused business model is founded on the following fundamental principles:

- identify and develop meaningful therapies for patients with neuromuscular and cardiac diseases;
- bring together the leading experts in neuromuscular and cardiac diseases, science, technology, disease management and care; and
- be guided by the needs of these patients.

On December 2, 2022, we completed our acquisition of AavantiBio, Inc., or AavantiBio, a privately held gene therapy company focused on transforming the lives of patients with Friedreich's ataxia, or FA, and rare cardiomyopathies, or the Acquisition. Upon the consummation of the Acquisition, we acquired AavantiBio's gene therapy programs, AVB-202-TT for the treatment of FA and AVB-401 for the treatment of BAG3-mediated dilated cardiomyopathy, or DCM, additional assets for the treatment of cardiac diseases, platform technologies and know-how related thereto.

Our Pipeline

We are focused on developing transformative treatments to improve the lives of patients with rare neuromuscular and cardiac diseases. Our current programs are all designed to treat these diseases with gene transfer products. Gene transfer, a type of gene therapy, is designed to address diseases caused by mutated genes through the delivery of functional versions of those genes, called transgenes. The transgenes are then utilized by the body to produce proteins that are absent or not functional prior to treatment, potentially offering long-lasting clinical benefit. In addition to a transgene, our gene transfer candidates include a viral capsid or vector (a protein shell utilized as a vehicle to deliver a transgene to cells in the body) and a promoter (a specialized DNA sequence that directs cells to produce the protein in specific tissues). The capsid is modified to no longer self-replicate yet still retain its ability to introduce new genetic material directly into patients' cells. Adeno-associated virus, or AAV, capsids have been approved for use to deliver transgenes to patients, including via systemic delivery. The use of AAV capsids to deliver gene therapies has also been extensively studied by third parties in human clinical trials for multiple disease indications, and in certain of these trials AAV was delivered systemically to the patient.

Lead Neuromuscular Program

About Duchenne muscular dystrophy

Duchenne is a genetic muscle-wasting disease predominantly affecting boys, with symptoms that usually manifest between three and five years of age. Duchenne is a progressive, irreversible, and ultimately fatal disease that affects approximately one in every 3,500 to 5,000 live male births and has an estimated prevalence of 5,000 to 15,000 cases in the United States alone. Duchenne is caused by mutations in the dystrophin gene, which results in the absence or near-absence of dystrophin protein. Dystrophin protein works to strengthen muscle fibers and protect them from daily wear and tear. Dystrophin protein also serves as the cornerstone of the dystrophin glycoprotein complex, or DGC, a group of proteins that links the inner and outer components of muscle cells to ensure proper muscle function. Without functioning dystrophin and DGC, muscles suffer excessive damage from normal daily activities and are unable to regenerate, leading to the build-up of fibrotic, or scar, and fat tissue. More than 1,000 dystrophin gene mutations, which can be inherited or can occur spontaneously, have been identified in people with Duchenne. By their early teens, Duchenne patients typically lose their

ability to walk and become dependent on a wheelchair for mobility. By their 20s, patients essentially become paralyzed from the neck down and require a ventilator to breathe. Though disease severity and life expectancy vary, a patient's quality of life dramatically decreases over time, with death typically occurring by early adulthood from either cardiac or respiratory complications.

There is no cure for Duchenne, and for the vast majority of patients, there are no satisfactory symptomatic or disease-modifying treatments. Glucocorticoid treatment, the current standard-of-care, has been shown to temporarily improve muscle strength, prolong the period of ambulation and slow the progression of Duchenne. However, glucocorticoid use is associated with well-known adverse side effects, including: severe weight gain, stunted growth, weakening of bone structure and metabolic dysfunctions, among others. The most commonly used glucocorticoids include prednisone and deflazacort (EMFLAZA).

Despite recent therapeutic advances, including the FDA approval of ELEVIDYS, a gene transfer therapy for certain pediatric patients with Duchenne, Duchenne represents a significant societal and economic burden. The economic burden, estimated at $1.2 billion annually in the United States (excluding costly mortality and end-of-life care expenses), includes costs associated with hospital admissions, medication, frequent doctor visits and investment in assistive devices, as well as indirect costs related to productivity losses for the caregivers and costs due to pain, anxiety and social handicap. Of this amount, approximately 45% is represented by indirect costs. Only a small proportion of Duchenne patients are employed and many caregivers reduce their hours or stop working altogether to care for their children, who progressively require more help with everyday tasks, such as eating, dressing and using the bathroom. In some cases, patients also experience serious mental health issues that require additional support and treatment.

We are actively involved in engaging with the Duchenne patient, clinical and research communities to support advancement of therapies for patients with Duchenne. In November 2021, in collaboration with REGENXBIO Inc., we formally launched the Pathway Development Consortium, or the PDC, a multistakeholder initiative which aims to identify, develop, expand and maintain pathways to effective therapies for patients diagnosed early in life with rare diseases, including Duchenne. The PDC seeks to achieve these goals by bringing together a broad and diverse group of stakeholders from the rare disease and AAV gene therapy communities, including patients, industry, regulators, academia and payers, among others, for meaningful scientific and policy discussions.

SGT-003

Our lead neuromuscular program is directed to Duchenne. Our efforts are focused on our gene transfer candidate, SGT-003, which is designed to address the underlying genetic cause of Duchenne by delivering a synthetic transgene that produces dystrophin-like protein that is only expressed in muscles of the body, including skeletal, cardiac and respiratory muscles and is agnostic to specific mutations in the gene. Our Duchenne candidate capsid is derived from a naturally occurring, non-pathogenic virus called AAV, which was selected for its ability to efficiently enter skeletal, diaphragm and cardiac muscle tissues. The capsid is designed to carry a synthetic dystrophin transgene construct, called microdystrophin, that retains the most critical components of the full-size dystrophin gene yet is small enough to fit within AAV packaging constraints and a muscle specific promoter.

Our microdystrophin is based on three decades of development and optimization work at the University of Missouri and the University of Washington as well as other academic institutions. In preclinical studies, the laboratories of Jeffrey Chamberlain, Ph.D., from the University of Washington, and Dongsheng Duan, Ph.D., from the University of Missouri, identified a proprietary configuration of genetic components that, when administered systemically, produces functional microdystrophin protein expression that not only stabilizes muscle membranes and protects muscle against injury, but also simultaneously restores the localization of DGC to the muscle membrane, notably increasing neuronal Nitric Oxide Synthase, or nNOS, concentration. In subsequent published studies, Drs. Duan and Chamberlain demonstrated in animal models that, in comparison to earlier configurations, nNOS-restoring microdystrophins were more effective in improving muscle function and resistance to fatigue.

We believe the unique functionality of our proprietary microdystrophin has the potential to result in functional benefits including diminished muscle fatigue and protection against ischemic muscle damage, which can lead to loss of functional muscle.

The expression of our microdystrophin is regulated by a modified, synthetic muscle-specific promoter cassette called CK8, which is derived from the naturally occurring muscle creatine kinase promoter. Regulatory cassettes, such as CK8, are used to prompt gene expression specifically in muscle tissues. In comparison to other regulatory cassettes, we chose CK8 due to its small size and its ability to drive microdystrophin transgene expression in skeletal, diaphragm and cardiac muscle tissues. In our preclinical studies in small and large animal models, CK8 restricted microdystrophin transgene expression to these muscles.

SGT-003 is a clinical-stage candidate designed to preserve muscle function in Duchenne patients after a single administration. SGT-003 utilizes an updated construct, combining our proprietary microdystrophin containing nNOS with AAV-SLB101, a novel, rationally designed capsid derived from AAV9 and designed for enhanced muscle tropism, reduced liver uptake and to more selectively deliver the drug to target tissue. We believe the SGT-003 construct is meaningfully differentiated from other approved and in development gene transfer candidates and may provide differentiated clinical benefit.

Following in vitro studies in mouse and human muscle cells, AAV-SLB101 was evaluated in a head-to-head study against AAV9 with the CK8-microdystrophin construct in the dystrophin-negative mouse model of Duchenne (mdx mouse). Separate groups of mice were administered a single intravenous dose of either construct, and the biodistribution, microdystrophin protein expression, and biomarker analyses were performed at the conclusion of the study. Overall, the in vivo study mdx mouse data supported the results seen from in vitro assays and further demonstrated the potential benefits of SGT-003. The mdx mice dosed with the novel AAV-SLB101 capsid showed increased biodistribution (vector genome copies) in representative muscle tissues and increased microdystrophin expression compared to those administered the AAV9 capsid. Additionally, there were lower vector genome copies observed in the liver compared to AAV9-administered mice, with the data supporting a preferential distribution of the novel capsid towards muscle tissue and away from the liver. These data supported the proof of concept for the novel capsid microdystrophin construct in Duchenne and formed a basis for establishing and advancing the SGT-003 program.

In April 2022, we released additional preclinical data from reporter transgene studies in non-human primates, or NHPs, and both mdx and wild type mice suggesting that AAV-SLB101 may have meaningful advantages for the delivery of muscle-related gene therapies. Data from the NHP study, which used a reporter transgene in AAV-SLB101 demonstrated increased muscle tropism, decreased liver biodistribution and improved efficiency compared with AAV9. The results from the NHP study are consistent with the data from the reporter transgene studies in both mdx and wild type mouse models, which suggested improved muscle tropism and reduced liver uptake.

Based on our preclinical data, we submitted an investigational new drug application, or IND, for SGT-003 to the FDA, which was cleared in November 2023. In addition, international clinical trial application submissions are planned to initiate beginning in the first half of 2024. Our INSPIRE Duchenne trial is a Phase 1/2 first in human, open-label, multicenter trial to determine the safety and tolerability of SGT-003 in pediatric patients with Duchenne at a dose of 1E14vg/kg. We anticipate dosing the first patient in the second quarter of 2024. SGT-003 will be administered as a one-time intravenous infusion to patients in two cohorts with a minimum of three patients each, with the potential for cohort expansion. Cohort 1 will study patients aged four to less than six years of age with Duchenne. Cohort 2 will study patients aged six to less than eight years of age. We plan to evaluate long-term safety and efficacy for a total of five years following treatment.

We anticipate providing an initial safety update of the INSPIRE Duchenne trial, in mid-2024, subject to initiating patient dosing in the second quarter of 2024, and we anticipate providing initial data from the INSPIRE Duchenne trial in the second half of 2024.

The FDA has granted orphan drug designation and Fast Track designation for SGT-003 for the treatment of Duchenne.

Lead Cardiac Program

Genetic cardiac disease, or inherited cardiac conditions, is an umbrella term to describe cardiac diseases caused by mutations in one or more genes. Primary inherited arrhythmia syndromes present as abnormal cardiac arrhythmia, including life threatening ventricular arrhythmia, in the setting of structurally normal hearts and are in general genetically determined. Cardiomyopathy is a disease of the heart muscle that impairs the ability of the heart to pump blood to the rest of the body, resulting in arrhythmias, backup of blood into the lungs and other parts of the body, and ultimately heart failure. Forms of cardiomyopathy include DCM, hypertrophic and arrhythmogenic cardiomyopathy.

About CPVT

Our lead cardiac program is directed to the primary inherited arrhythmia syndrome CPVT. CPVT is a rare, serious and life-threatening disease which primarily manifests in children in the first and second decades of life, with the mean onset of CPVT symptoms being between seven and twelve years. CPVT is an inherited cardiac arrhythmia syndrome characterized by adrenergically induced polymorphic arrythmias in the presence of a normal resting sinus rhythm and a structurally normal heart. It is estimated that the prevalence of CPVT is 1 per 10,000 persons.

CPVT manifestations typically involve syncope, cardiac arrest and/or sudden cardiac death. The most common symptoms/signs include syncope (52-100%), cardiac arrest (8–48%), seizure-like events (40%), and hypoxic-ischemic encephalopathy (20%). CPVT is a significant cause of sudden death at a young age and mortality is high (up to 50%). Data from the recent Pediatric and Congenital Electrophysiology Society CPVT registry suggest that three of every four children

with CPVT present with life-threatening symptoms, which often occur during resting wakeful activities highlighting the unpredictable nature of CPVT.

To date, there are no medicines approved for the treatment of the underlying causes of CPVT and management is directed toward manifestations of the disease with the goal of reducing arrhythmias or eliminating the incidence of life-threatening arrhythmias. Current treatments for CPVT include lifestyle management changes, such as restriction of rigorous physical exercise and avoidance of emotional distress, which are very challenging in the pediatric population, as well as pharmacotherapies requiring strict compliance (e.g. beta-blockers or flecainide alone, or as combination therapy). Despite available pharmacotherapy options, the occurrence of breakthrough arrhythmia in approximately 30% of patients demonstrates that lifelong compliance is a critical problem. In addition, in some cases implantable cardioverter-defibrillators and/or left cardiac sympathetic denervation are used as treatment for symptomatic CPVT patients, but both are associated with attendant morbidity.

SGT-501

SGT-501 is a gene therapy candidate for the treatment of CPVT caused by gain of function mutation in the ryanodine receptor 2 (coded for by the RYR2 gene), which is referred to as CPVT-1, as well as loss of function mutations in the calsequestrin 2, or CASQ2, gene, which is referred to as CPVT-2. Mutations in RYR2 or CASQ2 genes disrupt cardiac calcium, or Ca^{++}, release into the cytoplasm triggering abnormal contraction and relaxation leading to arrythmias.

Our approach focuses on AAV-mediated therapeutic overexpression of CASQ2, a calcium-binding protein which, through its role in Ca^{++}regulation, is integral to excitation-contraction coupling in the heart and in regulating the rate of heart beats. CASQ2 expression via AAV is intended to provide durable and continual protection from Ca^{++} leaks seen in patients with CPVT-1 and CPVT-2.

SGT-501 uses AAV8, a muscle tropic capsid, to deliver a functional CASQ2 transgene. Collectively, overexpression of CASQ2 in CPVT patients converges on a mechanism that drives buffering of free sarcoplasmic reticulum luminal calcium such that diastolic calcium leaks through the RYR2 into the cytosol are less likely. This mechanism of action is intended to support maintenance of normal cardiac rhythm and protect against triggered activity and arrhythmias.

Two nonclinical mouse studies have demonstrated proof of concept for CASQ2 gene replacement using a recombinant AAV8 serotype capsid encoding CASQ2 to mitigate effects associated with CPVT.

We believe that the SGT-501 construct design and emerging preclinical data supports further development of SGT-501 for the treatment of CPVT. We are conducting preclinical studies of SGT-501 to identify the minimally active dose and to define our good laboratory practices toxicology study plans. We anticipate submitting an IND to the FDA for SGT-501 for the treatment of patients with CPVT-1 in early 2025. The FDA and the European Medicines Agency, or EMA, have granted orphan drug designation for SGT-501 for the treatment of CPVT.

Other Candidates

Cardiac

We are currently developing a preclinical stage candidate, AVB-401, for the treatment of BAG3-mediated DCM utilizing the AAVrh74 capsid and a muscle-specific promoter. BAG3-mediated DCM is a rare cardiac disease characterized by mutations in the BAG3 gene, which codes for the BCL-2-associated athanogene 3, or BAG3, protein. Sufficient levels of functional BAG3 are required for healthy cardiac function. BAG3 gene mutations lead to reduced BAG3 protein levels and ultimately DCM. Deletions and truncations in the BAG3 protein that result in haplo-insufficiency have been associated with the development of DCM resulting from myofilament damage, poor contraction, left ventricular dysfunction, dilatation and heart failure. Preclinical data in wild type mice indicate that the AAVrh74 capsid and muscle-specific promoter combination demonstrated enhanced cardiac biodistribution and expression and decreased liver expression relative to an AAV9 capsid and constitutive promoter combination at doses of 5E13 vg/kg or less.

We also have two cardiac pipeline gene transfer programs, SGT-601 (formerly AVB-501) for TNNT2 DCM and SGT-701 (formerly AVB-601) for RBM20 DCM, that are both currently in early preclinical development. TNNT2 DCM is a rare cardiac disease characterized by mutations in the gene that codes for cardiac troponin T protein, which helps coordinate contraction of the heart muscle. TNNT2 gene mutations lead to reduced cardiac troponin T protein levels and DCM, which ultimately leads to heart failure. RBM20 DCM is a rare inherited disease characterized by mutations in the RBM20 gene, a cardiac splicing factor that regulates alternative splicing, and codes for RNA binding motif protein 20. RBM20 mutations can cause a clinically aggressive form of DCM and is correlated with high rates of heart failure, arrhythmias, and sudden cardiac death.

Neuromuscular

We also have a neuromuscular gene transfer program for the treatment of FA, a rare, inherited, multisystem genetic disease having both neurological and cardiac manifestations caused by loss of functional frataxin protein, or FXN, due to defects in both copies of the frataxin gene. Proof of concept data in animal models suggest that gene therapy may be a viable treatment for FA. Our approach for the treatment of FA is to target both the neurological and cardiac impairments experienced by patients through dual route of administration (intravenous and intrathecal) of gene transfer therapy to more comprehensively target disease pathology. Preclinical data in cardiac-specific FXN knockout mice supported enhanced survival and cardiac function. We continue to evaluate construct design and potential preclinical testing with a focus on the neurodegenerative portions of the disease.

Platform Technologies

In addition to our gene transfer candidates, we have development programs focusing on platform technologies, including novel capsid libraries and dual gene expression, a technology that allows us to package multiple transgenes into one capsid. These programs are part of our ongoing research efforts to develop innovative technologies that we believe may hold potential to translate into meaningful treatments, and drive future pipeline expansion, which we may seek to out-license to or develop through partnerships and collaborations with other biotechnology companies.

Novel Capsid Programs

Our novel capsid programs are directed toward developing skeletal and cardiac capsid libraries using two approaches designed to enhance skeletal and/or cardiac muscle tropism: rational design and directed evolution. Preclinical data in both wild-type and disease animal models demonstrated that we have developed a library of novel capsids that have shown increased muscle tropism with concomitant decreased liver biodistribution, resulting in improved efficiency compared to AAV9.

We have developed a rationally designed library of novel AAV capsids, including SLB-101, the novel capsid used in SGT-003 for Duchenne, with the goal of improving skeletal muscle tropism. We approached this through the insertion of unique peptide sequences into traditional capsids and initially evaluated these candidates through an in vitro screening platform. The primary goal of developing this library was to generate capsids that preferentially target and transduce skeletal muscle cells, compared to traditional capsids such as AAV9. Candidate novel capsids were packaged with our microdystrophin transgene under the control of a muscle-specific promoter, such as CK8, and used to transduce muscle cells. In vitro studies performed in mouse muscle cell lines utilizing numerous novel capsid candidates showed multiple-fold increases in microdystrophin expression over AAV9. Further in vitro characterization of these capsids was performed in human Duchenne muscle cell lines. Results from these studies showed similar findings of multiple-fold increases in expression for novel capsid candidates over AAV9.

Non-specific novel capsids were packaged comprised of a bioluminescent protein (luciferase) under the control of a ubiquitous promoter in order to allow expression across a wide range of tissue types. These constructs were further evaluated in vivo in both mdx and wild-type mice to understand the potential broader applicability of these capsids for other indications. Results from this study supports preferential targeting of muscle, with increases in biodistribution and expression over AAV9 across muscle tissues and decreased biodistribution and expression compared to AAV9 in the liver, and the potential applicability to a wide variety of indications that may benefit from such a targeting profile, in addition to Duchenne. We are continuing to develop this rationally designed novel capsid library. Using both directed evolution and rational design, we are also developing a library of novel AAV capsids with the goal of enhancing cardiac tissue tropism and avoiding liver transduction.

We are using non-human primates, pigs, and mice as selection models to screen libraries at the level of RNA expression. Selection for effective transduction in cardiac tissue across different mammalian species is intended to identify capsid variants that potentially utilize conserved transduction mechanisms and may therefore be more likely to exhibit efficacy in humans.

Manufacturing and Supply

Currently, we are working to develop and optimize a transient transfection manufacturing process for producing drug product for our current and future Candidates. This process will build on industry-wide accepted practices and is expected to increase the yield, robustness and scalability of our current methods.

SGT-003 and SGT-501 are manufactured using transient transfection which requires processing steps that are more complex than those required for most chemical pharmaceuticals. We also intend to use transient transfection manufacturing for our other Candidates. We selected a manufacturing process that we believe will be scalable to support clinical and commercial production needs for SGT-003 and SGT-501. The transient transfection process was selected in order to efficiently advance SGT-003 and SGT-501 along their respective development timelines.

We currently rely on third-party manufacturers for SGT-003 and SGT-501 and plan to rely on third-party manufacturers for our Candidates. In October 2021, we announced a partnership with a cell and gene therapy-focused contract development and manufacturing organization, for the development and clinical stage manufacture of SGT-003.

We are supplying, and expect to continue to supply, our ongoing and future clinical development programs with drug produced at a cGMP compliant facility located at one of our contract manufacturing organizations. We ultimately intend to establish the capability and capacity to supply Candidates at commercial scale.

Intellectual property

Our commercial success depends in part on our ability to obtain and maintain proprietary or intellectual property protection for our pipeline programs, our platform technologies and other know-how, to operate without infringing, misappropriating or otherwise violating the intellectual property rights of others, and to prevent others from infringing, misappropriating or otherwise violating our intellectual property rights. We also rely on patents, trade secrets, know-how, confidentiality procedures and agreements, and continuing technological innovation to develop and maintain our proprietary and intellectual property position.

We own and in-license various patents, patent applications, know-how and trade secrets relating to the development and commercialization of our gene therapy candidates and platform technologies. As of February 29, 2024, our patent portfolio includes both owned and in-licensed patent families relating to our gene therapy programs and platform technologies.

For some patents, substantive prosecution of our patent applications has not yet commenced at the U.S. Patent and Trademark Office, or USPTO. We cannot predict whether such pending patent applications will result in the issuance of patents that effectively protect our candidates and our platform technologies, or if such issued patents or any of our licensor's issued patents will effectively prevent others from commercializing competitive products. In any event, patent prosecution is a lengthy process, during which the scope of the claims initially submitted for examination by the patent offices in various jurisdictions are often significantly narrowed by the time they issue, if they issue at all.

The term of individual patents depends upon the legal term for patents in the countries in which they are obtained. In most countries, including the United States, the patent term is 20 years from the earliest filing date of a non-provisional patent application. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the USPTO in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier filed patent. The term of a patent that covers a drug or biological product may also be eligible for patent term extension when FDA approval is granted, subject to certain limitations and provided statutory and regulatory requirements are met (for more information, please see "Business— Government regulation and product licensure —U.S. patent term restoration"). In the future, if and when our candidates receive approval from the FDA or foreign regulatory authorities, we expect to apply for patent term extensions on issued patents we may obtain in the future covering those products, depending upon the length of the clinical trials for each product and other factors. There can be no assurance that any of our pending patent applications will issue or that we will benefit from any patent term extension or favorable adjustment to the term of any of our patents.

As with other biotechnology and pharmaceutical companies, our ability to maintain and solidify our proprietary and intellectual property position for our candidates will depend on our success in obtaining effective patent claims and enforcing those claims if granted. However, our owned and licensed pending patent applications, and any patent applications that we may in the future file or license from third parties may not result in the issuance of patents. We also cannot predict the breadth of claims that may be allowed or enforced in our patents. Any issued patents that we may receive in the future may be challenged, invalidated or circumvented. In addition, because of the extensive time required for clinical development and regulatory review of a candidate we may develop, it is possible that, before any of our candidates can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thereby limiting protection such patent would afford the respective product and any competitive advantage such patent may provide.

In addition to patents, we rely upon unpatented trade secrets and know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary information, in part, by executing confidentiality agreements with our collaborators and scientific advisors, and non-competition, non-solicitation, confidentiality, and invention assignment agreements with our employees and consultants. We have also executed agreements requiring assignment of inventions with selected scientific advisors and collaborators. The confidentiality agreements we enter into are designed to protect our proprietary information and the agreements or clauses requiring assignment of inventions to us are designed to grant us ownership of technologies that are developed through our relationship with the respective counterparty. We cannot guarantee, however, that these agreements will afford us adequate protection of our intellectual property and proprietary information rights.

We also seek trademark protection in the United States and internationally where available and when appropriate. We currently own U.S. federal registrations for the marks SOLID, SOLID BIOSCIENCES and SOLID BIOSCIENCES logo, as well as registrations in the European Union, United Kingdom, Japan, and Hong Kong for the mark SOLID BIOSCIENCES, registrations in the European Union and United Kingdom for the marks SOLID BIOSCIENCES logo and SOLID GT. We also own pending trademark applications in the U.S. and in foreign jurisdictions for the mark AAVANTIBIO and a pending trademark application in the U.S. for the mark AAVANTIBIO logo.

Duchenne

Exclusive of our platform technologies, our Duchenne program includes three patent families with respect to microdystrophin and promoter sequences. We have filed one pending U.S. non-provisional patent application and ten pending patent applications in foreign jurisdictions, and have also exclusively licensed two issued U.S. patents, two pending U.S. non-provisional patent applications, and sixteen granted patents and eight pending patent applications in foreign jurisdictions. The issued U.S. patents are projected to expire between 2028 and 2036, excluding any patent term adjustments and any patent term extensions, and any U.S. patents that may issue from the pending U.S. non-provisional patent applications would be projected to expire between 2036 and 2042, excluding any patent term adjustments and any patent term extensions.

CPVT

Exclusive of our platform technologies, our CPVT program includes two patent families. We have exclusively licensed four issued U.S. patents, one pending U.S. non-provisional patent application, and six pending patent applications in foreign jurisdictions. The issued U.S. patents are projected to expire in 2032, excluding any patent term adjustments and any patent term extensions, and any U.S. patents that may issue from the pending U.S. non-provisional patent applications currently pending would be projected to expire in 2039, excluding any patent term adjustments and any patent term extensions.

Platform technologies

We own or license patents, patent applications and know-how related to various platform technologies. Certain of these technologies may be applicable to one or more of our current or future gene therapy candidates.

Our capsid program includes one patent family related to modified AAV capsids. We have filed one pending U.S. patent application and ten pending patent applications in foreign jurisdictions. Any U.S. patents that may issue from the pending U.S. non-provisional patent application would be projected to expire in 2040, excluding any patent term adjustments and any patent term extensions.

Strategic partnerships and collaborations/licenses

We have certain obligations under licensing agreements with third parties that include annual maintenance fees and payments that are contingent upon achieving various development, commercial and regulatory milestones. Pursuant to many of these license agreements, we are required to make milestone payments if certain development, regulatory and commercial sales milestones are achieved, and may have certain additional research funding obligations. Also, pursuant to the terms of many of these license agreements, when and if commercial sales of a licensed product commence, we must pay royalties to our licensors on net sales of the respective licensed products.

University of Washington License Agreement

In 2015, we entered into a license agreement with the University of Washington, acting through UW CoMotion, under which we obtained an exclusive, royalty-bearing, sublicensable, worldwide license under certain patent applications owned by the University of Washington relating to novel micro-dystrophins to develop, manufacture, and commercialize products for use in the treatment of Duchenne and related disease indications caused by a lack of functional dystrophin. We have the right to grant sublicenses to third parties contingent upon written approval by the University of Washington prior to executing such sublicense, which approval may not be unreasonably withheld.

In consideration for the rights granted by the agreement, we paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. We are required to reimburse the University of Washington for costs incurred in applying for, prosecuting and maintaining patents and pay up to an aggregate of approximately $1 million upon the achievement of certain milestones. There were no milestones achieved during the years ended December 31, 2023, 2022, and 2021. In October 2017, the first milestone was achieved under this agreement. The milestone payment was recorded as a research and development expense in the fourth quarter of 2017. In October 2020, the license agreement was amended such that we were required to pay the University of Washington $375 thousand in connection with the execution of the collaboration and license agreement with Ultragenyx, or the Collaboration Agreement, in October 2020. This payment was recorded as a research and development expense in the fourth quarter of 2020. The license agreement was also amended such that we are required to pay an aggregate of approximately $3.4 million upon the achievement of certain milestones. We must also pay royalties of a low single digit percentage of future sales by us and our sublicensees of products developed under the licensed patent rights. In addition, we must pay an annual maintenance fee until certain milestones are achieved, at which time a minimum annual royalty requirement will replace such maintenance fee and will apply to us and our sublicensees.

We are obligated to use our commercially reasonable efforts, consistent with sound and reasonable business practices and judgment, to commercialize the inventions covered by the licensed patent rights and to make and sell products based on that patent as soon as practicable and maximize sales thereof.

The University of Washington controls the prosecution and maintenance of the licensed patents in consultation with us and at our expense. In countries in which we have not requested prosecution or maintenance of licensed patents, the University of Washington may prosecute and maintain such licensed patents at its own cost. We have the first right to enforce such licensed patents at our expense. However, we may not enter into any settlement in any manner relating to the licensed patents without the University of Washington's prior written consent.

The license agreement remains in effect until the expiration of the last-to-expire patent licensed under the agreement. We may terminate the agreement at any time upon providing sixty days' written notice to the University of Washington. The University of Washington may terminate the agreement upon our uncured, material breach of the agreement or if we enter into an insolvency-related event.

The University of Missouri License Agreement

In 2015, we entered into a license agreement with the Curators of the University of Missouri, or the University of Missouri, a public corporation of Missouri, under which we obtained an exclusive, royalty-bearing, sublicensable, worldwide license under certain patents and patent applications owned by the University of Missouri relating to a novel synthetic microdystrophin gene to make, sell and distribute products for use in the treatment of Duchenne and related disease indications resulting from a lack of functional dystrophin.

In consideration for the rights granted by the agreement, we paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. We were required to reimburse the University of Missouri for costs incurred in applying for, prosecuting and maintaining the licensed patents and pay up to an aggregate of approximately $1 million upon the achievement of certain milestones for each product developed based on the licensed patents.

Under the agreement, in the event we grant a sublicense to another party, we are required to pay the University of Missouri a percentage of the consideration received. The license agreement was amended such that we were required to pay, and did pay, the University of Missouri $0.8 million in February 2021 and $1.3 million in February 2022 as a result of the execution of the Collaboration Agreement with Ultragenyx in October 2020. These amounts were recorded as a research and development expense in the fourth quarter of 2020. The license agreement was also amended such that we are required to make aggregate milestone payments of approximately $1.9 million upon the achievement of certain milestones.

There were no milestones achieved during the years ended December 31, 2023, 2022, and 2021. We must pay a royalty of a low single digit percentage of future sales or by its sublicensees of products developed using the licensed patents. In addition, we must pay an annual maintenance fee until certain milestones are achieved, after which time a minimum annual royalty will replace such maintenance fee.

Under the agreement, we granted the University of Missouri a non-exclusive, royalty-free, irrevocable, paid-up license, with the right to grant sublicenses to non-profit, academic, educational or governmental institutions, to practice and use improvements made by us using the licensed patent rights, solely for non-commercial research purposes.

We are obligated to use our reasonable best efforts to introduce products based on the licensed patent rights into the commercial market as soon as possible, consistent with sound and reasonable business practices and judgment, and thereafter to keep such products reasonably available to the public.

The University of Missouri controls the prosecution and maintenance of the licensed patents in consultation with us and at our expense. In countries in which we have not requested prosecution or maintenance of licensed patents, the University of Missouri may prosecute and maintain such licensed patents at its own cost. We have the first right to enforce such licensed patents at our expense. However, any settlement, consent judgment or other voluntary disposition of litigation that materially limits the scope, validity or enforceability of the licensed patent or admits fault or wrongdoing on the part of the University of Missouri must be pre-approved in writing by the University of Missouri. The license agreement remains in effect until the expiration of the last-to-expire patent or the abandonment of the last to be abandoned patent application licensed under the agreement. The University of Missouri may terminate the agreement, or render the license granted thereunder non-exclusive, in individual countries if we and our sublicensees fail to achieve certain milestones. We may terminate the license agreement at any time upon providing six months' written notice to the University of Missouri and paying a termination fee. Each of the University of Missouri and we may also terminate the agreement for an uncured default or breach of the agreement by the other party. Our ability to cure such breach only applies to the first two notices of such breach provided by the University of Missouri, and thereafter, the University of Missouri may terminate the agreement for our default or breach of the agreement upon thirty days' written notice without an opportunity to cure such default or breach.

University of Florida License Agreements

We, and our subsidiary AavantiBio, have entered into several license agreements with the University of Florida Research Foundation, Inc., or UFRF. Broadly, the agreements relate to FA and our early stage cardiac candidates, including AVB-401, SGT-601 and SGT-701. Under each agreement we obtained an exclusive, royalty-bearing, sublicensable, world-wide license to certain patents and patent applications and a royalty-bearing non-exclusive license under the know-how, to make, have made, use, see, have sold, import and export licensed products. UFRF retains the right to practice the patent rights and know-how for internal non-commercial research, including research sponsored by commercial entities, and educational purposes.

In consideration for the rights granted under each agreement, AavantiBio paid a one-time non-refundable license fee. In connection with each agreement, we are required to pay an annual license maintenance fee until the first commercial sale of a licensed product after which time a minimum annual royalty will replace such maintenance fees. Under each agreement, we are required to reimburse UFRF for costs incurred in applying for, prosecuting and maintaining patents, pay up to an aggregate of approximately $2.9 million upon the achievement of certain intellectual property, clinical and regulatory milestones for each licensed product under the agreement and pay a low, single digit royalty on annual net sales by us and our sublicensees of licensed products on a licensed-product-by-licensed product basis. For any licensed product covered by both of these agreements, we are only obligated to make one payment for each milestone achieved and royalty payment due. Under each agreement, in the event we grant a sublicense to another party, we are required to pay UFRF a percentage of the consideration received.

Under each agreement, we have the right to grant sublicenses to third parties through multiple tiers, to the extent we are in compliance with our diligence obligations under the agreement and that sublicensee is subject to the terms of such agreement.

Under each agreement, we are obligated to use commercially reasonable efforts to develop and commercialize products covered by the licensed patent rights or know-how and to achieve certain regulatory and commercialization milestones within estimated time periods.

Under each agreement, UFRF controls the prosecution and maintenance of the licensed patents in consultation with us and at our expense. In countries in which we have not requested prosecution or maintenance of licensed patents in a particular country or jurisdiction, the license granted to such patent rights will terminate in such country or jurisdiction. We have the first right to enforce such licensed patents at our expense.

Each of the agreements terminates on a licensed product-by-licensed product basis on the later of: (i) expiration of the patent rights covering such licensed product or (ii) ten (10) years from the first commercial sale of such licensed product. After five years, we may terminate an agreement for any reason giving advance written notice and reason for termination. UFRF may terminate an agreement for our uncured default or breach of the agreement. UFRF may immediately terminate an agreement if we bring or assist others in bringing a patent challenge against the licensed patent rights. If UFRF sends us a written demand to terminate a sublicense agreement due to such sublicensee bringing or assisting a patent challenge, UFRF may terminate such agreement if we do not terminate the license with such sublicensee.

Maugeri License Agreement

In June 2023 we entered into a license agreement, or the Maugeri License Agreement, with ICS Maugeri S.p.A. SB, or Maugeri, to focus on our development and commercialization of cardiac-related products based on Maugeri's inventions. Pursuant to the Maugeri License Agreement, Maugeri granted us an exclusive worldwide sublicensable license in certain Maugeri patent rights, including existing patent rights, and those in any improvements or know-how made in performance of

the Maugeri License Agreement, and a non-exclusive worldwide sublicensable license in certain Maugeri know-how, including existing know-how, and on any improvement thereto, in each case, subject to certain conditions, that is necessary or reasonably useful to develop licensed products under the terms of the Maugeri License Agreement. We will conduct certain activities agreed to by the parties with respect to the research and development of licensed products. A condition precedent to the effectiveness of the Maugeri License Agreement was regulatory review in Italy, which was completed in the third quarter of 2023 and, upon the completion of the condition precedent, the Maugeri License Agreement became effective.

We paid Maugeri an upfront license fee of €1,500, which was recorded as research and development expense during the second quarter of 2023. Additionally, we agreed to cumulative developmental, regulatory, and commercial milestone payments of up to €15,000, cumulative sales milestone payments of up to €15,000, upon achievement of specified milestone events, and tiered royalties on worldwide net sales in the low-to-mid-single-digits.

The Maugeri License Agreement continues until the latest expiry of (i) the last valid claim (as defined in the Maugeri License Agreement), (ii) regulatory exclusivity, and (iii) all payment obligations. Either party may terminate the Maugeri License Agreement for the other party's uncured material breach. We may also terminate the Maugeri License Agreement in our sole discretion upon 60 days' prior written notice to Maugeri and payment of a fee.

Ultragenyx Collaboration Agreement

On October 22, 2020, or the Effective Date, we entered into a collaboration and license agreement with Ultragenyx, to focus on the development and commercialization of new gene therapies for Duchenne. We granted Ultragenyx an exclusive worldwide license for any pharmaceutical product that expresses our proprietary microdystrophin construct from AAV8 and variants thereof in clade E for the treatment of Duchenne and other diseases resulting from the lack of functional dystrophin. We retain exclusive rights to all other uses of our microdystrophin proteins, including under our SGT-003 program.

We have conducted and may conduct in the future certain activities agreed to by the parties with respect to the development of licensed products. Ultragenyx is obligated to reimburse us for personnel and out-of-pocket costs that we incur in conducting such development activities. Otherwise, Ultragenyx has decision-making authority with respect to the development, manufacturing and commercialization of licensed products. In connection with the execution of the Collaboration Agreement, we also entered into a stock purchase agreement and an investor agreement with Ultragenyx, pursuant to which we issued and sold 521,719 shares of our common stock to Ultragenyx at a price of $76.669 per share for an aggregate purchase price of approximately $40.0 million. The shares purchased by Ultragenyx were subject to a lock-up period until the earliest to occur of (i) 18 months from the closing date, (ii) the termination of the Collaboration Agreement or (iii) other specified events. Pursuant to the terms of the investor agreement, Ultragenyx agreed that, so long as it holds at least 10% of our outstanding common stock, the shares will be subject to a voting agreement, such that until the earliest to occur of certain specified events, and subject to specified conditions, Ultragenyx will, and will cause its permitted transferees to, vote in accordance with the recommendation of our Board of Directors with respect to specified matters.

Ultragenyx also agreed to pay up to $255.0 million in cumulative milestone payments per product upon achievement of specified milestone events, and tiered royalties on worldwide net sales at low double digit to mid-teens percentages. Upon achievement of proof-of-concept, we have the right to opt-in to co-fund collaboration programs in return for participation in a profit share or increased royalty payments. None of the payments under the Collaboration Agreement are refundable.

For each licensed product for which Ultragenyx decides to initiate a registrational trial in humans, we have the option to fund 30% of the development costs in the United States and European Union for such licensed product and forgo the development milestones and regulatory milestones, or the Development Option, and receive tiered royalties on a licensed product-by-licensed product and country-by-country basis ranging from a mid-teens percentage to a low twenties percentage based on Ultragenyx's, and any of its affiliates' and sublicensees' annual worldwide net sales of each such licensed product.

For each licensed product for which we exercise the Development Option, we may also elect to share 30% of the net income and net losses on net sales of such licensed product in the United States and European Union, or the Income Share Option. For licensed products for which we have exercised the Income Share Option, we will not be entitled to milestone payments and Ultragenyx will pay us tiered royalties on a licensed product-by-licensed product and country-by-country basis ranging from a mid-teens percentage to a low twenties percentage based on Ultragenyx's, and any of its affiliates' and sublicensees', annual net sales of each such licensed product outside of the United States and European Union.

We and Ultragenyx established a Joint Steering Committee, or the JSC. The JSC will, among other responsibilities, review and oversee certain development activities performed under the Collaboration Agreement, including reviewing the development plan and budget for the development activities to be performed by us.

The term of the Collaboration Agreement began on the Effective Date and expires upon the expiration of all payment obligations from Ultragenyx to us under the Collaboration Agreement. Ultragenyx also has the ability to terminate for convenience with prior written notice to us, and either party may terminate for an uncured material breach.

As described in Note 3, we entered into the Collaboration Agreement with Ultragenyx for the research, development and commercialization of other pharmaceutical products that express our MD5 nNOS binding domain form of microdystrophin protein. During the year ended December 31, 2023, we recognized no revenue associated with the Collaboration Agreement. As of December 31, 2023, there was no deferred revenue related to the Collaboration Agreement. There are no amounts due from Ultragenyx as of December 31, 2023.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. This is also true in treatments of neuromuscular diseases, such as Duchenne, cardiac diseases as well as in gene therapy. While we believe that our focus, strength of team, expertise in gene therapy, scientific knowledge and intellectual property provide us with competitive advantages, we face competition from several different sources, including large and small biopharmaceutical companies, academic research institutions, government agencies and public and private research institutions. Not only must we compete with other companies that are focused on gene transfer technology, but any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, clinical trials, regulatory approvals and product marketing than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, enrolling patients in clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We are aware of a number of companies and research institutions developing gene transfer programs progressing in Duchenne. For example, in June 2023, Sarepta Therapeutics, Inc., or Sarepta, announced that it had received accelerated approval for its gene therapy candidate ELEVIDYS for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne. In December 2023, Sarepta announced that it submitted a supplemental biologics license application, or BLA, to broaden the approved indication for ELEVIDYS to all patients (all ages and ambulation status) with Duchenne, and on February 16, 2024 Sarepta announced that the FDA accepted the supplemental BLA for priority review and set a PDUFA date of June 21, 2024. We are also aware of several companies and research institutions conducting clinical trials of product candidates focused on systemic gene transfers for Duchenne, including Pfizer Inc. with a product candidate currently in Phase 3 clinical development, Genethon with a product candidate currently being evaluated in a Phase 1/2/3 clinical trial, and REGENXBIO Inc. with a product candidate in Phase 1/2 clinical development.

We are also aware of several companies and research institutions conducting clinicals trials in small molecule product candidates focused on CPVT, including Armgo Pharmaceuticals, Inc. with an orally administered Rycal in a Phase 2 clinical trial and Cardurion Pharmaceuticals, Inc. with an orally administered CAMKII-delta inhibitor candidate in a Phase 2 clinical trial.

Government regulation and product licensure

U.S. government regulation and product licensure

In the United States, biologic products including gene therapy products, such as our lead candidates, are licensed for marketing by the FDA under the Public Health Service Act, or PHS Act, and regulated by the FDA under the Federal Food, Drug, and Cosmetic Act, or FD&C Act, as well as by other federal, state and local statutes and regulations. Both the FD&C Act and the PHS Act and their corresponding rules and regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, reporting, advertising and other promotional practices involving biologic products. FDA approval must be obtained before conducting human clinical testing of biologic products. FDA must license a biologic product before it may be marketed within the United States.

U.S. biologic products development process

A company, institution, or organization which takes responsibility for the initiation and management of a clinical development program for such products is referred to as a sponsor. A sponsor seeking approval to market and distribute a new biological product in the United States must typically secure the following:

- completion of preclinical laboratory tests and in vivo studies according to the FDA's good laboratory practices, or GLP, requirements and applicable requirements for the humane use of laboratory animals or other applicable regulations;

- design of a clinical protocol and submission to the FDA of an application for an IND, which allows human clinical trials to begin unless the FDA objects within 30 days;
- approval by an institutional review board, or IRB, reviewing each clinical site before each clinical trial may be initiated;
- approval by an institutional biosafety committee, or IBC, assessing the safety of the clinical research and identifying any potential risk to public health or the environment;
- performance of adequate and well controlled human clinical trials according to the FDA's regulations commonly referred to as good clinical practices, or GCPs, and any additional requirements for the protection of human research subjects and their health information, to establish the safety, potency and purity of the proposed biologic product for each of its intended uses;
- preparation and submission to the FDA of a BLA for marketing approval that includes substantive evidence of safety, purity and potency from results of preclinical testing and clinical trials, and detailed information about the chemistry, manufacturing and controls, or CMC, for the product, reports of the outcomes and full data sets of the clinical trials and proposed labeling and packaging for the product;
- review of the product candidate by an FDA advisory committee, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biologic product candidate is produced to assess compliance with cGMP requirements and to assure that the facilities, methods and controls are adequate to preserve the biologic product candidate's identity, safety, strength, quality and purity;
- potential FDA audit of the nonclinical and clinical trial sites that generated the data in support of the BLA;
- payment of user fees;
- FDA review and licensure of the BLA; and
- compliance with any post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and the potential requirement to conduct post-approval studies.

Preclinical Studies and Investigational New Drug Application

Before testing any biologic product candidate in humans, including a gene therapy product candidate, the product candidate must undergo preclinical testing. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as in vivo studies to assess the potential safety and activity of the product candidate and to establish a rationale for therapeutic use. These studies are generally referred to as IND-enabling studies. The conduct of certain nonclinical studies must comply with federal regulations and requirements, including GLPs and the U.S. Department of Agriculture's Animal Welfare Act, if applicable.

The clinical trial sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. An IND is an exemption from the FD&C Act that allows an unapproved product to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA authorization to administer an investigational product to humans. Such authorization must be secured prior to interstate shipment and administration of any product candidate that is not the subject of an approved BLA. In addition to reviewing an IND to assure the safety and rights of patients, the FDA also focuses on any CMC issues and the quality of the investigation. Some preclinical tests may continue even after the IND is submitted.

The IND becomes effective 30 days after receipt by the FDA, unless the FDA notifies the sponsor of deficiencies that require correction before human studies can begin. The sponsor cannot initiate studies until the FDA notifies the sponsor that the submitted corrections are satisfactory. The FDA may also place the clinical trial on a full clinical hold or partial clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin.

In addition, following the clearance of an IND, the FDA may impose a full or partial clinical hold at any time during clinical trials. A partial clinical hold is a delay or suspension of only part of the clinical work requested under the IND (e.g., a specific protocol or part of a protocol is not allowed to proceed; however, other protocols or parts of the protocol are allowed to proceed under the IND). If the FDA requires that progress to the next study is contingent on (i) FDA review of additional data and (ii) subsequent specific permission for the study to proceed, this represents a partial clinical hold.

Human clinical trials under an IND

Clinical trials involve the administration of the biologic product candidate to healthy volunteers or subjects under the supervision of qualified investigators, generally physicians not employed by, or under the control of, the trial sponsor. Clinical trials are conducted under written study protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical trial will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. Clinical trials must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research subjects provide informed consent.

Further, each clinical trial must be reviewed and approved by an IRB at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative, reviews and approves the study protocol and must monitor the clinical trial until completed.

Additionally, some trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data safety monitoring board or committee, or DSMB, or a data safety monitoring committee, or DMC. This group provides authorization as to whether or not a trial may move forward at designated check points based on access that only the group maintains to available data from the study. Clinical trials involving recombinant DNA also must be reviewed by an IBC a local institutional committee that reviews and oversees basic and clinical research and utilizes recombinant DNA at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- *Phase 1.* The investigational biologic product is initially introduced into a small group of healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early understanding of its effectiveness. In the case of some product candidates for severe or life-threatening diseases, especially when the product candidate may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients. Phase 1 clinical trials of gene therapies are typically conducted in patients rather than healthy volunteers.
- *Phase 2*. The biologic product candidate is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.
- *Phase 3*. Phase 3 clinical trials are commonly referred to as "pivotal" studies, which typically denotes a study that presents the data that the FDA or other relevant regulatory agency will use to determine whether or not to approve a biologic product. In Phase 3 clinical trials, the investigational biologic product is administered to an expanded patient population, generally at multiple geographically dispersed clinical trial sites in adequate and well controlled clinical trials to generate sufficient data to statistically confirm the potency and safety of the product for approval. These clinical trials are intended to establish the overall risk/benefit ratio of the product candidate and provide an adequate basis for product labeling.

Post-approval clinical trials, sometimes also referred to as post-marketing clinical trials, may be conducted after initial approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up.

A clinical trial may combine the elements of more than one phase and the FDA often requires more than one Phase 3 trial to support marketing approval of a product candidate. A company's designation of a clinical trial as being of a particular phase is not necessarily indicative that the study will be sufficient to satisfy the FDA requirements of that phase because this determination cannot be made until the protocol and data have been submitted to and reviewed by the FDA. Generally, pivotal trials are Phase 3 trials, but they may be Phase 2 trials if the design provides a well-controlled and reliable assessment of clinical benefit, particularly in an area of unmet medical need.

In December 2022, with the passage of Food and Drug Omnibus Reform Act, or FDORA, Congress required sponsors to develop and submit a diversity action plan for each Phase 3 clinical trial or any other "pivotal study" of a new biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. Specifically, action plans must include the sponsor's goals for enrollment, the underlying rationale

for those goals, and an explanation of how the sponsor intends to meet them. In January 2024, the FDA issued draft guidance setting out its policies for the collection of race and ethnicity data in clinical trials.

In June 2023, the FDA issued draft guidance with updated recommendations for GCPs aimed at modernizing the design and conduct of clinical trials. The updates are intended to help pave the way for more efficient clinical trials to facilitate the development of medical products. The draft guidance is adopted from the International Council for Harmonisation's recently updated E6(R3) draft guideline that was developed to enable the incorporation of rapidly developing technological and methodological innovations into the clinical trial enterprise. In addition, the FDA issued draft guidance outlining recommendations for the implementation of decentralized clinical trials.

The FDA or the sponsor or its DSMB/DMC may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the biologic product candidate has been associated with unexpected serious harm to patients.

Finally, sponsors of certain clinical trials are required to register and disclose certain clinical trial information on a public registry (clinicaltrials.gov) maintained by the NIH. In particular, information related to the product, patient population, phase of investigation, study sites and investigators and other aspects of the clinical trial is made public as part of the registration of the clinical trial. The NIH's Final Rule on registration and reporting requirements for clinical trials became effective in 2017. Although the FDA has historically not enforced these reporting requirements due to the Department of Health and Human Services', or HHS's, long delay in issuing final implementing regulations, the FDA has issued several pre-notices for voluntary corrective action and several notices of non-compliance during the past two years. While these notices of non-compliance did not result in civil monetary penalties, the failure to submit clinical trial information to clinicaltrials.gov, as required, is a prohibited act under the FD&C Act with violations subject to potential civil monetary penalties of up to $10,000 for each day the violation continues.

Interactions with the FDA during the clinical development program

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data and clinical trial investigators. Written IND safety reports must be promptly submitted to the FDA, the IRB and the investigators for serious and unexpected adverse events, any findings from other trials, in vivo laboratory tests or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA.

In addition, sponsors are given opportunities to meet with the FDA at certain points in the clinical development program. Specifically, sponsors may meet with the FDA prior to the submission of an IND, or pre-IND application meeting, at the end of a Phase 2 clinical trial, or EOP2 meeting, and before a BLA is submitted, or pre-BLA meeting. Meetings at other times may also be requested. There are five types of meetings that occur between sponsors and the FDA. Type A meetings are those that are necessary for an otherwise stalled product development program to proceed or to address an important safety issue. Type B meetings include pre-IND application and pre-BLA meetings, as well as Type B EOP2 meetings. A Type C meeting is any meeting other than a Type A or Type B meeting regarding the development and review of a product. A Type D meeting is focused on a narrow set of issues (and should be limited to no more than two focused topics) and should not require input from more than three disciplines or divisions. Finally, INTERACT meetings are intended for novel products and development programs that present unique challenges in the early development of an investigational product.

Clinical studies outside the United States in support of FDA approval

In connection with our clinical development program, we may conduct trials at sites outside the United States. When a foreign clinical study is conducted under an IND, all IND requirements must be met unless waived. When a foreign clinical study is not conducted under an IND, the sponsor must ensure that the study complies with certain regulatory requirements of the FDA in order to use the study as support for an IND or application for marketing approval. Specifically, the studies must be conducted in accordance with GCP requirements, including undergoing review and receiving approval by an independent ethics committee and seeking and receiving informed consent from subjects. GCP requirements encompass both ethical and data integrity standards for clinical studies. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical studies, as well as the quality and integrity of the resulting data. They further help ensure that non-IND foreign studies are conducted in a manner comparable to that required for IND studies.

The acceptance by the FDA of study data from clinical trials conducted outside the United States in support of U.S. approval may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the U.S., the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations; and (iii) the data may be considered valid without the need for an on-site inspection by the FDA, or if the FDA considers such inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means.

In addition, even where the foreign study data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the study is well-designed and well-conducted in accordance with GCP requirements and the FDA is able to validate the data from the study through an onsite inspection if deemed necessary. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials are subject to the applicable local laws of the foreign jurisdictions where the trials are conducted.

Expanded Access to an Investigational Drug for Treatment Use

Expanded access, sometimes called "compassionate use," is the use of investigational new drug products outside of clinical trials to treat patients with serious or immediately life-threatening diseases or conditions when there are no comparable or satisfactory alternative treatment options. The rules and regulations related to expanded access are intended to improve access to investigational drugs for patients who may benefit from investigational therapies. FDA regulations allow access to investigational drugs under an IND by the company or the treating physician for treatment purposes on a case-by-case basis for: individual patients (single-patient IND applications for treatment in emergency settings and non-emergency settings); intermediate-size patient populations; and larger populations for use of the drug under a treatment protocol or Treatment IND Application.

When considering an IND application for expanded access to an investigational product with the purpose of treating a patient or a group of patients, the sponsor and treating physicians or investigators will determine suitability when all of the following criteria apply: patient(s) have a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; the potential patient benefit justifies the potential risks of the treatment and the potential risks are not unreasonable in the context or condition to be treated; and the expanded use of the investigational drug for the requested treatment will not interfere with initiation, conduct, or completion of clinical investigations that could support marketing approval of the product or otherwise compromise the potential development of the product.

There is no obligation for a sponsor to make its investigational products available for expanded access; however, as required by the 21st Century Cures Act, or the Cures Act, passed in 2016, if a sponsor has a policy regarding how it responds to expanded access requests, it must make that policy publicly available. Sponsors are required to make such policies publicly available upon the earlier of initiation of a Phase 2 or Phase 3 trial; or 15 days after the drug or biologic receives designation as a breakthrough therapy, fast track product, or regenerative medicine advanced therapy.

In addition, on May 30, 2018, the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a drug manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act, but the manufacturer must develop an internal policy and respond to patient requests according to that policy.

Special regulations and guidance governing gene therapy products

The FDA has defined a gene therapy product as one that mediates its effects by transcription and/or translation of transferred genetic material and/or by integrating into the host genome and which is administered as nucleic acids, viruses, or genetically engineered microorganisms. The products may be used to modify cells in vivo or transferred to cells ex vivo prior to administration to the recipient.

Within the FDA, the Center for Biologics Evaluation and Research, or CBER, regulates gene therapy products. CBER's Office of Therapeutic Products is responsible for the review of gene therapy and related products, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. The NIH also advises the FDA on gene therapy issues and other issues related to emerging technologies. The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols.

If a gene therapy trial is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, prior to the submission of an IND to the FDA, a protocol and related documents must be submitted to, and the study registered with, the NIH Office of Biotechnology Activities, pursuant to the NIH Guidelines for Research Involving

Recombinant DNA Molecules, or NIH Guidelines. Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for research involving recombinant DNA. However, many companies and other institutions, not otherwise subject to the NIH Guidelines, voluntarily follow them.

The FDA has issued various guidance documents regarding gene therapies, including final guidance documents released in January 2020 relating to chemistry, manufacturing and controls information for gene therapy INDs, gene therapies for rare diseases and gene therapies for retinal disorders, a final guidance in October 2022 for Human Gene Therapy for Neurodegenerative Diseases, as well as a draft guidance in July 2023 on comparability requirements for manufacturing changes in gene therapy products. In December 2023, a draft guidance on potency assurance for cellular and gene therapy products was released. Although the FDA has indicated that these and other guidance documents it previously issued are not legally binding, we believe that our compliance with them is likely necessary to gain approval for any gene therapy product candidate we may develop. The guidance documents provide additional factors that the FDA will consider at each of the above stages of development and relate to, among other things, the proper preclinical assessment of gene therapies; the chemistry, manufacturing, and control information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, for AAV capsids specifically, the FDA typically recommends that sponsors continue to monitor participants for potential gene therapy-related adverse events for up to a 5-year period. Other types of gene therapy or gene editing products may require longer follow up, potentially up to a maximum 15-year period.

Finally, for a gene therapy product and where applicable, the FDA also will not approve the product if the manufacturer is not in compliance with good tissue practices, or GTP. These standards are found in FDA regulations and guidance that govern the methods used in, and the facilities and controls used for, the manufacture of human cells, tissues, and cellular and tissue-based products, or HCT/Ps, which are human cells or tissue intended for implantation, transplant, infusion, or transfer into a human recipient. The primary intent of the GTP requirements is to ensure that T-cell and tissue-based products are manufactured in a manner designed to prevent the introduction, transmission, and spread of communicable disease. FDA regulations also require tissue establishments to register and list their HCT/Ps with the FDA and, when applicable, to evaluate donors through screening and testing.

Pediatric studies

Under the Pediatric Research Equity Act of 2003, or PREA, a BLA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. Sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the sponsor plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The sponsor, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other, and agree upon a final plan. The FDA or the sponsor may request an amendment to the plan at any time.

For products intended to treat a serious or life-threatening disease or condition, the FDA must, upon the request of a sponsor, meet to discuss preparation of the initial pediatric study plan or to discuss deferral or waiver of pediatric assessments. In addition, the FDA will meet early in the development process to discuss pediatric study plans with sponsors and FDA must meet with sponsors by no later than the end-of-phase 1 meeting for serious or life-threatening diseases and by no later than 90 days after the FDA's receipt of the study plan.

The FDA may, on its own initiative or at the request of the sponsor, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. The FDA is required to send a PREA Non-Compliance letter to sponsors who have failed to submit their pediatric assessments required under PREA, have failed to seek or obtain a deferral or deferral extension or have failed to request approval for a required pediatric formulation. It further requires the FDA to publicly post the PREA Non-Compliance letter and sponsor's response. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation, although FDA has recently taken steps to limit what it considers abuse of this statutory exemption. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in the Food and Drug Administration Safety and Innovation Act, or FDASIA. The FDA also maintains a list of diseases that are exempt from the requirements PREA, due to low prevalence of disease in the pediatric population. In May 2023, the FDA issued new draft guidance that further describes the pediatric study requirements under the PREA.

Compliance with cGMP requirements

Manufacturers of biologics must comply with applicable cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Manufacturers and others involved in the manufacture and

distribution of such products also must register their establishments with the FDA and certain state agencies. Both domestic and foreign manufacturing establishments must register and provide additional information to the FDA upon their initial participation in the manufacturing process. Establishments may be subject to periodic, unannounced inspections by government authorities to ensure compliance with cGMP requirements and other laws. Discovery of problems may result in a government entity placing restrictions on a product, manufacturer or holder of an approved BLA, and may extend to requiring withdrawal of the product from the market. The PREVENT Pandemics Act, which was enacted in December 2022, clarifies that foreign drug manufacturing establishments are subject to registration and listing requirements even if a biologic undergoes further manufacture, preparation, propagation, compounding, or processing at a separate establishment outside the United States prior to being imported or offered for import into the United States.

Concurrent with clinical trials, companies usually complete additional preclinical studies and must also develop additional information about the physical characteristics of the biologic product candidate as well as finalize a process for manufacturing the product candidate in commercial quantities in accordance with cGMP requirements. To help reduce the risk of the introduction of adventitious agents or of causing other adverse events with the use of biologic products, the PHS Act emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other requirements, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biologic product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the biologic product candidate does not undergo unacceptable deterioration over its shelf life.

Submission and filing of a BLA

After the completion of clinical trials of a biologic product, FDA licensure of a BLA must be obtained before commercial marketing of the biologic product. The BLA must include results of product development, laboratory and animal studies, human studies, information on the manufacture and composition of the product, proposed labeling and other relevant information. In addition, under the PREA, a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the biologic product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a significant user fee. Under federal law, the submission of most BLAs is subject to an application user fee, which for federal fiscal year 2024 is approximately $4.05 million for an application requiring clinical data. The sponsor of an approved BLA is also subject to an annual program fee, which for federal fiscal year 2024 is currently $416,734 per eligible prescription product. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on BLAs for product candidates designated as orphan drugs, unless the product candidate also includes a non-orphan indication.

The FDA reviews a BLA within 60 days of submission to determine if it is substantially complete before the agency accepts it for filing, and it must so notify the sponsor of that determination within the 60 days. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In the event that FDA determines that an application does not satisfy this standard, it will issue a Refuse to File, or RTF, determination to the sponsor. The BLA may be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the BLA.

With filing of the application, the FDA reviews the BLA to determine, among other things, whether the proposed product is safe and potent, or effective, for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biologic products or biologic products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

During the biologic product approval process, the FDA also will determine whether a REMS, is necessary to assure the safe use of the biologic product. REMS use risk minimization strategies beyond the professional labeling to ensure that the benefits of the product outweigh the potential risks. To determine whether a REMS is needed, the FDA will consider the size of the population likely to use the product, seriousness of the disease, expected benefit of the product, expected duration of treatment, seriousness of known or potential adverse events and whether the product is a new molecular entity. A REMS could include medication guides, physician communication plans and elements to assure safe use, such as restricted

distribution methods, patient registries and other risk minimization tools. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

In connection with its review of a BLA, the FDA will inspect the facilities at which the product candidate is manufactured. The FDA will not approve the product candidate unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND study requirements and GCP requirements to ensure the integrity of the clinical data. cGMP, GLP and GCP compliance requires significant expenditure of time, money and effort in the areas of training, recordkeeping, production and quality control.

With passage of FDORA, Congress clarified FDA's authority to conduct inspections by expressly permitting inspection of facilities involved in the preparation, conduct, or analysis of clinical and non-clinical studies submitted to FDA as well as other persons holding study records or involved in the study process.

Decisions on a BLA

After evaluating the application and all related information, including the advisory committee recommendations, if any, and inspection reports of manufacturing facilities and clinical trial sites, the FDA will issue either a Complete Response Letter, or CRL, or an approval letter. To reach this determination, the FDA must determine that the expected benefits of the proposed product outweigh its potential risks to patients. This "benefit-risk" assessment is informed by the extensive body of evidence about the product in the BLA.

A CRL indicates that the review cycle of the application is complete, and the application will not be approved in its present form. A CRL generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. A CRL generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. The deficiencies identified may be minor, for example, requiring labeling changes; or major, for example, requiring additional clinical trials. Additionally, the CRL may include recommended actions that the sponsor might take to place the application in a condition for approval. If a CRL is issued, the sponsor may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application. For those seeking to challenge FDA's CRL decision, the agency has indicated that sponsors may request a formal hearing on the CRL or they may file a request for reconsideration or a request for a formal dispute resolution.

If a product receives regulatory approval, the FDA will issue an approval letter. The approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing or dispensing in the form of a REMS, or otherwise limit the scope of any approval. In addition, the FDA may require post-marketing clinical trials, designed to further assess a biologic product's safety, purity and potency, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

The FDA has agreed to specified performance goals in the review of BLAs under the PDUFA. One such goal is to review standard BLAs in ten months after the FDA accepts the BLA for filing, and priority BLAs in six months, whereupon a review decision is to be made. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs and its review goals are subject to change from time to time. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the BLA sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

Biosimilars and reference product exclusivity

The Patient Protection and Affordable Care Act and the companion Health Care and Education Reconciliation Act, or the Health Care Reform Law, which was signed into law on March 23, 2010, included a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA. That Act established a regulatory scheme authorizing the FDA to approve biosimilars and interchangeable biosimilars. To date, the FDA has approved a number of biosimilars and it has also issued numerous guidance documents outlining its approach to reviewing and licensing biosimilars and interchangeable biosimilars under the PHS Act.

Under the BPCIA, a manufacturer may submit an application for licensure of a biologic product that is "biosimilar to" or "interchangeable with" a previously approved biological product or "reference product." In order for the FDA to approve a biosimilar product, it must find that there are no clinically meaningful differences between the reference product and proposed biosimilar product in terms of safety, purity and potency. For the FDA to approve a biosimilar product as interchangeable with a reference product, the agency must find that the biosimilar product can be expected to produce the

same clinical results as the reference product, and (for products administered multiple times) that the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. In December 2022, Congress clarified through FDORA that FDA may approve multiple first interchangeable biosimilar biological products so long as the products are all approved on the first day on which such a product is approved as interchangeable with the reference product.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date of first licensure of the reference product. The FDA may not approve a biosimilar product until 12 years from the date on which the reference product was first licensed. Even if a product is considered to be a reference product eligible for exclusivity, another company could market a competing version of that product if the FDA approves a full BLA for such product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. There have been recent government proposals to reduce the 12-year reference product exclusivity period, but none has been enacted to date. Since passage of the BPCIA, many states have passed laws or amendments to laws, which address pharmacy practices involving biosimilar products.

As of December 27, 2020 (enacted as part of the Consolidated Appropriations Act, 2021), the "patent dance" lists became public information as listed in the Purple Book (FDA's "Database of Licensed Biological Products"). In particular, reference product BLA holders must submit to the FDA within 30 days of exchanging a patent list (patents with expiry dates) with a biosimilar applicant, as well as any supplemental lists. This information was previously maintained as confidential as between the BLA holder and biosimilar applicant. Despite publication of these lists, a BLA holder may assert other patents against future filers, and does not exclude enforcement of newly granted patents.

Additionally, under the Act, the FDA must now publish in the Purple Book the following information about patented biological products:

- a list of each biological product, by nonproprietary name, for which a biologics license is in effect;
- the date of licensure and the application number;
- the licensure status and, as available, the marketing status; and
- exclusivity periods.

The FDA must publish in the Purple Book all of the above information in the first instance within 180 days of enactment and update every 30 days.

Pediatric exclusivity

Pediatric exclusivity is another type of non-patent exclusivity in the United States and, if granted, provides for the attachment of an additional six months of regulatory exclusivity to the term of any existing regulatory exclusivity, including reference product and orphan exclusivity. This six-month exclusivity may be granted if an application sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity cover the product are extended by six months. Thus, pediatric exclusivity adds six months to existing exclusivity periods applicable to biological products under the BPCIA—namely, the four-year period during which the FDA will not consider an application for a biosimilar product, and the 12-year period during which the FDA will not approve a biosimilar application.

Orphan drug designation and exclusivity

Under the Orphan Drug Act, the FDA may designate a biologic product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a biologic product available in the United States for treatment of the disease or condition will be recovered from sales of the product). Orphan product designation must be requested before submitting a BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, meaning that the FDA may not approve any other applications to market the same drug or biologic product for the same indication for seven years, except in limited circumstances, such as if the party holding the exclusivity fails to assure the availability of sufficient quantities of the drug to

meet the needs of patients with the disease or condition for which the drug was designated. In addition, the FDA may not approve other applications to market the same drug or biologic product for the same indication for seven years unless the sponsor of the other product demonstrates that its product is clinically superior to the product with orphan drug exclusivity. Under Omnibus legislation enacted in December 2020, this clinical superiority requirement applies to drugs and biologics that received orphan drug designation before enactment of the FDA Reauthorization Act in 2017, but have not yet been approved or licensed by FDA.

Orphan exclusivity does not block the approval of a different product for the same rare disease or condition, nor does it block the approval of the same product for different indications. In particular, the concept of what constitutes the "same drug" for purposes of orphan drug exclusivity remains in flux in the context of gene therapies, and the FDA issued final guidance in September 2021 suggesting that it would not consider two gene therapy products to be different drugs solely based on minor differences in the transgenes or capsids. If a product designated as an orphan drug ultimately receives marketing approval for an indication broader than what was designated in its orphan drug application, it may not be entitled to exclusivity. Orphan medicinal product status in the European Union has similar, but not identical, benefits.

In September 2021, the Court of Appeals for the 11th Circuit held that, for the purpose of determining the scope of market exclusivity, the term “same disease or condition” in the statute means the designated “rare disease or condition” and could not be interpreted by the FDA to mean the “indication or use.” Thus, the court concluded, orphan drug exclusivity applies to the entire designated disease or condition rather than the “indication or use.” Although there have been legislative proposals to overrule this decision, they have not been enacted into law. In January 2023, FDA announced that, in matters beyond the scope of that court order, FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug was approved.

Expedited development and review programs

The FDA is authorized to expedite the review of BLAs in several ways. Under the Fast Track program, the sponsor of a biologic product candidate may request the FDA to designate the product for a specific indication as a Fast Track product concurrent with or after the filing of the IND. Biologic products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. In addition to other benefits, such as the ability to have greater interactions with the FDA, the FDA may initiate review of sections of a Fast Track BLA before the application is complete, a process known as rolling review.

Any product submitted to the FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as breakthrough therapy designation, priority review and accelerated approval.

- *Breakthrough therapy designation.* To qualify for the breakthrough therapy program, product candidates must be intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence must indicate that such product candidates may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. The FDA will seek to ensure the sponsor of a breakthrough therapy product candidate receives intensive guidance on an efficient drug development program, intensive involvement of senior managers and experienced staff on a proactive, collaborative and cross-disciplinary review and rolling review.
- *Priority review.* A product candidate is eligible for priority review if it treats a serious condition and, if approved, it would be a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention compared to marketed products. FDA aims to complete its review of priority review applications within six months as opposed to 10 months for standard review.
- *Accelerated approval.* Drug or biologic products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval. Accelerated approval means that a product candidate may be approved on the basis of adequate and well controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity and prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug or biologic product candidate receiving accelerated approval perform adequate and well controlled post-marketing clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials.

  With passage of FDORA, Congress modified certain provisions governing accelerated approval of drug and biologic products. Specifically, the new legislation authorized the FDA to: require a sponsor to have its confirmatory clinical trial underway before accelerated approval is awarded, require a sponsor of a product granted

accelerated approval to submit progress reports on its post-approval studies to FDA every six months (until the study is completed) and use expedited procedures to withdraw accelerated approval of a BLA after the confirmatory trial fails to verify the product's clinical benefit. Further, FDORA requires the FDA to publish on its website "the rationale for why a post-approval study is not appropriate or necessary" whenever it decides not to require such a study upon granting accelerated approval. In March 2023, the FDA issued draft guidance that outlines its current thinking and approach to accelerated approval.

- *Regenerative advanced therapy.* With passage of the Cures Act in December 2016, Congress authorized the FDA to accelerate review and approval of products designated as regenerative advanced therapies. A product is eligible for this designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product candidate has the potential to address unmet medical needs for such disease or condition. The benefits of a regenerative advanced therapy designation include early interactions with the FDA to expedite development and review, benefits available to breakthrough therapies, potential eligibility for priority review and accelerated approval based on surrogate or intermediate endpoints.

None of these expedited programs change the standards for approval but they may help expedite the development or approval process of product candidates.

Rare Pediatric Disease Designation and Priority Review Vouchers

In 2012, Congress enacted the FDASIA, requiring the FDA to award priority review vouchers, or PRVs, to sponsors of certain rare pediatric disease product applications. This program is designed to encourage development of new drug and biological products for prevention and treatment of "rare pediatric diseases" by, upon initial approval of an application meeting certain specified criteria, providing companies with a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease product receiving a PRV may sell or otherwise transfer the voucher to another company. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted an application relying on the priority review voucher. The FDA may also revoke any PRV if the rare pediatric disease product for which the voucher was awarded is not marketed in the United States within one year following the date of approval.

In order to receive a PRV upon BLA approval, the product must receive designation from the FDA as a product for a rare pediatric disease prior to submission of the marketing application. A "rare pediatric disease" is a disease that is serious or life-threatening, in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and affects fewer than 200,000 people in the United States, or affects more than 200,000 people in the United States but there is no reasonable expectation that the cost of developing and making available in the United States a product for such disease or condition will be recovered from sales in the United States of such product. In addition to receiving rare pediatric disease designation, in order to receive a PRV, the BLA must be given priority review, rely on clinical data derived from studies examining a pediatric population and dosages of the product intended for that population, not seek approval for a different adult indication in the original rare pediatric disease product application and be for a product that does not include a previously approved active ingredient.

The Rare Pediatric Disease PRV program was scheduled to expire after September 30, 2020. After that, only drugs designated as rare pediatric treatments and approved by the FDA by October 1, 2022, could receive a voucher. In December 2020, however, Congress renewed the program as part of the 2021 Coronavirus Response and Relief Supplemental Consolidated Appropriations Act through the federal fiscal year 2024. Thus, under the current statutory sunset provisions, FDA may only award PRVs for approved rare pediatric disease product applications if sponsors have rare pediatric disease designation for the drug granted by September 30, 2024. The FDA may not award any rare pediatric disease PRVs after September 30, 2026.

Post-approval requirements

After regulatory approval of a product is obtained, there may be a number of post-approval requirements. For example, as a condition of approval of a BLA, the FDA may require post-marketing testing and surveillance to monitor the product's safety or efficacy. In addition, holders of an approved BLA are required to keep extensive records, to report certain adverse reactions and production problems to the FDA, to provide updated safety and efficacy information and to comply with requirements concerning advertising and promotional labeling for their products. Also, quality control and manufacturing procedures must continue to conform to cGMP regulations and practices, as well as the manufacturing conditions of approval set forth in the BLA. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior regulatory approval before being implemented. Regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. The FDA periodically inspects manufacturing facilities to assess compliance with

cGMP requirements, which impose certain procedural, substantive and recordkeeping requirements. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

Further, although physicians may prescribe legally available products for unapproved uses or patient populations, which are commonly referred to as "off-label uses," manufacturers may not market or promote such uses. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability. In September 2021, the FDA published final regulations which describe the types of evidence that the FDA will consider in determining the intended use of a biologic. If a company is found to have promoted off-label uses, it may become subject to administrative and judicial enforcement by the FDA, the Department of Justice, or the Office of the Inspector General of the HHS, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug products.

It may be permissible, under very specific, narrow conditions, for a manufacturer to engage in nonpromotional, non-misleading communication regarding off-label information, such as distributing scientific or medical journal information. Moreover, with passage of the Pre-Approval Information Exchange Act in December 2022, sponsors of products that have not been approved may proactively communicate to payors certain information about products in development to help expedite patient access upon product approval. Previously, such communications were permitted under FDA guidance but the new legislation explicitly provides protection to sponsors who convey certain information about products in development to payors, including unapproved uses of approved products. Further, in October 2023, the FDA published draft guidance outlining the FDA's non-binding policies governing the distribution of scientific information on unapproved uses to healthcare providers. This draft guidance calls for such communications to be truthful, non-misleading, factual and unbiased and include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use..

U.S. patent term restoration

Depending upon the timing, duration and specifics of FDA approval of product candidates, some of a sponsor's U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent terms lost during product development and FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period generally is one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved biologic product is eligible for the extension, the application for the extension must be submitted prior to the expiration of the patent, and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. Moreover, a given patent may only be extended once based on a single product. The USPTO in consultation with the FDA, reviews and approves the application for any patent term extension or restoration.

Federal and state data privacy laws

There are multiple privacy and data security laws that may impact our business activities, in the United States and in other countries where we conduct trials or where we may do business in the future. These laws are evolving and may increase both our obligations and our regulatory risks in the future. In the health care industry generally, under The Health Insurance Portability and Accountability Act, or HIPAA, the HHS has issued regulations to protect the privacy and security of protected health information used or disclosed by covered entities including certain healthcare providers, health plans, and healthcare clearinghouses. HIPAA also regulates standardization of data content, codes and formats used in healthcare transactions and standardization of identifiers for health plans and providers. HIPAA also imposes certain obligations on the business associates of covered entities that obtain protected health information in providing services to or on behalf of covered entities. HIPAA may apply to us in certain circumstances and may also apply to our business partners in ways that may impact our relationships with them. Our clinical trials will be regulated by the Common Rule, which also includes specific privacy-related provisions. In addition to federal privacy regulations, there are a number of state laws governing confidentiality and security of health information that may be applicable to our business. In addition to possible federal civil and criminal penalties for HIPAA violations, state attorneys general are authorized to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, state attorneys general (along with private plaintiffs) have brought civil actions seeking injunctions and damages resulting from alleged violations of HIPAA's privacy and security rules. State attorneys general also have authority to enforce state privacy and security laws. New laws and regulations governing privacy and security may be adopted in the future as well.

In 2018 California passed into law the California Consumer Privacy Act, or the CCPA, which took effect on January 1, 2020, and imposed many requirements on businesses that process the personal information of California residents. Many of the CCPA's requirements are similar to those found in the General Data Protection Regulation, or the GDPR, including requiring businesses to provide notice to data subjects regarding the information collected about them and how such information is used and shared, and providing data subjects the right to request access to such personal information and, in certain cases, request the erasure of such personal information. The CCPA also affords California residents the right to opt-out of "sales" of their personal information. The CCPA contains significant penalties for companies that violate its requirements. In November 2020, California voters passed a ballot initiative for the California Privacy Rights Act, or the CPRA, which went into effect on January 1, 2023, and significantly expanded the CCPA to incorporate additional GDPR-like provisions including requiring that the use, retention, and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information, and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA also created a new enforcement agency—the California Privacy Protection Agency—whose sole responsibility is to enforce the CPRA, which will further increase compliance risk. The provisions in the CPRA may apply to some of our business activities.

In addition to California, a number of states have passed comprehensive privacy laws similar to the CCPA and CPRA. These laws are either in effect or will go into effect sometime before the end of 2026. Like the CCPA and CPRA, these laws create obligations related to the processing of personal information, as well as special obligations for the processing of "sensitive" data, which includes health data in some cases. Some of the provisions of these laws may apply to our business activities. There are also states that are strongly considering or have already passed comprehensive privacy laws during the 2024 legislative sessions that will go into effect in 2025 and beyond. Other states will be considering similar laws in the future, and Congress has also been debating passing a federal privacy law. There are also states that are specifically regulating health information that may affect our business. For example, the State of Washington passed the My Health My Data Act in 2023 which specifically regulated health information that is not otherwise regulated by the HIPAA rules, and the law also has a private right of action, which further increases the relevant compliance risk. Connecticut and Nevada have also passed similar laws regulating consumer health data, and more states are considering such legislation in 2024. These laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available under such laws, it is possible that some of our current or future business activities, including certain clinical research, sales and marketing practices, and the provision of certain items and services to our customers, could be subject to challenge under one or more of such privacy and data security laws. The heightening compliance environment and the need to build and maintain robust and secure systems to comply with different privacy compliance and/or reporting requirements in multiple jurisdictions could increase the possibility that a healthcare company may fail to comply fully with one or more of these requirements. If our operations are found to be in violation of any of the privacy or data security laws or regulations described above that are applicable to us, or any other laws that apply to us, we may be subject to penalties, including potentially significant criminal, civil, and administrative penalties, damages, fines, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and/or oversight if we become subject to a consent decree or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. To the extent that any product candidates we may develop, once approved, are sold in a foreign country, we may be subject to similar foreign laws.

Government regulation outside of the U.S.

In addition to regulations in the United States, a manufacturer is subject to a variety of regulations in foreign jurisdictions to the extent it chooses to sell any products in those foreign countries. Even if a manufacturer obtains FDA approval of a product, it must still obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Because biologically sourced materials are subject to unique contamination risks, their use may also be restricted in some countries. As in the United States, medicinal products can be marketed only if a marketing authorization from the competent regulatory agencies has been obtained. Similar to the United States, the various phases of preclinical and clinical research in the European Union are subject to significant regulatory controls.

With the exception of the European Union and European Economic Area, or EEA, applying the harmonized regulatory rules for medicinal products, the approval process and requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly between countries and jurisdictions and can involve additional testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does

not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

Non-clinical studies

Non-clinical studies are performed to demonstrate the health or environmental safety of new chemical or biological substances. Non-clinical (pharmaco-toxicological) studies must be conducted in compliance with the principles of good laboratory practice as set forth in EU Directive 2004/10/EC (unless otherwise justified for certain particular medicinal products – e.g., radio-pharmaceutical precursors for radio-labeling purposes). In particular, non-clinical studies, both in vitro and in vivo, must be planned, performed, monitored, recorded, reported and archived in accordance with the GLP principles, which define a set of rules and criteria for a quality system for the organizational process and the conditions for non-clinical studies. These GLP standards reflect the Organization for Economic Co-operation and Development requirements.

Clinical trial approval in the European Union

On January 31, 2022, the new Clinical Trials Regulation (EU) No 536/2014, or CTR, became effective in the European Union and replaced the prior Clinical Trials Directive 2001/20/EC. The new regulation aims at simplifying and streamlining the authorization, conduct and transparency of clinical trials in the European Union. Under the new coordinated procedure for the approval of clinical trials, the sponsor of a clinical trial to be conducted in more than one Member State of the European Union, or EU Member State, will only be required to submit a single application for approval. The submission will be made through the Clinical Trials Information System, a new clinical trials portal overseen by the EMA and available to clinical trial sponsors, competent authorities of the EU Member States and the public.

Beyond streamlining the process, CTR includes a single set of documents to be prepared and submitted for the application as well as simplified reporting procedures for clinical trial sponsors, and a harmonized procedure for the assessment of applications for clinical trials, which is divided in two parts. Part I is assessed by the competent authorities of all EU Member States in which an application for authorization of a clinical trial has been submitted (Member States concerned). Part II is assessed separately by each Member State concerned. Strict deadlines have been established for the assessment of clinical trial applications. The role of the relevant ethics committees in the assessment procedure will continue to be governed by the national law of the concerned EU Member State. However, overall related timelines will be defined by the CTR.

The CTR did not change the preexisting requirement that a sponsor must obtain prior approval from the competent national authority of the EU Member State in which the clinical trial is to be conducted. If the clinical trial is conducted in different EU Member States, the competent authorities in each of these EU Member States must provide their approval for the conduct of the clinical trial. Furthermore, the sponsor may only start a clinical trial at a specific study site after the applicable ethics committee has issued a favorable opinion.

The CTR foresees a three-year transition period. The extent to which ongoing and new clinical trials will be governed by the CTR varies. Clinical trials for which an application was submitted (i) prior to January 31, 2022 under the Clinical Trials Directive, or (ii) between January 31, 2022 and January 31, 2023 and for which the sponsor has opted for the application of the Clinical Trials Directive remain governed by said directive until January 31, 2025. After this date, all clinical trials (including those which are ongoing) will become subject to the provisions of the CTR.

Parties conducting certain clinical studies must post clinical trial information in the European Union at the EU Clinical Trials Register.

PRIME designation

In March 2016, the EMA, launched the PRIority MEdicines, or PRIME, initiative to foster research and development of medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients without treatment options. PRIME aims to strengthen clinical trial designs to facilitate the generation of high-quality data for the evaluation of an application for marketing authorization. To be accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on preclinical and/or early clinical data. These medicines are considered priority medicines within the European Union.

After an investigational candidate has been selected for PRIME, developers are assigned a rapporteur from the Committee for Human Medicinal Products, or CHMP, to provide continuous support and help to build knowledge ahead of a marketing authorization application, or MAA. A multidisciplinary group of experts will provide broader guidance on the overall development plan and regulatory strategy of the product. Companies are also eligible for accelerated assessment at the time of their regulatory application.

Pediatric studies

Sponsors developing a new medicinal product must agree upon a Pediatric Investigation Plan, or PIP, with the EMA's pediatric committee, or PDCO, and must conduct pediatric clinical trials in accordance with that PIP, unless a waiver applies (e.g., because the relevant disease or condition occurs only in adults). The PIP sets out the timing and measures proposed to generate data to support a pediatric indication of the drug for which marketing authorization is being sought. The marketing authorization application for the product must include the results of pediatric clinical trials conducted in accordance with the PIP, unless a waiver applies, or a deferral has been granted by the PDCO of the obligation to implement some or all of the measures of the PIP until there are sufficient data to demonstrate the efficacy and safety of the product in adults, in which case the pediatric clinical trials must be completed at a later date.

Marketing authorization

In the European Union, marketing authorizations for medicinal products may be obtained through several different procedures founded on the same basic regulatory process.

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid across the European Economic Area (i.e., the European Union as well as Iceland, Liechtenstein and Norway). The centralized procedure is compulsory for medicinal products produced by certain biotechnological processes, products designated as orphan medicinal products, and products with a new active substance indicated for the treatment of certain diseases. It is optional for those products that are highly innovative or for which a centralized process is in the interest of patients. Under the centralized procedure in the European Union, the maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops, when additional written or oral information is to be provided by the sponsor in response to questions asked by the CHMP. Accelerated evaluation may be granted by the CHMP in exceptional cases. These are defined as circumstances in which a medicinal product is expected to be of a "major public health interest." Three cumulative criteria must be fulfilled in such circumstances: the seriousness of the disease, such as severely disabling or life-threatening diseases, to be treated; the absence or insufficiency of an appropriate alternative therapeutic approach; and anticipation of high therapeutic benefit. In these circumstances, the EMA ensures that the opinion of the CHMP is given within 150 days.

The EMA's Committee for Advanced Therapies, or CAT, is responsible for assessing the quality, safety and efficacy of advanced-therapy medicinal products. Advanced-therapy medical products include gene therapy medicine, somatic cell therapy medicines and tissue-engineered medicines. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. In the European Union, the development and evaluation of a gene therapy medicinal product must be considered in the context of the relevant EU guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for somatic cell therapy medicinal products and require that we comply with these new guidelines. Similarly, complex regulatory environments exist in other jurisdictions in which we might consider seeking regulatory approvals for our product candidates, further complicating the regulatory landscape. As a result, the procedures and standards applied to gene therapy products and cell therapy products may be applied to any of our gene therapy or genome editing candidates, but that remains uncertain at this point.

Specifically, the grant of marketing authorization in the European Union for products containing viable human tissues or cells such as gene therapy medicinal products is governed by Regulation 1394/2007/EC on advanced therapy medicinal products, read in combination with Directive 2001/83/EC of the European Parliament and of the Council, commonly known as the Community code on medicinal products. Regulation 1394/2007/EC includes specific rules concerning the authorization, supervision, and pharmacovigilance of gene therapy medicinal products, somatic cell therapy medicinal products, and tissue engineered products. Manufacturers of advanced therapy medicinal products must demonstrate the quality, safety, and efficacy of their products to EMA which provides an opinion regarding the MAA. The European Commission grants or refuses marketing authorization in light of the opinion delivered by EMA.

The decentralized procedure provides for approval by one or more other concerned EU Member States of an assessment of an application for marketing authorization conducted by one EU Member State, known as the reference EU Member State. In accordance with this procedure, a sponsor submits an application for marketing authorization to the reference EU Member State and the concerned EU Member States. This application is identical to the application that would be submitted to the EMA for authorization through the centralized procedure. The reference EU Member State prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. The resulting assessment report is submitted to the concerned EU Member States which, within 90 days of receipt, must decide whether to approve the assessment report and related materials.

If a concerned EU Member State cannot approve the assessment report and related materials due to concerns relating to a potential serious risk to public health, disputed elements may be referred to the European Commission, whose decision is binding on all EU Member States. In accordance with the mutual recognition procedure, the sponsor applies for national marketing authorization in one EU Member State. Upon receipt of this authorization the sponsor can then seek the recognition of this authorization by other EU Member States. Authorization in accordance with either of these procedures will result in authorization of the medicinal product only in the reference EU Member State and in the other concerned EU Member States.

A marketing authorization may be granted only to a sponsor established in the European Union. Regulation No. 1901/2006 provides that, prior to obtaining a marketing authorization in the European Union, a sponsor must demonstrate compliance with all measures included in a PIP approved by the PDCO, covering all subsets of the pediatric population, unless the EMA has granted a product-specific waiver, class waiver, or a deferral for one or more of the measures included in the PIP.

Conditional marketing authorization

In specific circumstances, European Union legislation on Conditional Marketing Authorizations for Medicinal Products for Human Use, or conditional marketing authorization, enables sponsors to obtain a conditional marketing authorization prior to obtaining the comprehensive clinical data required for an application for a full marketing authorization. Such conditional approvals may be granted for product candidates (including medicines designated as orphan medicinal products) if the risk-benefit balance of the product candidate is positive, it is likely that the sponsor will be in a position to provide the required comprehensive clinical trial data, the product fulfills unmet medical needs and the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required. A conditional marketing authorization may contain specific obligations to be fulfilled by the marketing authorization holder, including obligations with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data.

Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions and/or specific obligations. The timelines for the centralized procedure described above also apply with respect to the review by the CHMP of applications for a conditional marketing authorization.

Exceptional circumstances

A marketing authorization may also be granted "under exceptional circumstances" when the applicant can show that it is unable to provide comprehensive data on the efficacy and safety under normal conditions of use even after the product has been authorized and subject to specific procedures being introduced. This may arise in particular when the intended indications are very rare and, in the present state of scientific knowledge, it is not possible to provide comprehensive information, or when generating data may be contrary to generally accepted ethical principles. This marketing authorization is close to the conditional marketing authorization as it is reserved to medicinal products to be approved for severe diseases or unmet medical needs and the applicant does not hold the complete data set legally required for the grant of a marketing authorization. However, unlike the conditional marketing authorization, the applicant does not have to provide the missing data and will never have to. Although the marketing authorization "under exceptional circumstances" is granted definitively, the risk-benefit balance of the medicinal product is reviewed annually and the MA is withdrawn in case the risk-benefit ratio is no longer favorable. Under these procedures, before granting the marketing authorization, the EMA or the competent authorities of the EU Member States make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety, and efficacy. Except conditional marketing authorizations, marketing authorizations have an initial duration of five years. After these five years, the authorization may be renewed on the basis of a reevaluation of the risk-benefit balance.

Orphan drug designation and exclusivity

The criteria for designating an orphan medicinal product in the European Union are similar in principle to those in the United States. Under Article 3 of Regulation (EC) 141/2000, a medicinal product may be designated as orphan if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition, (2) either (a) such condition affects no more than five in 10,000 persons in the European Union when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the European Union to justify investment and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the European Union, or if such a method exists, the product will be of significant benefit to those affected by the condition. The term "significant benefit" is defined in Regulation (EC) 847/2000 to mean a clinically relevant advantage or a major contribution to patient care.

Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and are, upon grant of a marketing authorization, entitled to ten years of market exclusivity for the approved therapeutic indication. During this ten year market exclusivity period, the EMA or the competent authorities of the Member States of the European Economic Area, or EEA, cannot accept an application for a marketing authorization for a similar medicinal product for the same indication. A similar medicinal product is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The application for orphan designation must be submitted before the application for marketing authorization. The sponsor will receive a fee reduction for the marketing authorization application if the orphan designation has been granted, but not if the designation is still pending at the time the marketing authorization is submitted. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The ten-year market exclusivity in the European Union may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, marketing authorization may be granted to a similar product for the same indication at any time if: (1) the second sponsor can establish that its product, although similar, is safer, more effective or otherwise clinically superior; (2) the sponsor consents to a second orphan medicinal product application; or (3) the sponsor cannot supply enough orphan medicinal product.

Pediatric Exclusivity

Products that are granted a marketing authorization with the results of the pediatric clinical trials conducted in accordance with the PIP are eligible for a six month extension of the protection under a supplementary protection certificate (if any is in effect at the time of approval) even where the trial results are negative. In the case of orphan medicinal products, a two-year extension of the orphan market exclusivity may be available. This pediatric reward is subject to specific conditions and is not automatically available when data in compliance with the PIP are developed and submitted.

Brexit and the regulatory framework in the United Kingdom

The United Kingdom's withdrawal from the European Union took place on January 31, 2020. As of January 1, 2021, the Medicines and Healthcare products Regulatory Agency, or the MHRA, became responsible for supervising medicines and medical devices in Great Britain, comprising England, Scotland and Wales under domestic law whereas Northern Ireland continues to be subject to EU rules under the Northern Ireland Protocol.

On February 27, 2023, the UK government and the European Commission announced a political agreement in principle to replace the Northern Ireland Protocol with a new set of arrangements, known as the "Windsor Framework". This new framework fundamentally changes the existing system under the Northern Ireland Protocol, including with respect to the regulation of medicinal products in the UK In particular, the MHRA will be responsible for approving all medicinal products destined for the UK market (i.e., Great Britain and Northern Ireland), and the EMA will no longer have any role in approving medicinal products destined for Northern Ireland. A single UK-wide marketing authorization will be granted by the MHRA for all medicinal products to be sold in the UK, enabling products to be sold in a single pack and under a single authorization throughout the UK The Windsor Framework was approved by the EU-UK Joint Committee on March 24, 2023, so the UK government and the European Union will enact legislative measures to bring it into law. On June 9, 2023, the MHRA announced that the medicines aspects of the Windsor Framework will apply from January 1, 2025. The Human Medicines Regulations 2012 (SI 2012/1916) (as amended), or HMR, is the primary legal instrument for the regulation of medicines in the UK. The HMR has incorporated into the domestic law the body of EU law instruments governing medicinal products that pre-existed prior to the UK's withdrawal from the European Union.

EU laws which have been transposed into UK law through secondary legislation continue to be applicable as "retained EU law." However, new legislation such as the (EU) Clinical Trials Regulation will not be applicable in Great Britain. Since a significant proportion of the regulatory framework for pharmaceutical products in the UK covering the quality, safety, and efficacy of pharmaceutical products, clinical trials, marketing authorizations, commercial sales, and distribution of pharmaceutical products is derived from EU directives and regulations, Brexit may have a material impact upon the regulatory regime with respect to the development, manufacture, importation, approval, and commercialization of our product candidates in the UK. For example, the UK is no longer covered by the centralized procedures for obtaining EU-wide marketing authorizations from the EMA, and a separate marketing authorization will be required to market our product candidates in the UK. A new international recognition framework has been in place since January 1, 2024, whereby the MHRA will have regard to decisions on the approval of marketing authorizations made by the EMA and certain other regulators when determining an application for a new Great Britian marketing authorization.

As with other issues related to withdrawal of the UK from the European Union, there are open questions about how personal data will be protected in the UK and whether personal information can transfer from the European Union to the UK. Following the withdrawal of the UK from the European Union, the UK Data Protection Act of 2018 applies to the processing

of personal data that takes place in the UK and includes parallel obligations to those set forth by the GDPR. While the Data Protection Act of 2018 in the UK that "implements" and complements the GDPR has achieved Royal Assent on May 23, 2018 and is now effective in the UK, it is still unclear whether transfer of data from the EEA to the UK will remain lawful under the GDPR. The UK government has already determined that it considers all EU and EEA member states to be adequate for the purposes of data protection, ensuring that data flows from the UK to the EU/EEA remain unaffected. In addition, a recent decision from the European Commission appears to deem the UK as being "essentially adequate" for purposes of data transfer from the European Union to the UK, although this decision may be re-evaluated in the future.

General data protection regulation

The collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the EU, including personal health data, is subject to the GDPR, which became effective on May 25, 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the EU, including the U.S., and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR.

Compliance with the GDPR will be a rigorous and time-intensive process that may increase the cost of doing business or require companies to change their business practices to ensure full compliance. In July 2020, the Court of Justice of the European Union, or the CJEU, invalidated the EU-U.S. Privacy Shield framework, one of the mechanisms used to legitimize the transfer of personal data from the EEA to the United States. The CJEU decision also drew into question the long-term viability of an alternative means of data transfer, the standard contractual clauses, for transfers of personal data from the EEA to the United States.

Additionally, in October 2022, President Biden signed an executive order to implement the EU-U.S. Data Privacy Framework, which would serve as a replacement to the EU-U.S. Privacy Shield. The European Commission initiated the process to adopt an adequacy decision for the EU-U.S. Data Privacy Framework in December 2022 and the European Commission adopted the adequacy decision on July 10, 2023. The adequacy decision permits U.S. companies who self-certify to the EU-U.S. Data Privacy Framework to rely on it as a valid data transfer mechanism for data transfers from the European Union to the United States. However, some privacy advocacy groups have already suggested that they will be challenging the EU-U.S. Data Privacy Framework. If these challenges are successful, they may not only impact the EU-U.S. Data Privacy Framework, but also further limit the viability of the standard contractual clauses and other data transfer mechanisms. The uncertainty around this issue may further impact our business operations in the EU.

Following the withdrawal of the United Kingdom from the European Union, the U.K. Data Protection Act 2018 applies to the processing of personal data that takes place in the United Kingdom and includes parallel obligations to those set forth by GDPR, including in relation to data transfers.

Healthcare law and regulation

Healthcare providers and third-party payors play a primary role in the recommendation and prescription of products that are granted marketing approval. Arrangements with providers, consultants, third-party payors and customers are subject to broadly applicable fraud and abuse, anti-kickback, false claims laws, reporting of payments to physicians and teaching physicians and patient privacy laws and regulations and other healthcare laws and regulations that may constrain business and/or financial arrangements. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, paying, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;
- the federal civil and criminal false claims laws, including the civil False Claims Act, and civil monetary penalties laws, which prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false, fictitious or fraudulent or knowingly

making, using or causing to made or used a false record or statement to avoid, decrease or conceal an obligation to pay money to the federal government;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal laws that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and their respective implementing regulations, including the Final Omnibus Rule published in January 2013, which impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal false statements statute, which prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the federal transparency requirements known as the federal Physician Payments Sunshine Act, under the Health Care Reform Law, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to the Centers for Medicare & Medicaid Services, or CMS, within the United States Department of Health and Human Services, information related to payments and other transfers of value made by that entity to physicians, other healthcare professionals and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to healthcare items or services that are reimbursed by non-governmental third-party payors, including private insurers.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Pharmaceutical insurance coverage and health care reform

In the United States and markets in other countries, patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated health care costs. Significant uncertainty exists as to the coverage and reimbursement status of products approved by the FDA and other government authorities. Thus, even if a product candidate is approved, sales of the product will depend, in part, on the extent to which third-party payors, including government health programs in the United States such as Medicare and Medicaid, commercial health insurers and managed care organizations, provide coverage and establish adequate reimbursement levels for, the product. The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors are increasingly challenging the prices charged, examining the medical necessity and reviewing the cost-effectiveness of medical products and services and imposing controls to manage costs. Third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the approved products for a particular indication.

By way of example, the United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. In March 2010, the United States Congress enacted the Health Care Reform Law, which, among other things, includes changes to the coverage and payment for products under government health care programs. In addition, other legislative changes have been proposed and adopted since the Health Care Reform Law was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013. Under current legislation, the actual reductions in Medicare payments may vary up to 4%. The Consolidated Appropriations Act, which was signed into law by President Biden in December 2022, made several changes to sequestration of the Medicare program. Section 1001 of the Consolidated Appropriations Act delays the 4% Statutory Pay-As-You-Go Act of 2010 sequester for two years, through the end of 2024.

Triggered by enactment of the American Rescue Plan Act of 2021, the 4% cut to the Medicare program would have taken effect in January 2023. The Consolidated Appropriations Act's health care offset title includes Section 4163, which extends the two percent Budget Control Act of 2011 Medicare sequester for six months into 2032 and lowers the payment reduction percentages in years 2030 and 2031.

The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our candidates for which we may obtain regulatory approval or the frequency with which any such candidate is prescribed or used. Indeed, under current legislation, the actual reductions in Medicare payments may vary up to 4%.

Since enactment of the Health Care Reform Law, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, or the TCJA, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the Patient Protection and Affordable Care Act, or PPACA, brought by several states without specifically ruling on the constitutionality of the PPACA. Litigation and legislation over the Health Care Reform Law are likely to continue, with unpredictable and uncertain results.

Although the previous administration took executive actions to undermine or delay implementation of the Health Care Reform Law, President Biden rescinded those actions with the issuance of an Executive Order on January 28, 2021, which directs federal agencies to reconsider rules and other policies that limit Americans' access to health care, and consider actions that will protect and strengthen that access. Under this Order, federal agencies are directed to re-examine: policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the Health Care Reform Law that may reduce coverage or undermine the programs, including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the Health Care Reform Law; and policies that reduce affordability of coverage or financial assistance, including for dependents.

Pharmaceutical Prices

The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States. There have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to pharmaceutical pricing, review the relationship between pricing and manufacturer patient programs, and reduce the costs of pharmaceuticals under Medicare and Medicaid. In 2020, the prior administration issued several executive orders intended to lower the costs of prescription products and certain provisions in these orders have been incorporated into regulations. These regulations include an interim final rule implementing a most favored nation model for prices that would tie Medicare Part B payments for certain physician-administered pharmaceuticals to the lowest price paid in other economically advanced countries, effective January 1, 2021. That rule, however, has been subject to a nationwide preliminary injunction and, on December 29, 2021, CMS issued a final rule to rescind it. With issuance of this rule, CMS stated that it will explore all options to incorporate value into payments for Medicare Part B pharmaceuticals and improve beneficiaries' access to evidence-based care.

In addition, in October 2020, HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program, or SIP, to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by the Pharmaceutical Research and Manufacturers of America, or PhRMA, but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. Nine states have passed laws allowing for the importation of drugs from Canada. Certain of these states have submitted Section 804 Importation Program proposals and are awaiting FDA approval. In January 2024, the FDA approved Florida's plan for Canadian drug importation.

Further, on November 20, 2020, HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The final rule would also eliminate the current safe harbor for Medicare drug rebates and create new safe harbors for beneficiary point-of-sale discounts and pharmacy benefit manager service fees. It originally was set to go into effect on January 1, 2022, but with passage of the Inflation Reduction Act, or the IRA, has been delayed by Congress to January 1, 2032.

The IRA has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price

negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the Department of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Medicare Part D drugs in 2027, 15 Medicare Part B or Part D drugs in 2028, and 20 Medicare Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years, but it does not apply to drugs and biologics that have been approved for a single rare disease or condition. Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $4,000 a year in 2024 and, thereafter beginning in 2025, at $2,000 a year.

The IRA includes a provision exempting orphan drugs from Medicare price negotiation but this exclusion has been interpreted by CMS in final guidance issued in July 2023 to apply only to those orphan drugs with an approved indication (or indications) for a single rare disease or condition. The final guidance clarifies that CMS will consider only active designations/approvals when evaluating a drug for the exclusion, such that designations/indications withdrawn before the selected drug publication date will not be considered. CMS also clarified that, if a drug loses its orphan drug exclusion status, the agency will use the earliest date of approval/licensure to determine whether the product is a qualifying single source drug subject to price negotiations.

In addition, the IRA potentially raises legal risks with respect to individuals participating in a Medicare Part D prescription drug plan who may experience a gap in coverage if they required coverage above their initial annual coverage limit before they reached the higher threshold, or "catastrophic period" of the plan. Individuals requiring services exceeding the initial annual coverage limit and below the catastrophic period, must pay 100% of the cost of their prescriptions until they reach the catastrophic period. Among other things, the IRA contains many provisions aimed at reducing this financial burden on individuals by reducing the co-insurance and co-payment costs, expanding eligibility for lower income subsidy plans, and price caps on annual out-of-pocket expenses, each of which could have potential pricing and reporting implications.

On June 6, 2023, Merck & Co. filed a lawsuit against the HHS and CMS asserting that, among other things, the IRA's Drug Price Negotiation Program for Medicare constitutes an uncompensated taking in violation of the Fifth Amendment of the Constitution. Subsequently, a number of other parties, including the U.S. Chamber of Commerce and pharmaceutical companies, also filed lawsuits in various courts with similar constitutional claims against HHS and CMS. We expect that litigation involving these and other provisions of the IRA will continue, with unpredictable and uncertain results.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. A number of states, for example, require drug manufacturers and other entities in the drug supply chain, including health carriers, pharmacy benefit managers, wholesale distributors, to disclose information about pricing of pharmaceuticals. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs.

In the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. For example, the European Union provides options for its EU Member States to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. Member States may approve a specific price for a product, or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other EU Member States allow companies to fix their own prices for products but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the European Union have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage health care expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the European Union. The downward pressure on health care costs in general, particularly prescription products, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU Member States, and parallel trade, i.e., arbitrage between low-priced and high-priced EU Member States, can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any products, if approved in those countries.

Environmental regulations

We are also subject to regulation under the Occupational Safety and Health Act, the Environmental Protection Act, the Toxic Substances Control Act, the Resource Conservation and Recovery Act and other present and potential federal, state or local regulations. These and other laws govern our use, handling and disposal of various biological and chemical substances used in, and waste generated by, our operations. Our research and development involves the controlled use of hazardous materials, chemicals and viruses.

Human Capital

We recognize that attracting, motivating and retaining talented employees is vital to our success. We value the health and wellness of our employees and their families. It is our goal to deliver innovative programs that provide choice, quality and value. We aim to create an equitable, inclusive and empowering environment in which our employees can grow and advance their careers, with the overall goal of developing, expanding and retaining our workforce to support our current pipeline and future business goals. Our success also depends on our ability to attract, engage and retain a diverse group of employees. Our efforts to recruit and retain a diverse and passionate workforce include providing competitive compensation and benefits packages.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees, consultants and directors through the granting of stock-based compensation awards. We offer a comprehensive benefits program that provides resources to help employees manage their health, finances, and life outside of work.

As of December 31, 2023, we employed 88 full-time employees, including 65 in research and development and 23 in general and administrative positions, and of which 19 of our employees hold Ph.D. or M.D. degrees.

Corporate Information

Our principal executive offices are located at 500 Rutherford Avenue, Third Floor, Charlestown, Massachusetts 02129 and our telephone number is (617) 337-4680. Our website address is www.solidbio.com. The information contained in, or accessible through, our website does not constitute a part of this Annual Report on Form 10-K.

We make available free of charge on our website our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC.

Item 1A. Risk Factors.

You should carefully consider the following risk factors, in addition to the other information contained in this Annual Report on Form 10-K, including the section of this report titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes. If any of the events described in the following risk factors and the risks described elsewhere in this Annual Report on Form 10-K occurs, our business, operating results and financial condition could be seriously harmed and the trading price of our common stock could decline. This Annual Report on Form 10-K also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements as a result of factors that are described below and elsewhere in this Annual Report on Form 10-K. The risks and uncertainties described below are not the only ones we face. Additional risks not presently known to us or other factors not perceived by us to present significant risks to our business at this time also may impair our business operations.

Risks related to the Acquisition

We may fail to realize the anticipated benefits of our acquisition of AavantiBio, those benefits may take longer to realize than expected, and we may encounter significant integration difficulties.

On December 2, 2022, we completed our acquisition, or the Acquisition, of AavantiBio, Inc., or AavantiBio, and acquired AavantiBio's pipeline programs which included cardiac and neuromuscular programs. Our ability to realize the anticipated benefits of the Acquisition will depend, to a large extent, on our ongoing ability to integrate AavantiBio and these cardiac and neuromuscular programs into our business and business strategy and realize anticipated growth opportunities and synergies. We may fail to realize some or all of the anticipated benefits of the Acquisition. Potential difficulties we may encounter in the integration process include the following:

- the inability to successfully combine the businesses of Solid and AavantiBio in a manner that permits us to achieve the anticipated benefits from the Acquisition, which would result in the anticipated benefits of the Acquisition not being realized partly or wholly in the time frame currently anticipated or at all;
- difficulties in managing the expanded operations of a more complex company following the Acquisition;
- creation of uniform standards, controls, procedures, policies and information systems;
- difficulties in assimilating AavantiBio employees in our business, in maintaining employee morale and in attracting and retaining key personnel; and
- potential unknown liabilities, adverse consequences, or unforeseen increased expenses, delays or regulatory conditions associated with the Acquisition.

Also, we now possess certain liabilities and obligations, including contractual liabilities and obligations, that were assumed by us upon closing of the Acquisition. Further, it is possible that undisclosed, contingent or other liabilities, problems or obligations may arise in the future of which we were previously unaware. These disclosed and undisclosed liabilities could have an adverse effect on our business, financial condition and results of operations.

Any or all of these factors could decrease or delay the expected accretive effect of the Acquisition and negatively impact our stock price. As a result, it cannot be assured that we will be successful in the integration of AavantiBio with our business or that we will realize the benefits anticipated from the Acquisition or in the anticipated time frames or at all.

Our stockholders may not realize a benefit from the Acquisition and the related private placement commensurate with the ownership dilution they experienced in connection with the Acquisition and the related private placement.

If we are unable to realize the full strategic and financial benefits anticipated from the Acquisition, our stockholders will have experienced substantial dilution of their ownership interests without receiving any commensurate benefit, or only receiving part of the commensurate benefit to the extent the combined company is able to realize only part of the benefits anticipated from the Acquisition and the related private placement.

We may be exposed to increased litigation, including stockholder litigation, which could have an adverse effect on our business and operations.

We may be exposed to increased litigation from stockholders, customers, suppliers, consumers and other third parties due to the combination of Solid's and AavantiBio's businesses following the Acquisition. Such litigation may have an

adverse impact on our business and results of operations or may cause disruptions to our operations. In addition, in the past, stockholders have initiated class action lawsuits against biotechnology companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources, which could have a material adverse effect on our business, financial condition and results of operations.

Risks related to our financial position and need for capital requirements

We have incurred significant net losses since inception and anticipate that we will continue to incur net losses for the foreseeable future and may never achieve or maintain profitability.

Since inception, we have incurred significant net losses. Our net losses were $96.0 million, $86.0 million and $72.2 million for the years ended December 31, 2023, 2022 and 2021, respectively. As of December 31, 2023, we had an accumulated deficit of $658.8 million. Prior to the Acquisition, we had devoted substantially all of our efforts to research and development, including clinical development of SGT-001, which we are no longer developing, and preclinical development of SGT-003, as well as to building out our management team and infrastructure. Following the Acquisition, we also began devoting efforts to preclinical development of our other Candidates. We expect that it could be several years before we have a commercialized product, and we may never have a commercialized product. We expect to continue to incur significant expenses and see continued operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if, and as, we:

- enroll patients in our INSPIRE Duchenne trial and advance clinical development of SGT-003;
- advance our other Candidates into clinical trials;
- continue research and preclinical development of our Candidates and adjacent technologies such as assays;
- seek to identify additional candidates;
- engage in regulatory interactions with the FDA and other regulatory authorities;
- submit regulatory filings relating to the development of our Candidates and seek marketing approvals for our Candidates that successfully complete clinical trials, if any;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- arrange manufacturing for larger quantities of our product candidates for preclinical and clinical development and potential commercialization;
- maintain, expand, protect and enforce our intellectual property portfolio;
- hire and retain additional clinical, quality control and scientific personnel;
- build out new facilities or expand existing facilities to support our activities;
- acquire or in-license other drugs, drug candidates, technologies and intellectual property;
- fund a portion of the development or commercialization of products in collaboration with Ultragenyx pursuant to our collaboration and license agreement with Ultragenyx; and
- add operational, financial and management information systems and personnel.

To become and remain profitable, we must develop and eventually commercialize one or more product candidates with significant market potential. This will require us to be successful in a range of challenging activities, and our expenses will increase substantially as we enroll patients in and conduct the INSPIRE Duchenne trial and continue to develop our pipeline and complete ongoing and planned preclinical studies and clinical trials of our Candidates, obtain marketing approval for our Candidates, develop adjacent technologies such as assays, develop and validate commercial-scale manufacturing processes, manufacture, market and sell any future candidates for which we may obtain marketing approval and satisfy any post-marketing requirements. Moreover, the manufacturing process requires materials which may fluctuate in cost or be limited or unavailable to us, as well as relationships with contract development and manufacturing organizations to facilitate the manufacturing process. We may never succeed in any of these activities and, even if we do, we may never generate revenue that is significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our

business or continue our operations. A decline in the value of our company also could cause stockholders to lose all or part of their investment.

We will need additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, conduct clinical trials of, and seek marketing approval for our Candidates and otherwise integrate the operations of AavantiBio into our business. In addition, if we obtain marketing approval for our Candidates, we expect to incur significant expenses related to product sales, marketing, manufacturing and distribution. We also expect to continue to incur additional costs associated with operating as a public company. While we believe that our cash, cash equivalents and available-for-sale securities as of December 31, 2023, together with the net proceeds from our private placement that closed in January 2024, will be sufficient to fund our operating expenses and capital requirements into 2026, we have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently anticipate. In order to continue to operate our business beyond that time, we will need to raise additional funds. However, there can be no assurance that we will be able to generate funds on terms acceptable to us, on a timely basis, or at all. In addition, we anticipate that we will need additional funding to complete the development of our Candidates.

Our future capital requirements will depend on many factors, including:

- the progress, costs and results of the INSPIRE Duchenne trial and any future clinical trials of our Candidates;
- the costs, timing and outcome of regulatory review of our Candidates;
- the scope, progress, results and costs of discovery, laboratory testing, manufacturing, preclinical development and clinical trials for our Candidates;
- the costs associated with manufacturing and use of third-party manufacturers;
- the revenue, if any, received from commercial sale of our Candidates, should any of our future candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining, defending and enforcing our intellectual property rights and defending intellectual property-related claims;
- the outcome of any lawsuits filed against us;
- the terms of our current and any future license agreements and collaborations;
- the success of our collaboration with Ultragenyx;
- our ability to establish and maintain additional strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the payment or receipt of milestones, royalties and other collaboration-based revenues, if any;
- the extent to which we acquire or in-license other candidates, technologies and intellectual property; and
- if and as we need to adapt our business in response to public health emergencies or pandemics, such as the recent COVID-19 pandemic, and collateral consequences related thereto.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to submit a biologic license application, or BLA, or obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenue, if any, will be derived from or based on sales of product candidates that may not be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all, and may be impacted by the economic climate and market conditions. Our ability to raise additional funds may be adversely impacted by general economic conditions, both inside and outside the U.S., including disruptions to, and instability and volatility in, the credit and financial markets in the U.S. and worldwide, heightened inflation, interest rate and currency rate fluctuations, and economic slowdown or recession as well as concerns related to public health emergencies or pandemics, such as the recent COVID-19 pandemic, and geopolitical events, including civil or political unrest. In addition, market instability and volatility, high levels of inflation and interest rate fluctuations may increase our cost of financing or restrict our access to potential sources of future liquidity. Alternatively, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or Candidates.

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, ownership of our common stock will be diluted and the terms may include liquidation or other preferences that adversely affect the rights of our current stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and could involve restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or Candidates, or grant licenses on terms unfavorable to us.

We have never generated revenue from product sales and do not expect to do so for the foreseeable future, if ever.

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize our Candidates. We do not anticipate generating revenue from product sales for the foreseeable future, if ever. Our ability to generate future revenue from product sales depends heavily on our success in:

- completing research and development of our Candidates in a timely and successful manner;
- seeking and obtaining regulatory and marketing approvals for any of our Candidates for which we complete clinical trials;
- launching and commercializing Candidates for which we obtain regulatory and marketing approval by establishing a sales force and marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- maintaining and enhancing a commercially viable, sustainable, scalable, reproducible and transferable manufacturing processes for our Candidates that is compliant with cGMPs;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate, in terms of cost, amount and quality, products and services to support clinical development and the commercial demand for our Candidates, if approved;
- obtaining market acceptance, if and when approved, of our Candidates as a viable treatment option by patients, the medical community and third-party payors;
- qualifying for coverage and adequate reimbursement by government and third-party payors for our Candidates both in the U.S. and internationally;
- effectively addressing any competing technological and market developments;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations under such arrangements;

- maintaining, protecting, enforcing and expanding our portfolio of intellectual property rights, including patents, trademarks, trade secrets and know-how;
- avoiding and defending against intellectual property infringement, misappropriation and other claims;
- implementing additional internal systems and infrastructure, as needed; and
- attracting, hiring and retaining qualified personnel.

Our limited operating history may make it difficult for our stockholders to evaluate the success of our business to date and to assess our future viability.

Our operations to date, with respect to the development of SGT-001, which we are no longer developing, and SGT-003, have been limited to organizing and staffing our company, business planning, raising capital, acquiring rights to our technology, conducting research and development activities, establishing research and development collaborations, establishing arrangements for the manufacture of SGT-001 and SGT-003, identifying SGT-001 and SGT-003 as potential gene transfer candidates and undertaking preclinical studies and clinical trials of SGT-001 and SGT-003. Following the Acquisition, we have expanded our operations to include the development of additional Candidates. As a company, we have limited experience in clinical development and we have not yet demonstrated the ability to complete clinical trials of any product candidate, obtain marketing approvals, manufacture at commercial-scale or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions our stockholders make about our prospects may not be as accurate as they could be if we had a longer operating history or prior experience integrating acquired businesses into our existing business.

Public health emergencies or pandemics, including the recent COVID-19 pandemic, may affect our ability to initiate and complete current or future preclinical studies or clinical trials, disrupt regulatory activities, disrupt our manufacturing and supply chain or have other adverse effects on our business and operations.

Public health emergencies or pandemics, including the recent COVID-19 pandemic, could adversely affect our business, financial condition, results of operations, and prospects.

We and our third-party manufacturers for supply of drug product for our candidates, and prospective contract research organizations, or CROs, may face disruptions as a result of such pandemics that may affect our ability to initiate and complete preclinical studies or clinical trials, including disruptions in procuring items that are essential for our research and development activities, including, for example, raw materials used in the manufacturing of drug product for our candidates, and laboratory supplies for our current and future preclinical studies and clinical trials, in each case, for which there may be shortages because of ongoing efforts to address the outbreak. We and our third-party manufacturers, and prospective CROs, may face disruptions related to future clinical trials arising from delays in IND-enabling studies, manufacturing disruptions, and the ability to obtain necessary institutional review board or other necessary site approvals, as well as other delays at clinical trial sites.

We may also face difficulties recruiting or enrolling patients for our clinical trials if patients are affected by the recent COVID-19 pandemic or other public health emergencies or are fearful of visiting or traveling to, or unable to travel to, clinical trial sites. For example, we experienced a few missed or postponed patient visits in our IGNITE DMD trial for SGT-001, which we are no longer developing, due to site closures early in the COVID-19 pandemic.

The response to public health emergencies or pandemics may redirect resources with respect to regulatory and intellectual property matters in a way that would adversely impact our ability to progress regulatory approvals and protect our intellectual property. In addition, we may face impediments to regulatory meetings and approvals due to measures intended to limit in-person interactions.

While the public health emergency declarations related to COVID-19 ended on May 11, 2023 the FDA retained a number of COVID-19 related policies. It is unclear how, if at all, these policies will impact our efforts to develop and commercialize our product candidates.

Unfavorable global economic conditions could harm our business, financial condition or results of operations.

Our results of operations could be harmed by general conditions in the global economy and in the global financial markets. A severe or prolonged economic downturn, including the impact of increased interest rates and inflation (such as the recent rise in inflation in the United States), could result in a variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could strain our manufacturers, possibly resulting in manufacturing disruption, or cause delays in

payments for our services by third-party payors or our future collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could harm our business.

We hold a portion of our cash and cash equivalents that we use to meet our working capital and operating expense needs in deposit accounts that could be adversely affected if the financial institutions holding such funds fail.

We hold a portion of our cash and cash equivalents that we use to meet our working capital and operating expense needs in deposit accounts at multiple financial institutions. The balance held in these accounts may exceed the Federal Deposit Insurance Corporation, or FDIC, standard deposit insurance limit of $250,000. If a financial institution in which we hold such funds fails or is subject to significant adverse conditions in the financial or credit markets, we could be subject to a risk of loss of all or a portion of such uninsured funds or be subject to a delay in accessing all or a portion of such uninsured funds. Any such loss or lack of access to these funds could adversely impact our short-term liquidity and ability to meet our operating expense obligations, including payroll obligations.

For example, on March 10, 2023, Silicon Valley Bank, or SVB, and Signature Bank, were closed by state regulators and the FDIC was appointed receiver for each bank. The FDIC created successor bridge banks and all deposits of SVB and Signature Bank were transferred to the bridge banks under a systemic risk exception approved by the United States Department of the Treasury, the Federal Reserve and the FDIC. If financial institutions in which we hold funds for working capital and operating expenses were to fail, we cannot provide any assurances that such governmental agencies would take action to protect our uninsured deposits or investments in a similar manner.

We also maintain investment accounts with other financial institutions in which we hold our investments and marketable securities and, if access to the funds we use for working capital and operating expenses is impaired, we may not be able to sell investments or transfer funds from our investment accounts to other operating accounts on a timely basis sufficient to meet our operating expense obligations.

Risks related to the development of our product candidates

Our gene transfer candidates are based on novel technology, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. To our knowledge, only a limited number of gene transfer products have been approved for commercialization in the United States and the European Union.

We are evaluating SGT-003 for the treatment of Duchenne and are advancing a portfolio of programs for the treatment of other rare genetic diseases, and our future success depends on our successful development of these Candidates. Our risk of failure is high. We have experienced problems and delays in developing SGT-001, which we are no longer developing, and may in the future experience problems or delays in developing Candidates. Any such problems or delays would cause unanticipated costs, and any development problems may not be solved. For example, we or another party may uncover a previously unknown risk associated with our Candidates, the adeno-associated virus, or AAV, capsid, construct or other issues resulting in toxicity or lack of efficacy that may be more problematic than we currently believe and this may prolong the period of observation required for obtaining, or result in the failure to obtain, regulatory approval or may necessitate additional clinical testing.

In addition, our ability to conduct and complete our preclinical development testing and studies is contingent on our ability to source animals and other supplies required for the conduct of such testing and studies and the performance of animal models. If we are unable to obtain all necessary animals and other supplies required for the conduct of our preclinical testing and studies, or the animal models do not perform as expected, we may be unable to complete such preclinical development testing and studies in a timely manner or at all. For example, some of our IND-enabling toxicology and other studies require certain non-human primates, or NHPs, that may be imported from countries in which trade relation with the U.S. are or may become challenging or through vendors who may not be able to timely source certain NHPs or at all, which may impair our ability to complete preclinical development testing and studies to support IND or similar applications or delay submission of such applications. Additionally, we may fail to demonstrate adequate product candidate efficacy and/or safety as required by regulatory authorities. We may fail to access relevant, adequate, or necessary animal models, including genetic models of disease and non-human primates in particular, for use in such studies as requested by regulatory authorities. We may also experience substantial delays as a result of our reliance on CROs to conduct all animal model experimentation necessary to assess the efficacy and safety of our product candidates. Any of these factors may result in delays to candidate progression, inability to obtain regulatory approval, and/or substantial increases in candidate development costs.

In addition, the product specifications and the clinical trial requirements of the FDA, the European Commission, the European Medicines Agency, or the EMA, and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidate. The regulatory approval process for novel product candidates such as ours is unclear and can be more expensive and take longer than for other, better known or more extensively studied product candidates. To our knowledge, only a limited number of gene transfer products have been approved for commercialization in the United States and the European Union. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our gene transfer candidates in either the United States or the European Union, if at all. Approvals by the European Commission may not be indicative of what the FDA may require for approval and vice versa.

Our gene transfer candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.

Our current Candidates have not yet been studied in human patients. During the conduct of clinical trials, patients may experience changes in their health, including illnesses, injuries, discomforts or a fatal outcome. Often, it is not possible to determine whether the product candidate being studied caused these conditions. For instance, we reported a serious adverse event in IGNITE DMD, which resulted in a clinical hold in November 2019, which has since been resolved. In April 2021, a patient treated with SGT-001 in IGNITE DMD experienced a systemic inflammatory response classified as a serious adverse event and considered by the investigator to be drug related.

In addition, it is possible that as we test Candidates in larger, longer and more extensive clinical programs, or as use of these candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in earlier clinical trials, as well as conditions that did not occur or went undetected in previous clinical trials, will be reported by subjects. Many times, side effects are only detectable after investigational products are tested in large-scale, Phase 3 clinical trials or, in some cases, after they are made available to patients on a commercial scale after approval. If additional clinical experience indicates that a Candidate has side effects or causes serious or life-threatening side effects, the development of the candidate may fail or be delayed, or, if the candidate has received regulatory approval, such approval may be withdrawn.

There have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia and death seen in other clinical trials. The FDA convened the Cellular, Tissue, and Gene Therapies Advisory Committee in September 2021 to discuss toxicity risks of AAV based gene therapy products and discussed risks included oncogenicity risks due to capsid genome integration, hepatotoxicity, thrombotic microangiopathy, and neurotoxicity (especially related to dorsal root ganglion toxicity). While new recombinant capsids have been developed with the intent to reduce these side effects, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There have been reports of significant adverse side effects, including muscle weakness and myocarditis, in clinical trials of other gene therapy treatments for Duchenne that may be related to the type and location of the specific gene mutation causing the disease. One clinical trial sponsor reported a death, preceded by hypovolemia and cardiogenic shock, of a non-ambulatory Duchenne subject with advanced disease and cardiac dysfunction. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material. Possible adverse side effects that may occur with treatment with gene therapy products include an immunologic reaction early after administration that could substantially limit the effectiveness of the treatment or represent safety risks for patients. Additionally, in previous clinical trials involving AAV capsids for gene therapy, some subjects experienced the development of a positive ELISPOT test associated with T-cell responses, which is of unclear clinical translatability. If T-cells are activated, the cellular immune response system may trigger the removal of transduced cells. If our gene transfer candidates demonstrate a similar effect or other undesirable side effects, we may decide or be required to halt or delay further clinical development of our Candidates involving AAV capsids for gene therapy.

Adverse side effects may be observed following administration of any AAV gene therapy, including SGT-003 or other Candidates. Not all contemplated AAV delivery systems have been validated in human clinical trials previously, such as AAV-SLB101, which is a novel capsid. If a delivery system does not meet the safety criteria or cannot provide the desired efficacy results, then we may be forced to suspend or terminate our development of SGT-003 or other Candidates. The FDA convened the Cellular, Tissue, and Gene Therapies Advisory Committee in September 2021 to discuss toxicity risks of AAV based gene therapy products. Discussed risks included oncogenicity risks due to capsid genome integration, hepatotoxicity, thrombotic microangiopathy, and neurotoxicity (especially related to dorsal root ganglion toxicity). If any such adverse side effects were to occur in the future and we are unable to demonstrate that they were not caused by the administration process or related procedures, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, SGT-003 or other Candidates for any or all targeted indications. Even if we are

able to demonstrate that any serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the clinical trial. Patients will also create antibodies to the AAV capsid and a second administration of gene transfer might not be safe or successful.

Additionally, if one or more of our Candidates receive marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy, or REMS, to ensure that the benefits outweigh the risks, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by our Candidates, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such a product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

One of our prior clinical trials had been placed on clinical hold by the FDA in the past, and we cannot guarantee that similar events will not happen in future clinical trials for our Candidates.

In November 2019, the FDA placed a clinical hold on SGT-001 following a serious adverse event in IGNITE DMD. The third patient in the 2E14 vg/kg cohort of IGNITE DMD, dosed in late October 2019, experienced a serious adverse event deemed related to the study drug that was characterized by complement activation, thrombocytopenia, decrease in red blood cell count, acute kidney injury, and cardio-pulmonary insufficiency. In April 2021, an eighth patient was treated with SGT-001. The patient experienced a systemic inflammatory response which has since fully resolved. The event was classified as a serious adverse event and considered by the investigator to be drug related. While SGT-003 utilizes a different capsid than SGT-001 and includes other changes to the construct and manufacturing process to help avoid or mitigate any such events, we cannot guarantee that similar serious adverse events or clinical holds will not happen in future clinical trials.

Delays in the completion of, or our inability to conduct, any clinical trial of SGT-003 or any other Candidate, as a result of similar serious adverse events or clinical holds or otherwise, will increase our costs, slow down or cease our product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of SGT-003 or other Candidates.

We have never completed a clinical trial and may be unable to do so for any product candidate, including SGT-003 and other Candidates.

We are early in our development efforts and we have never completed a clinical trial. We anticipate dosing our first patient in our INSPIRE Duchenne trial in the second quarter of 2024. All of our other Candidates are still in preclinical development. Preclinical studies involve a lengthy and expensive process with an uncertain outcome. There are many potential preclinical models to test for different disease states, and we could fail to choose the best or a predictive preclinical model to determine proof of concept and potential safety and efficacy of our candidates. We may decide to suspend further testing on our candidates or technologies if, in the judgment of our management and advisors, the preclinical test results do not support further development.

We will need to successfully initiate our planned and complete our ongoing clinical trials in order to obtain FDA approval to market SGT-003 and other Candidates. We have limited experience in preparing, submitting and prosecuting regulatory submissions, and have not previously submitted a BLA for any product candidate. We cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin or to begin as proposed, or that, once begun, issues will not arise that suspend or terminate such clinical trials. Carrying out later-stage clinical trials and the submission of a successful BLA is a complicated process. This may be particularly true for design of a pivotal trial for the treatment of Duchenne as the FDA has not given clear guidance as to the necessary endpoints for approval of a treatment for Duchenne. In addition, we cannot be certain how many clinical trials of SGT-003 or other Candidates will be required or how such trials should be designed. Consequently, we may be unable to successfully and efficiently execute and complete necessary clinical trials in a way that leads to BLA submission and approval of SGT-003 or other Candidates. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of product candidates that we develop. Failure to commence or complete, or delays in, clinical trials, could prevent us from or delay us in commercializing SGT-003 and other Candidates.

Success in preclinical studies or early clinical trials may not be indicative of results obtained in later trials.

Results from preclinical studies or early clinical trials are not necessarily predictive of future clinical trial results and are not necessarily indicative of final results. Our preclinical studies for certain Candidates in animals have been limited. We have not dosed human subjects with SGT-003 or any of our other Candidates. There is a high failure rate for gene therapy and biologic products proceeding through clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. We also may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our product candidate development. Our Candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies. This failure could cause us to abandon any of our Candidates.

Preliminary or interim data that we announce or publish from time to time may change as more data becomes available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may announce or publish preliminary or interim data from clinical trials. Positive preliminary or interim data may not be predictive of such trial's subsequent or overall results. Preliminary or interim data are subject to the risk that one or more of the outcomes may materially change as more data becomes available. Additionally, preliminary or interim data are subject to the risk that one or more of the biologic or clinical outcomes may materially change as patient enrollment continues and more patient data becomes available. Therefore, positive preliminary or interim data in any ongoing clinical trial may not be predictive of such results in the completed trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully evaluate all data. As a result, preliminary or interim data that we report may differ from future results from the clinical trials, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Preliminary or interim data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary or interim data we previously published. As a result, preliminary or interim data should be viewed with caution until the final data are available. Material adverse changes in the final data compared to preliminary or interim data could significantly harm our business prospects.

We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of our Candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidate for its intended indications. Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in obtaining animals in sufficient quantities to run our preclinical studies;
- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement with the appropriate external parties on dose escalation;
- delays in enrolling patients in clinical trials;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in opening clinical trial sites or obtaining required IRB or independent ethics committee approval at each clinical trial site;
- delays in recruiting suitable subjects to participate in our clinical trials, including because such trials have restrictive eligibility criteria or may be placebo-controlled trials and patients are not guaranteed to receive treatment with our product candidates, or as a result of alternative therapies or competing trials;
- difficulty in finding suitable animal models to demonstrate a disease specific phenotype;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with FDA good clinical practices, or GCPs, or applicable regulatory guidelines in the European Union and other countries;

- delays in the testing, validation, manufacturing and delivery of our Candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- delays in subjects completing participation in a trial or returning for post-treatment follow-up;
- clinical trial sites or subjects dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event, or after an inspection of our clinical trial operations, trial sites or manufacturing facilities or otherwise;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors;
- delays as a result of public health emergencies or pandemics, such as the recent COVID-19 pandemic or from the outbreak of another pandemic or contagious disease or other global instability could delay the initiation or rate of completion of any clinical trial; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Additionally, if the results of any clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our Candidates, we may:

- interrupt or halt clinical development;
- be delayed or fail in obtaining marketing approval for our Candidates;
- obtain approval for indications or patient populations that are not as broad as we intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way our products, if approved, are administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a modified REMS;
- be sued and held liable for harm caused to patients; or
- experience damage to our reputation.

In addition, the FDA's and other regulatory authorities' policies with respect to clinical trials may change and additional government regulations may be enacted. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted. For example, in December 2022, with the passage of Food and Drug Omnibus Reform Act, or FDORA, Congress required sponsors to develop and submit a diversity action plan for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. Similarly, the regulatory landscape related to clinical trials in the EU recently evolved. If we are not able to adapt to these and other changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted.

Our product development costs will increase if we experience delays in testing or marketing approvals. In addition, if we make manufacturing or other changes to our Candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. We may also determine to change the design or protocol of one or more of our clinical trials, which we have done in the past and which could result in delays. Significant preclinical study or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates.

If our third-party clinical trial vendors fail to comply with strict regulations, any clinical trials for our Candidates may be delayed or unsuccessful.

We do not have the personnel capacity to conduct or manage the clinical trials that will be necessary for the development of our Candidates. For our INSPIRE Duchenne trial we are relying, and for any future clinical trials we expect we will rely, on third parties to assist us in managing, monitoring and conducting our clinical trials. If these third parties fail to comply with applicable regulations or do not adequately fulfill their obligations under the terms of our agreements with them, we may not be able to enter into alternative arrangements without undue delay or additional expenditures and, therefore, clinical trials for SGT-003 or other Candidates may be delayed or unsuccessful.

Furthermore, the FDA can be expected to inspect some or all of the clinical sites participating in our clinical trials to determine if our clinical trials are being conducted according to GCPs. If the FDA determines that these clinical sites are not in compliance with applicable regulations, we may be required to delay, repeat or terminate the clinical trials.

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent us from proceeding with clinical trials of SGT-003 or other Candidates.

Identifying and qualifying patients to participate in any clinical trials of SGT-003 and other Candidates are critical to our success. Because of our primary focus on rare diseases, we may have difficulty enrolling a sufficient number of eligible patients. The timing of any clinical trials depends on our ability to recruit patients to participate as well as complete required follow-up periods. If patients are unwilling or unable to participate in our gene therapy clinical trials, including because of negative publicity from adverse events related to our candidates, other approved therapies, or due to competitive clinical trials or approvals for similar patient populations, clinical trials in products employing our capsid or our platform or for other reasons, the timeline for recruiting patients, conducting clinical trials and obtaining regulatory approval of SGT-003 or other Candidates may be delayed. We may also experience delays if patients withdraw from the clinical trial or do not complete the required monitoring period. Furthermore, we may face difficulties in recruiting patients to enroll in, or once enrolled, retaining patients in future clinical trials if they or their caretakers are affected by public health emergencies or pandemics, such as the recent COVID-19 virus or are fearful of traveling to, or are unable to travel to, our clinical trial sites because of public health emergencies or pandemics or other unforeseen events. These delays could result in increased costs, delays in advancing SGT-003 or other Candidates, delays in testing the effectiveness of our Candidates or termination of clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete any clinical trials in a timely manner. Patient enrollment and trial completion is affected by many factors, including:

- size of the patient population and the process for identifying subjects;
- design of the trial protocol;
- eligibility and exclusion criteria, including age, size and functional ability and pre-existing antibodies to AAV capsids that preclude subjects from being able to receive AAV-mediated gene transfer;
- restrictions on our ability to conduct clinical trials, including full and partial clinical holds on ongoing or planned clinical trials;
- perceived risks and benefits of the product candidate under study;
- perceived risks and benefits of gene therapy-based approaches to the treatment of diseases;
- release or disclosure of data from our completed or ongoing clinical trials;
- availability of competing therapies and clinical trials;
- severity of the disease;
- proximity and availability of clinical trial sites for prospective subjects;
- ability to obtain and maintain subject consent;
- risk that enrolled subjects will drop out before completion of the trial;
- patient referral practices of physicians;
- ability to monitor subjects adequately during and after treatment; and
- in the case of pivotal trials, the risk that patients may opt not to enroll because they are not assured treatment with our product candidate.

If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted. For example, in December 2022, with the passage of FDORA, Congress required sponsors to develop and submit a diversity action plan for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. Similarly, the regulatory landscape related to clinical trials in the EU recently evolved. The EU Clinical Trials Regulation, or CTR, which was adopted in April 2014 and repeals the EU Clinical Trials Directive, became applicable on January 31, 2022. The CTR aims to simplify and streamline the authorization, conduct and transparency of clinical trials in the EU. We have not previously secured authorization to conduct clinical studies in the European Union pursuant to the CTR and, accordingly, there is a risk that we may be delayed in commencing such studies. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted.

Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- different standards for the conduct of clinical trials;
- absence in some countries of established groups with sufficient regulatory expertise for review of gene therapy protocols;
- difficulty in identifying and partnering with qualified local consultants, physicians and partners; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology research and products.

Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize our Candidates and the approval may be for a more narrow indication than we seek.

We cannot commercialize our Candidates until the appropriate regulatory authorities have reviewed and approved the product candidate. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state and local statutes and regulations require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals. Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA advisory committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action or changes in regulatory authority policy during the period of product development, clinical trials and the regulatory review process.

Further, under the Pediatric Research Equity Act of 2003, a BLA or supplement to a BLA for certain biological products must contain data to assess the safety and effectiveness of the biological product in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective, unless the sponsor receives a deferral or waiver from the FDA. A deferral may be granted for several reasons, including a finding that the product or therapeutic candidate is ready for approval for use in adults before pediatric trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric trials begin. The applicable legislation in the European Union also requires sponsors to either conduct clinical trials in a pediatric population in accordance with a Pediatric Investigation Plan approved by the Pediatric Committee of the EMA or to obtain a waiver or deferral from the conduct of these studies by this Committee. For any of our product candidates for which we are seeking regulatory approval in the U.S. or the European Union, we cannot guarantee that we will be able to obtain a waiver or alternatively complete any required studies and other requirements in a timely manner, or at all, which could result in associated reputational harm and subject us to enforcement action.

Even if we receive regulatory approval, regulatory authorities may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or a REMS. Regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

Even if we obtain regulatory approval for a product candidate, our product candidates will remain subject to regulatory oversight.

Even if we obtain any regulatory approval for any of our Candidates, we will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. Any regulatory approvals that we receive for our product candidates may also be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or conditions of approval, or requirements for potentially costly post-marketing testing and surveillance to monitor the safety, purity, and potency of the biologic product. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may have various consequences, including:

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions and warnings on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- damage to relationships with any potential collaborators;
- unfavorable press coverage and damage to our reputation;
- refusal to permit the import or export of our products;
- product seizure;
- injunctions or the imposition of civil or criminal penalties; or
- litigation involving patients using our products.

In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMPs applicable to drug manufacturers or quality assurance standards applicable to medical device manufacturers, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, any contract manufacturers we may engage in the future, our future collaborators and their contract manufacturers will also be subject to other regulatory requirements, including submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements regarding the distribution of samples to clinicians, recordkeeping, and costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product such as the requirement to implement a REMS.

Finally, our ability to develop and market new drug products may be impacted by ongoing litigation challenging the FDA's approval of mifepristone. Specifically, in April 2023, the U.S. District Court for the Northern District of Texas stayed the approval by the FDA of mifepristone, a drug product which was originally approved in 2000 and whose distribution is governed by various conditions adopted under a REMS. In reaching that decision, the district court made a number of findings that may negatively impact the development, approval and distribution of drug products in the U.S. Among other determinations, the district court held that plaintiffs were likely to prevail in their claim that FDA had acted arbitrarily and capriciously in approving mifepristone without sufficiently considering evidence bearing on whether the drug was safe to use under the conditions identified in its labeling. Further, the district court read the standing requirements governing litigation in federal court as permitting a plaintiff to bring a lawsuit against the FDA in connection with its decision to approve an new drug application or establish requirements under a REMS based on a showing that the plaintiff or its members would be harmed to the extent that FDA's drug approval decision effectively compelled the plaintiffs to provide care for patients suffering adverse events caused by a given drug.

On April 12, 2023, the district court decision was stayed, in part, by the U.S. Court of Appeals for the Fifth Circuit. Thereafter, on April 21, 2023, the U.S. Supreme Court entered a stay of the district court's decision, in its entirety, pending disposition of the appeal of the district court decision in the Court of Appeals for the Fifth Circuit and the disposition of any petition for a writ of certiorari to or the Supreme Court. The Court of Appeals for the Fifth Circuit held oral argument in the case on May 17, 2023 and, on August 16, 2023, issued its decision. The court declined to order the removal of mifepristone from the market, finding that a challenge to the FDA's initial approval in 2000 is barred by the statute of limitations. But the Appeals Court did hold that plaintiffs were likely to prevail in their claim that changes allowing for expanded access of mifepristone that FDA authorized in 2016 and 2021 were arbitrary and capricious. On September 8, 2023, the Justice Department and a manufacturer of mifepristone filed petitions for a writ of certiorari, requesting that asked the U.S. Supreme Court to review the Appeals Court decision. On December 13, 2023, the Supreme Court granted these petitions for writ of certiorari for the appeals court decision.

Non-compliance with European Union requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also lead to significant penalties and sanctions. Further, similar restrictions apply to approved products in the EU. The holder of a marketing authorization is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. These include: compliance with the EU's stringent pharmacovigilance or safety reporting rules, which can impose post-authorization studies and additional monitoring obligations; the manufacturing of authorized medicinal products, for which a separate manufacturer's license is mandatory; and the marketing and promotion of authorized drugs, which are strictly regulated in the EU and are also subject to EU Member State laws.

Accordingly, assuming we, or our collaborators, receive marketing approval for one or more of our product candidates, we, and our collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control. If we, and our collaborators, are not able to comply with post-approval regulatory requirements, our or our collaborators' ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Any regulatory approval to market our products will be limited by indication. If we fail to comply or are found to be in violation of FDA regulations restricting the promotion of our products for unapproved uses, we could be subject to criminal penalties, substantial fines or other sanctions and damage awards.

The regulations relating to the promotion of products for unapproved uses are complex and subject to substantial interpretation by the FDA, EMA, Medicines and Healthcare products Regulatory Agency and other government agencies. In September 2021, the FDA published final regulations which describe the types of evidence that the agency will consider in determining the intended use of a drug product. Physicians may nevertheless prescribe our products off-label to their patients in a manner that is inconsistent with the approved label. We intend to implement compliance and training programs designed to ensure that our sales and marketing practices comply with applicable regulations. Notwithstanding these programs, the FDA or other government agencies may allege or find that our practices constitute prohibited promotion of our products for unapproved uses. We also cannot be sure that our employees will comply with company policies and applicable regulations regarding the promotion of products for unapproved uses.

Notwithstanding the regulatory restrictions on off-label promotion, the FDA and other regulatory authorities allow companies to engage in truthful, non-misleading, and non-promotional scientific communications concerning their products in certain circumstances. For example, in October 2023, the FDA published draft guidance outlining the agency's non-binding policies governing the distribution of scientific information on unapproved uses to healthcare providers. This draft guidance calls for such communications to be truthful, non-misleading, factual, and unbiased and include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use. In addition, under some relatively recent guidance from the FDA and the Pre-Approval Information Exchange Act, or PIE Act, signed into law as part of the Consolidated Appropriations Act of 2023, companies may also promote information that is consistent with the prescribing information and proactively speak to formulary committee members of payors regarding data for an unapproved drug or unapproved uses of an approved drug. We may engage in these discussions and communicate with healthcare providers, payors and other constituencies in compliance with all applicable laws, regulatory guidance and industry best practices. We will need to carefully navigate the FDA's various regulations, guidance and policies, along with recently enacted legislation, to ensure compliance with restrictions governing promotion of our products.

In recent years, a significant number of pharmaceutical and biotechnology companies have been the target of inquiries and investigations by various federal and state regulatory, investigative, prosecutorial and administrative entities in connection with the promotion of products for unapproved uses and other sales practices, including the Department of Justice and various U.S. Attorneys' Offices, the Office of Inspector General of the Department of HHS, the FDA, the Federal Trade Commission, or the FTC, and various state Attorneys General offices. These investigations have alleged violations of various federal and state laws and regulations, including claims asserting antitrust violations, violations of the FD&C Act, the False Claims Act, the Prescription Drug Marketing Act and anti-kickback laws and other alleged violations in connection with the promotion of products for unapproved uses, pricing and Medicare and/or Medicaid reimbursement. Many of these investigations originate as "*qui tam*" actions under the False Claims Act. Under the False Claims Act, any individual can bring a claim on behalf of the government alleging that a person or entity has presented a false claim or caused a false claim to be submitted to the government for payment. The person bringing a *qui tam* suit is entitled to a share of any recovery or settlement. *Qui tam* suits, also commonly referred to as "whistleblower suits," are often brought by current or former employees. In a *qui tam* suit, the government must decide whether to intervene and prosecute the case. If it declines, the individual may pursue the case alone.

If the FDA or any other governmental agency initiates an enforcement action against us or if we are the subject of a *qui tam* suit and it is determined that we violated prohibitions relating to the promotion of products for unapproved uses, we could be subject to substantial civil or criminal fines or damage awards and other sanctions such as consent decrees and corporate integrity agreements pursuant to which our activities would be subject to ongoing scrutiny and monitoring to ensure compliance with applicable laws and regulations. Any such fines, awards or other sanctions would have an adverse effect on our revenue, business, financial prospects and reputation.

Even if we obtain and maintain approval of one or more of our Candidates from the FDA, we may never obtain approval for our product candidates outside of the United States, which would limit our market opportunities and adversely affect our business.

Even if we receive FDA approval of one or more of our Candidates in the United States, approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Future sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials, manufacturing and marketing approval. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. If we submit a marketing authorization application, or MAA, to the EMA for approval of SGT-003 or other Candidates in the European Union, obtaining such approval from the European Commission following the opinion of the EMA is a lengthy and expensive process. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our Candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for our Candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced, and our ability to realize the full market potential of our product candidates will be harmed.

Additionally, we could face heightened risks with respect to obtaining marketing authorization in the United Kingdom as a result of the withdrawal of the United Kingdom from the European Union, commonly referred to as Brexit. The United Kingdom is no longer part of the European Single Market and EU Customs Union. As of January 1, 2021, the MHRA became responsible for supervising medicines and medical devices in Great Britain, comprising England, Scotland and Wales under domestic law, whereas under the terms of the Northern Ireland Protocol, Northern Ireland is currently subject to European Union rules. The United Kingdom and European Union have however agreed to the Windsor Framework which fundamentally changes the existing system under the Northern Ireland Protocol, including with respect to the regulation of medicinal products in the United Kingdom. Once implemented, the changes introduced by the Windsor Framework will see the MHRA be responsible for approving all medicinal products destined for the UK market (i.e., Great Britain and Northern Ireland), and the EMA will no longer have any role in approving medicinal products destined for Northern Ireland. Any delay in obtaining, or an inability to obtain, any marketing authorizations, as a result of Brexit or otherwise, may force us to restrict or delay efforts to seek regulatory approval in the United Kingdom for our product candidates, which could significantly and materially harm our business.

In addition, foreign regulatory authorities may change their approval policies and new regulations may be enacted. For instance, the EU pharmaceutical legislation is currently undergoing a complete review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission's proposal for revision of several legislative instruments related to medicinal products (potentially reducing the duration of regulatory data protection, revising the eligibility for expedited pathways, etc.) was published on April 26, 2023. The proposed revisions remain to be agreed and adopted by the European Parliament and European Council and the proposals may therefore be substantially revised before adoption, which is not anticipated before early 2026. The revisions may however have a significant impact on the pharmaceutical industry and our business in the long term.

We expect that we will be subject to additional risks in commercializing any of our product candidates that receive marketing approval outside the United States, including tariffs, trade barriers and regulatory requirements; economic weakness, including inflation, or political instability in particular foreign economies and markets; compliance with tax, employment, immigration and labor laws for employees living or traveling abroad; foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country; and workforce uncertainty in countries where labor unrest is more common than in the United States.

Regulatory requirements governing gene therapy products are periodically updated and may continue to change in the future.

Regulatory requirements governing gene therapy products have changed frequently and will likely continue to change in the future. Moreover, there is substantial, and sometimes uncoordinated, overlap in those responsible for regulation of gene therapy products. For example, in the United States, the FDA has established the Office of Therapeutic Products within the Center for Biologics Evaluation and Research, or the CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical trials may also be subject to review and oversight by an institutional biosafety committee, a local institutional committee that reviews and oversees basic and clinical research conducted at the institution participating in the clinical trial. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation.

The FDA has issued various guidance documents regarding gene therapies, including final guidance documents released in January 2020 relating to chemistry, manufacturing and controls information for gene therapy INDs, gene therapies for rare diseases and gene therapies for retinal disorders, a final guidance in October 2022 for Human Gene Therapy for Neurodegenerative Diseases, as well as a draft guidance in July 2023 on comparability requirements for manufacturing changes in gene therapy products. In December 2023, a draft guidance on potency assurance for cellular and gene therapy products was released. Although the FDA has indicated that these and other guidance documents it previously issued are not legally binding, we believe that our compliance with them is likely necessary to gain approval for any gene therapy product candidate we may develop. The guidance documents provide additional factors that the FDA will consider at each of the above stages of development and relate to, among other things, the proper preclinical assessment of gene therapies; the chemistry, manufacturing, and control information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, for AAV capsids specifically, the FDA typically recommends that sponsors continue to monitor participants for potential gene therapy-related adverse events for up to a 5-year period. Other types of gene therapy or gene editing products may require longer follow up, potentially up to a maximum 15-year period.

Similarly, the EMA may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines. The grant of marketing authorization in the European Union for gene therapy products is governed by Regulation 1394/2007/EC on advanced therapy medicinal products, read in

combination with Directive 2001/83/EC of the European Parliament and of the Council, commonly known as the Community code on medicinal products. Regulation 1394/2007/EC includes specific rules concerning the authorization, supervision, and pharmacovigilance of gene therapy medicinal products. Manufacturers of advanced therapy medicinal products must demonstrate the quality, safety, and efficacy of their products to the EMA, which provides an opinion regarding the MAA. The European Commission grants or refuses marketing authorization in light of the opinion delivered by the EMA.

Finally, ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed their intentions to further regulate biotechnology. More restrictive regulations or claims that our product candidates are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

As we advance our product candidates through clinical development, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. These regulatory review committees and advisory groups and any new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of product candidates or lead to significant post-approval limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue.

We may not be able to obtain orphan drug exclusivity for one or more of our product candidates, and even if we do, that exclusivity may not prevent the FDA or the EMA from approving other competing products.

Under the Orphan Drug Act, the FDA may designate a product candidate as an orphan drug if it is a drug or biologic intended to treat a rare disease or condition. A similar regulatory scheme governs approval of orphan products by the EMA in the European Union. Generally, if a product candidate with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for a similar product for the same therapeutic indication for that time period. The applicable period is seven years in the United States and ten years in the European Union. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation, in particular if the product is sufficiently profitable so that market exclusivity is no longer justified.

The FDA has granted orphan drug designation to SGT-003 for the treatment of Duchenne and the FDA and EMA have granted orphan drug designation to SGT-501 for the treatment of CPVT.

In order for the FDA to grant orphan drug exclusivity to one of our products, the FDA must find that the product is indicated for the treatment of a condition or disease with a patient population of fewer than 200,000 individuals annually in the United States. The FDA may conclude that the condition or disease for which orphan drug exclusivity is sought does not meet this standard. Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different products can be approved for the same condition.

In addition, under the FDA September 2021 guidance for interpreting sameness of gene therapy products under the orphan drug regulations, even after an orphan drug is approved, the FDA can subsequently approve a similar product for the same condition if the FDA concludes that the later product is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity may also be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of the patients with the rare disease or condition.

The FDA Reauthorization Act of 2017, or FDARA, requires that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. FDARA reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority.

The FDA and Congress may further reevaluate the Orphan Drug Act and its regulations and policies. This may be particularly true in light of a decision from the Court of Appeals for the 11th Circuit in September 2021 finding that, for the purpose of determining the scope of exclusivity, the term "same disease or condition" means the designated "rare disease or condition" and could not be interpreted by the FDA to mean the "indication or use." Thus, the Court of Appeals concluded that orphan drug exclusivity applies to the entire designated disease or condition rather than the "indication or use." Although there have been legislative proposals to overrule this decision, they have not been enacted into law. In January 2023, the FDA

announced that, in matters beyond the scope of that court order, FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug was approved. We do not know if, when, or how the FDA or Congress may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA or Congress may make to its orphan drug regulations and policies, our business could be adversely impacted.

We may seek a breakthrough therapy designation for one or more of our product candidates, but we might not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.

We may seek a breakthrough therapy designation for one or more of our product candidates; however, we cannot assure our stockholders that one or more of our product candidates will meet the criteria for that designation. A breakthrough therapy is defined as a therapy that is intended, alone or in combination with one or more other therapies, to treat a serious condition, and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For therapies and biologics that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Therapies designated as breakthrough therapies by the FDA may also be eligible for priority review if supported by clinical data at the time the biologics license application is submitted to the FDA.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. Even if we receive breakthrough therapy designation, the receipt of such designation for a product candidate may not result in a faster development or regulatory review or approval process compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualifies as a breakthrough therapy, the FDA may later decide that the product candidate no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Accelerated approval by the FDA, even if granted for one or more of our product candidates, may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek approval of one or more of our product candidates using the FDA's accelerated approval pathway. A product may be eligible for accelerated approval if it treats a serious or life-threatening condition and generally provides a meaningful advantage over available therapies. In addition, it must demonstrate an effect on a surrogate or intermediate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. The FDA or other applicable regulatory agency makes the determination regarding whether a surrogate or intermediate endpoint is reasonably likely to predict long-term clinical benefit. Given that expression of microdystrophin has not yet been established to predict long-term clinical benefit, it is not currently accepted, and it is possible the FDA and/or other applicable regulatory agencies could decide never to accept it, as a surrogate endpoint for the accelerated approval pathway for the treatment of Duchenne.

As a condition of approval, the FDA may require that a sponsor of a drug or biologic product candidate receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. These confirmatory trials must be completed with due diligence and may be required to be initiated prior to submission of the BLA. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. Further, with passage of FDORA in December 2022, Congress modified certain provisions governing accelerated approval of drug and biologic products. Specifically, the new legislation authorized the FDA to: require a sponsor to have its confirmatory clinical trial underway before accelerated approval is awarded, require a sponsor of a product granted accelerated approval to submit progress reports on its post-approval studies to FDA every six months (until the study is completed) and use expedited procedures to withdraw accelerated approval of a BLA after the confirmatory trial fails to verify the product's clinical benefit.

There can be no assurance that the FDA or comparable foreign regulatory agencies will agree with our surrogate endpoints or intermediate clinical endpoints in any of our clinical trials, or that we will decide to pursue or submit any additional application for accelerated approval or any other form of expedited development, review or approval. Similarly, there can be no assurance that, after feedback from the FDA or comparable foreign regulatory agencies, we will continue to pursue or apply for accelerated approval or any other form of expedited development, review or approval. Furthermore, for any submission of an application for accelerated approval or application under another expedited regulatory designation, there can be no assurance that such submission or application will be accepted for filing or that any expedited development, review or approval will be granted on a timely basis, or at all.

A failure to obtain accelerated approval or any other form of expedited development, review or approval for our product candidates, or withdrawal of a product candidate, would result in a longer time period until commercialization of such product candidate, could increase the cost of development of such product candidate and could harm our competitive position in the marketplace.

A potential regenerative medicine advanced therapy designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek a regenerative medicine advanced therapy designation for some of our product candidates. A regenerative medicine advanced therapy is defined as cell therapies, therapeutic tissue engineering products, human cell and tissue products, and combination products using any such therapies or products. Gene therapies, including genetically modified cells, that lead to a durable modification of cells or tissues may meet the definition of a regenerative medicine therapy. The regenerative medicine advanced therapy program is intended to facilitate efficient development and expedite review of regenerative medicine advanced therapies, which are intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition. A BLA for a regenerative medicine advanced therapy may be eligible for priority review or accelerated approval through (1) surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit or (2) reliance upon data obtained from a meaningful number of sites. Benefits of such designation also include early interactions with the FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. A regenerative medicine therapy that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post-approval monitoring of all patients treated with such therapy prior to its approval.

Designation as a regenerative medicine advanced therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a regenerative medicine advanced therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a regenerative medicine advanced therapy designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as regenerative medicine advanced therapies, the FDA may later decide that the biological products no longer meet the conditions for qualification.

We may seek PRIME Designation in the EU for one or more of our product candidates, but we might not receive such designations and, even if we do, such designations may not lead to a faster development or regulatory review or approval process.

In the EU, we may seek PRIME designation for our product candidates in the future. PRIME is a voluntary program aimed at enhancing the EMA's role to reinforce scientific and regulatory support in order to optimize development and enable accelerated assessment of new medicines that are of major public health interest with the potential to address unmet medical needs. The program focuses on medicines that target conditions for which there exists no satisfactory method of

treatment in the EU or even if such a method exists, it may offer a major therapeutic advantage over existing treatments. PRIME is limited to medicines under development and not authorized in the EU and the sponsor intends to apply for an initial marketing authorization application through the centralized procedure. To be accepted for PRIME, a product candidate must meet the eligibility criteria in respect of its major public health interest and therapeutic innovation based on information that is capable of substantiating the claims.

The benefits of a PRIME designation include the appointment of a Committee for Medicinal Products for Human Use rapporteur to provide continued support and help to build knowledge ahead of a marketing authorization application, early dialogue and scientific advice at key development milestones, and the potential to qualify products for accelerated review, meaning reduction in the review time for an opinion on approvability to be issued earlier in the application process. PRIME enables a sponsor to request parallel EMA scientific advice and health technology assessment advice to facilitate timely market access. Even if we receive PRIME designation for any of our product candidates, the designation may not result in a materially faster development process, review or approval compared to conventional EMA procedures. Further, obtaining PRIME designation does not assure or increase the likelihood of EMA's grant of a marketing authorization.

We may seek a Rare Pediatric Disease Designation for our product candidates. However, a BLA for such product candidates may not meet the eligibility criteria for a priority review voucher upon approval.

With enactment of the Food and Drug Administration Safety and Innovation Act in 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease product applications that meet the criteria specified in the law. This provision is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application.

In order to receive a priority review voucher upon BLA approval, the product must receive designation from the FDA as a product for a rare pediatric disease prior to approval of the marketing application. A "rare pediatric disease" is a disease that is serious or life-threatening, in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and affects fewer than 200,000 people in the United States, or affects more than 200,000 people in the United States but there is no reasonable expectation that the cost of developing and making available in the United States a product for such disease or condition will be recovered from sales in the United States of such product. In addition to receiving rare pediatric disease designation, in order to receive a priority review voucher, the BLA must be given priority review, rely on clinical data derived from studies examining a pediatric population and dosages of the product intended for that population, not seek approval for a different adult indication in the original rare pediatric disease product application and be for a product that does not include a previously approved active ingredient.

Under the current statutory sunset provisions for the Rare Pediatric Disease Priority Review Voucher Program, after September 30, 2024, FDA may only award a voucher for an approved rare pediatric disease product application if the sponsor has rare pediatric disease designation for the drug, and that designation was granted by September 30, 2024. After September 30, 2026, FDA may not award any rare pediatric disease priority review vouchers. If we do not obtain approval of a BLA by these dates, and if the Rare Pediatric Disease Priority Review Voucher Program is not further extended by congressional action, we may not receive a Priority Review Voucher.

We may seek a fast track designation for one or more of our product candidates. However, such designation may not actually lead to a faster development or regulatory review or approval process. We might not receive such designation for one or more of our product candidates.

If a therapy is intended for the treatment of a serious condition and nonclinical or clinical data demonstrates the potential to address unmet medical need for this condition, a drug sponsor may apply for FDA fast track designation. However, fast track designation does not ensure that we will receive marketing approval or that approval will be granted within any particular timeframe. The FDA has broad discretion with respect to whether or not to grant fast track designation to a product candidate, so even if we believe a particular product candidate is eligible for such designation, the FDA may decide not to grant it. Moreover, we may not experience a faster development or regulatory review or approval process with fast track designation compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program or if the unmet need has been fulfilled with the approval of another product. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

The FDA has granted fast track designation to SGT-003 for the treatment of Duchenne.

We may seek priority review designation for one or more of our product candidates, but we might not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. We may request priority review for our product candidates, however, we cannot assume that one or more of our product candidates will meet the criteria for that designation. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster development or regulatory review or approval process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all.

Inadequate funding for the FDA, the SEC and other U.S. or foreign government agencies, including from government shut downs, or other disruptions to these agencies' operations, could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA, EMA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, in recent years, including 2018 and 2019, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical employees and stop critical activities. In addition, disruptions may result in events similar to the COVID-19 pandemic. During the COVID-19 pandemic, a number of companies announced receipt of complete response letters due to the FDA's inability to complete required inspections for their applications. In the event of a similar public health emergency in the future, the FDA may not be able to continue its current pace and review timelines could be extended. Regulatory authorities outside the United States facing similar circumstances may adopt similar restrictions or other policy measures in response to a similar public health emergency and may also experience delays in their regulatory activities.

If a prolonged government shutdown or other disruption occurs, it could significantly impact the ability of the FDA, EMA or other regulatory agency to review and process our regulatory submissions in a timely manner, which could have a material adverse effect on our business. Further, future government shutdowns or other disruptions affect other government agencies such as the SEC, which may also impact our business by delaying review of our public filings, to the extent such review is necessary and could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

We face significant competition and our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may adversely affect our ability to develop, successfully market, or commercialize our Candidates.

We operate in a highly competitive segment of the biopharmaceutical market. We face competition from many different sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions, government agencies and private and public research institutions. Our product candidates, if successfully developed and approved, will compete with established therapies as well as with new treatments that may be introduced by our competitors. There are a variety of product candidates, including gene therapies, in development for Duchenne, CPVT, other cardiomyopathies or FA. Many of our competitors have significantly greater

financial, product candidate development, manufacturing and marketing resources than we do. Large pharmaceutical and biotechnology companies have extensive experience in clinical testing and obtaining regulatory approval for their products, and mergers and acquisitions within these industries may result in even more resources being concentrated among a smaller number of larger competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, enrolling patients in clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

We are aware of a number of companies and research institutions developing gene transfer programs progressing in Duchenne. For example, in June 2023, Sarepta Therapeutics, Inc., or Sarepta, announced that it had received accelerated approval for its gene therapy candidate ELEVIDYS for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne. In December 2023, Sarepta announced that it submitted a supplemental BLA to broaden the approved indication for ELEVIDYS to all patients (all ages and ambulation status) with Duchenne, and on February 16, 2024 Sarepta announced that the FDA accepted the supplemental BLA for priority review and set a PDUFA date of June 21, 2024. We are also aware of several companies and research institutions conducting clinical trials of product candidates focused on systemic gene transfers for Duchenne, including Pfizer Inc. with a product candidate currently in Phase 3 clinical development, Genethon with a product candidate currently being evaluated in a Phase 1/2/3 clinical trial, and REGENXBIO Inc. with a product candidate in Phase 1/2 clinical development. We are also aware of several companies and research institutions conducting clinicals trials in small molecule product candidates focused on CPVT, including Armgo Pharmaceuticals, Inc. with an orally administered Rycal in a Phase 2 clinical trial and Cardurion Pharmaceuticals, Inc. with an orally administered CAMKII-delta inhibitor candidate in a Phase 2 clinical trial.

Our commercial opportunity could be reduced or eliminated if competitors develop and commercialize products that are first to market or are safer, more effective, have fewer or less severe side effects, have broader market acceptance, are more convenient or are less expensive than any product candidate that we may develop. Changes within the competitive landscape could lead us to alter clinical trial strategy, baseline eligibility criteria or make other modifications to clinical trial designs.

We are aware of several companies focused on developing gene therapies in various indications, as well as several companies addressing other methods for modifying genes and regulating gene expression. Any advances in gene therapy technology made by a competitor may be used to develop therapies that could compete against Candidates we develop.

We may fail to capitalize on other potential product candidates that may represent a greater commercial opportunity or for which there is a greater likelihood of success.

The success of our business depends upon our ability to develop and commercialize our Candidates. Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential than our Candidates. For example, in September 2022, we announced that we would be pausing activities for SGT-001, which we are now no longer developing.

In addition, in October 2020, we entered into a collaboration and license agreement with Ultragenyx, pursuant to which we granted Ultragenyx an exclusive worldwide license under certain intellectual property rights controlled by us to develop AAV8 or other clade E AAV variant pharmaceutical products that express our MD5 nNOS binding domain form of microdystrophin protein for the treatment of Duchenne and other disease indications resulting from a lack of functional dystrophin, which we refer to as the Licensed Products.

Our spending on current and future research and development programs may not yield any commercially viable product candidates. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaborations, licensing or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Alternatively, we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement. If any of these events occur, we may be forced to abandon our development efforts with respect to a particular product candidate or fail to develop a potentially successful product candidate.

Risks related to the manufacturing and commercialization of our product candidates

We have entered into, and may in the future enter into, collaborations with third parties for the development or commercialization of our product candidates. If our collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates and our business could be adversely affected.

In October 2020, we entered into a collaboration and license agreement with Ultragenyx, pursuant to which we granted Ultragenyx an exclusive worldwide license under certain intellectual property rights controlled by us to develop the Licensed Products.

While we have retained all rights to and are developing on our own SGT-003, we may in the future enter into development, distribution or marketing arrangements with third parties with respect to SGT-003 or other Candidates. Our likely collaborators for any such sales, marketing, distribution, development, licensing or broader collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we enter into any such arrangements with any third parties in the future, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our Candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities and efforts to successfully perform the functions assigned to them in these arrangements.

Collaborations that we enter into, including our collaboration with Ultragenyx, may not be successful, and any success will depend heavily on the efforts and activities of such collaborators. Collaborations pose a number of risks, including the following:

- collaborators have significant discretion in determining the amount and timing of efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development of our product candidates or may elect not to continue or renew development programs based on results of clinical trials or other studies, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may not pursue commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew commercialization programs based on results of clinical trials or other studies, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that may divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such product candidates on a discretionary basis;
- collaborators, including Ultragenyx, could develop products that compete directly or indirectly with our product candidates and products pursuant to the collaboration;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates and products if the collaborators believe that the competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- a collaborator may fail to comply with applicable regulatory requirements regarding the development, manufacture, distribution or marketing of a product candidate or product;
- a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;

- disagreements with collaborators, including disagreements over intellectual property or proprietary rights, contract interpretation or the preferred course of development, might cause delays or terminations of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly obtain, maintain, enforce, defend or protect our intellectual property or proprietary rights or may use our proprietary information in such a way as to potentially lead to disputes or legal proceedings that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- disputes may arise with respect to the ownership of intellectual property developed pursuant to our collaborations;
- collaborators may infringe, misappropriate or otherwise violate the intellectual property or proprietary rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated for the convenience of the collaborator, and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner, or at all. If any collaborations that we enter into do not result in the successful development and commercialization of products or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of our product candidates could be delayed and we may need additional resources to develop our product candidates. All of the risks relating to product development, regulatory approval and commercialization described herein also apply to the activities of our collaborators.

Additionally, subject to its contractual obligations to us, if a collaborator of ours is involved in a business combination, the collaborator might deemphasize or terminate the development or commercialization of any product candidate licensed to it by us. If one of our collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and our perception in the business and financial communities could be adversely affected.

We may not be successful in finding strategic collaborators for continuing development of our Candidates or platform technologies, or for successfully commercializing or competing in the market for certain indications.

We may seek to establish strategic partnerships for developing Candidates or platform technologies due to capital costs required to develop, manufacture and commercialize our product candidates or platform technologies. We may not be successful in our efforts to establish strategic partnerships or other alternative arrangements because, among other things, our research and development pipeline may be insufficient, Candidates or platform technologies may be deemed to be at too early of a stage of development for collaborative effort or third parties may not view our Candidates or platform technologies as having the requisite potential to demonstrate safety and efficacy. We cannot be certain that, following a strategic transaction, we will achieve an economic or business benefit that justifies such transaction. If we seek to but are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms or at all, we may have to curtail, reduce or delay the development of a product candidate, delay its potential commercialization, reduce the scope of any sales or marketing activities or increase our expenditures and undertake development, manufacturing or commercialization activities independently. If we elect to fund our own independent development or commercialization activities, we will need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development, manufacturing and commercialization activities, we may not be able to further develop our Candidates or platform technologies.

We have limited gene therapy manufacturing experience and could experience production problems and delays in obtaining regulatory approval of our manufacturing processes, which could result in delays in the development or commercialization of SGT-003, SGT-501, or other current and future candidates. In addition, changes to manufacturing sites or processes, or formulations for our product candidates may result in additional cost or delay.

We have limited experience manufacturing SGT-003, SGT-501 and our other current or future candidates. The manufacturing process we have used historically and the manufacturing process we plan to use in the future to produce product for our candidates are complex and our processes have not been validated for commercial use. As candidates progress through preclinical studies and clinical trials to marketing approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, are altered along the way in an

effort to optimize safety, quality, efficacy, yield, manufacturing batch size, minimize costs and achieve consistent results. For example, we have moved to a transient transfection-based manufacturing process for SGT-003. While we have observed positive results in preclinical studies using this new manufacturing process, any further changes in manufacturing or formulation may result in effects and results that are different from those observed in our completed preclinical studies to date. Similarly, in the future we may further optimize our existing process or introduce an alternative process or formulation of one or more of our candidates during the course of our planned preclinical studies or clinical trials. Such changes carry the risk that they will not achieve these intended objectives. Any of these changes could cause our candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the altered materials. This could delay initiation or completion of clinical trials, require the conduct of bridging studies or clinical trials or the repetition of one or more studies or clinical trials, increase development costs, delay approval of our candidates and jeopardize our ability to commercialize our candidates, if approved, and generate revenue.

The production of SGT-003 and SGT-501 uses a transient transfection-based process which requires processing steps that are more complex than those required for most chemical pharmaceuticals. We also intend to use transient transfection manufacturing for our other Candidates. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a gene therapy candidate such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we have and will continue to employ multiple steps to control our manufacturing processes to assure that the process works and that SGT-003, SGT-501 and our other Candidates are made strictly and consistently in compliance with such processes. We must supply all necessary documentation in support of an IND, BLA or MAA on a timely basis and must adhere to the FDA's and the European Union's cGMP requirements before we can obtain marketing approval for SGT-003, SGT-501, and other Candidates. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMP requirements, by performing extensive audits of contract laboratories, manufacturers and suppliers.

We currently rely on third-party manufacturers for SGT-003 and SGT-501 and plan to rely on third-party manufacturers for our Candidates. In order to produce sufficient quantities of product candidates for clinical trials and initial U.S. commercial demand, we have and will continue to further optimize and increase the capacity of our manufacturing process at our third-party manufacturers. We may need to make changes to our manufacturing processes, beyond implementation of a transient transfection-based manufacturing process. We may not be able to produce sufficient quantities of drug product due to several factors, including equipment malfunctions, facility contamination, material shortages or contamination, natural disasters, a public health issue (for example, an outbreak of a contagious disease such as the recent COVID-19 pandemic), disruption in utility services, human error or disruptions in the operations of our suppliers. For example, we have not released a manufacturing lot for clinical supply utilizing the transient transfection-based manufacturing process and may experience variability with respect to the success and yield between lots that will require continued engagement in process development activities to improve the reproducibility, reliability, quality and consistency of yields of the manufacturing process. Additional manufacturing runs will be required to produce necessary or adequate supply for our future clinical trials and there is no guarantee that all of those runs will be within specifications or produce adequate supply. If we are not able to produce sufficient supply on the timeline expected, our overall development schedule for SGT-003, SGT-501 and other Candidates could be delayed, and we could incur additional expense. Any such failure could delay or prevent our IND or commercialization of SGT-003, SGT-501 or other Candidates.

If supply from a manufacturing facility is interrupted, including as a result of equipment malfunctions, facility contamination, material shortages or contamination, natural disasters, public health emergencies or pandemics, such as the recent COVID-19 pandemic, disruption in utility services or human error, there could be a significant disruption in supply of SGT-003 or other Candidates. In such instance, we may need to locate appropriate replacement third-party manufacturers, and we may not be able to enter into arrangements with such additional third-party manufacturers on favorable terms or at all. Use of new third-party manufacturers could increase the risk of delays in production or insufficient supplies of our product candidates as we transfer our manufacturing technology to these manufacturers and as they gain experience manufacturing our product candidates.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

In addition, the FDA, the EMA and other foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA or other foreign regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Lot failures or product recalls could cause us to delay or abandon clinical trials or product launches.

We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to oversee our manufacturing and quality control process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

Any problems in our manufacturing process or facilities could make us a less attractive collaborator for potential partners, including biotechnology and pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in our manufacturing process or facilities also could restrict our ability to meet market demand for our product candidates.

We expect to utilize third parties to conduct our product manufacturing for the foreseeable future. Therefore, we are subject to the risk that these third parties may not perform satisfactorily or meet regulatory requirements.

We do not independently manufacture material for our ongoing or planned clinical programs and we are utilizing and expect to utilize materials manufactured by cGMP-compliant third-party suppliers. If these third-party manufacturers do not successfully carry out their contractual duties, meet expected deadlines or manufacture our product candidates in accordance with quality and regulatory requirements or if there are disagreements between us and these third-party manufacturers, we may not be able to complete, or may be delayed in completing, the clinical trials required for approval of our product candidates. In such instances, we may need to locate an appropriate replacement third-party relationship, which may not be readily available or on acceptable terms, which would cause additional delay or increased expense prior to the approval of our product candidates.

Additionally, we rely on our third-party manufacturers for their compliance with the cGMP and their maintenance of adequate quality control, quality assurance and qualified personnel. Furthermore, all of our third-party suppliers and manufacturers are engaged with other companies to supply and/or manufacture materials or products for such companies, which exposes them to regulatory risks for the production of such materials and products. FDA inspections may identify compliance issues at third-party manufacturer facilities or at the facilities of third-party suppliers that may disrupt production or distribution, or require substantial resources to correct and prevent recurrence of any deficiencies, and could result in fines or penalties by regulatory authorities. In addition, discovery of problems with a product or the failure to comply with applicable requirements may result in restrictions on a product, manufacturer or holder of an approved BLA, including withdrawal or recall of the product from the market or other voluntary, FDA-initiated or judicial action, including fines, injunctions, civil penalties, license revocations, seizure, total or partial suspension of production or criminal penalties, any of which could significantly and adversely affect supplies of our product candidates.

In addition, we do not currently have long-term supply or manufacturing arrangements in place for the production of our product candidates at commercial scale. Although we intend to establish additional sources for long-term supply, from one or more third-party manufacturers, if the gene therapy industry were to grow, we may encounter increasing competition for the materials necessary for the production of product candidates. We may experience difficulties in scaling up production beyond clinical batches. Furthermore, demand for third-party cGMP manufacturing facilities may grow at a faster rate than existing manufacturing capacity, which could disrupt our ability to find and retain third-party manufacturers capable of producing sufficient quantities of our product candidates for future clinical trials or to meet initial commercial demand in the United States. We currently rely, and expect to continue to rely, on additional third parties to manufacture materials for our

product candidates and to perform quality testing. We intend to maintain third-party manufacturers for these materials, as well as to serve as additional sources of our product candidates, which will expose us to risks including:

- reduced control of manufacturing activities;
- the inability of certain CMOs to produce our product candidates in the necessary quantities, or in compliance with current cGMP or in compliance with pertinent regulatory requirements and within our planned time frame and cost parameters;
- termination or nonrenewal of manufacturing and service agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturer and our and their suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier, natural disasters or public health issues.

Any of these events could lead to clinical trial delays or failure to obtain regulatory approval or impact our ability to successfully commercialize our product candidates. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of product manufacture.

If we are unable to establish sales, distribution and marketing capabilities or enter into agreements with third parties to market and sell our Candidates, we will be unable to generate any product revenue.

We currently have no sales, distribution or marketing organization. To successfully commercialize any product candidate that may result from our development programs, we will need to develop these capabilities, either on our own or with others. The establishment and development of our own commercial team or the establishment of a contract sales force to market any product candidate we may develop will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We may enter into collaborations regarding our Candidates with other entities to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any future collaborators do not commit sufficient resources to commercialize our product candidates, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded sales, distribution and marketing operations to recruit, hire, train and retain marketing and sales personnel. We will also face competition in our search for third parties to assist us with the sales and marketing efforts of any future products. Without an internal team or the support of a third party to perform marketing and sales functions, we will be unable to compete successfully against these more established companies.

If we are unable to establish medical affairs capabilities, we will be unable to establish an educated market of physicians to administer any future products.

We currently have no medical affairs team. If we are unable to successfully build a medical affairs team to address scientific and medical questions and provide expert guidance and education in the application, administration and utilization of any future products to physicians, we may not be able to establish an educated market for our products. The establishment and development of our own medical affairs team will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability.

If the market opportunities for any of our future products are smaller than we believe they are, our revenue prospects may be adversely affected and our business may suffer.

We currently focus our research and product development on treatments for rare genetic neuromuscular and cardiac indications. Our understanding of the patient population with these diseases is based on estimates in published literature and by disease-focused foundations. These estimates may prove to be incorrect and new studies may reduce the estimated incidence or prevalence of these diseases. The number of patients in the United States, the European Union and elsewhere may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our product candidates or patients may become increasingly difficult to identify and access.

Further, there are several factors that could contribute to reducing the actual number of patients who could receive our Candidates less than the potentially addressable market. These include the lack of widespread availability of, and limited reimbursement for, new therapies in many underdeveloped markets. Further, the severity of the progression of a degenerative disease such as Duchenne and FA up to the time of treatment will likely diminish the therapeutic benefit conferred by a gene therapy due to irreversible cell damage.

Certain patients' immune systems might prohibit the successful delivery of certain gene therapy products, thereby potentially limiting the population of patients amenable to gene transfer.

As with many AAV-mediated gene therapy approaches, certain patients' immune systems might prohibit the successful delivery of certain gene therapy products, thereby potentially limiting the population of patients amenable to gene transfer. While we are working to better understand the prevalence of antibodies to AAV, or seroprevalence, as it relates to gene therapy, the exact seroprevalence is currently unknown and varies by AAV serotype and age. We may not be able to address these potentially limiting factors for gene therapy as a treatment for certain patients.

The commercial success of any of our candidates, if approved, will depend upon market acceptance by physicians, patients, third-party payors and others in the medical community.

Even with the requisite approvals from the FDA in the United States, the European Commission in the European Union and other regulatory authorities internationally, the commercial success of our candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and, in particular for each of our current and future candidate, as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community due to ethical, social, medical and legal concerns. If our products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of gene therapy products and, in particular, our current and future candidates, if approved for commercial sale, will depend on multiple factors, including:

- the efficacy and safety of our current and future candidates as demonstrated in clinical trials;
- the efficacy and potential and perceived advantages of our product candidates over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which our product candidates are approved by the FDA, the European Commission or other regulatory authorities, as applicable;
- the willingness of physicians to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, the EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of products to meet market demand;
- publicity concerning our product candidates or competing products and treatments;
- any restrictions on the use of our products together with other medications; and
- favorable third-party payor coverage and adequate reimbursement.

Even if a potential product candidate displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

Our efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our potential product candidates. If our product candidates are approved but fail to achieve market acceptance among physicians, patients or third-party payors, we will not be able to generate significant revenue from any such product.

Our gene transfer approach utilizes capsids derived from a virus, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of gene transfer product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our Candidates.

Gene transfer remains a novel technology that faces many challenges imposed by the humoral immune response. The immunogenicity of AAV gene transfers is a very complex process that we and others continue to work understand through the extensive clinical experience that now exists over a broad spectrum of therapeutic areas and indications. Marked inflammatory toxicities have been observed, including complement activation, cytopenias, severe hepatotoxicity as well as transgene related toxicities representing part of the continuum of diverse aspects of clinical immune responses that can be observed post gene transfer.

In particular, our success will depend upon physicians who specialize in the treatment of our pipeline indications, prescribing treatments that involve the use of viral capsids in lieu of, or in addition to, other treatments with which they are more familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion may delay or impair the development and commercialization or demand for any product candidate we may develop. A public backlash developed against gene therapy following the death of a patient in 1999 during a gene therapy clinical trial of research subjects with ornithine transcarbamylase, or OTC, deficiency, a rare disorder in which the liver lacks a functional copy of the OTC gene. The death of the clinical trial subject was due to complications of adenovirus capsid administration. Dr. James M. Wilson, former chair of our Scientific Advisory Board, was a co-investigator of the 1999 trial while he was Director of the Institute for Human Gene Therapy of the University of Pennsylvania. Serious adverse events in our clinical trials, including the events that led to the previously-lifted clinical holds on IGNITE DMD or other clinical trials involving gene transfer products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our Candidates, stricter labeling requirements for our Candidates, if approved, and a decrease in demand for our Candidates.

Any contamination in our manufacturing process, shortages of materials or failure of any of our key suppliers to deliver necessary components could result in interruption in the supply of our product candidates and delays in our clinical development or commercialization schedules.

Given the nature of biologics manufacturing, there is a risk of contamination in our manufacturing processes. Any contamination could materially adversely affect our ability to produce our candidates on schedule and could cause reputational damage.

Some of the materials required in our manufacturing process are derived from biologic sources. Such materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our candidates could adversely impact or disrupt the manufacturing or the production of clinical material, which could materially and adversely affect our development timelines.

The insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. We expect the cost of a single administration of gene transfer products, such as those we are developing, to be substantial, when and if they achieve regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of our future products, if approved, will depend substantially, both domestically and abroad, on the extent to which the costs of such product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar health care management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective;
- durable and a one-time treatment, as applicable; and
- neither experimental nor investigational.

Obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize our future products, if approved. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment.

To our knowledge, only a limited number of gene transfer products have been approved for coverage and reimbursement by the Centers for Medicare & Medicaid Services, or the CMS, the agency responsible for administering the Medicaid program. It is difficult to predict what the CMS will decide with respect to coverage and reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these types of products either in the United States or the European Union. For example, several cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European Union member states and vice versa. It is difficult to predict what third-party payors will decide with respect to the coverage and reimbursement for our future products, if approved.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenue, if any.

Outside the United States, international operations generally are subject to extensive government price controls and other market regulations, and increasing emphasis on cost-containment initiatives in the European Union, Canada and other countries may put pricing pressure on us. In general, the prices of therapeutics outside the United States are substantially lower than in the United States. Other countries may allow companies to fix their own prices for therapeutics, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulations could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our product candidates may be reduced compared with the United States and may be insufficient to generate commercially reasonable product revenue.

Additionally, in countries where the pricing of gene therapy products is subject to governmental control, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various European Union member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. Reimbursement of our products may be unavailable or limited in scope or amount, which would adversely affect our revenue, if any.

If we obtain approval to commercialize our future products outside of the United States, in particular in the European Union, a variety of risks associated with international operations could materially adversely affect our business.

We expect that we will be subject to additional risks in commercializing our future products, if approved, outside the United States, including:

- different regulatory requirements for approval of therapeutics in foreign countries;
- reduced protection for intellectual property rights;
- the existence of additional third-party patent rights of potential relevance to our business;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;

- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- foreign reimbursement, pricing and insurance regimes;
- production shortages resulting from any events affecting material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires.

The failure to comply with applicable foreign regulatory requirements may result in, among other things, fines, suspension, variation or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

If we engage in future acquisitions or strategic collaborations, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

We may evaluate various acquisitions and strategic collaborations, including licensing or acquiring complementary products, intellectual property rights, technologies or businesses. Any potential acquisition or strategic collaboration may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing product candidates and initiatives in pursuing such acquisition or strategic collaboration;
- retention of key employees, the loss of key personnel and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and regulatory approvals; and
- our inability to generate revenue from acquired technology and/or products sufficient to meet our objectives in undertaking the acquisition or collaboration or even to offset transaction costs.

In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense. Moreover, we may not be able to locate suitable acquisition or collaboration opportunities and this inability could impair our ability to grow or obtain access to technology or products that may be important to the development of our business.

Risks related to our business operations

Our future success depends on our ability to retain key employees, consultants and advisors and to attract, retain and motivate qualified personnel.

We are highly dependent on members of our executive team, the loss of whose services may adversely impact the achievement of our objectives. While we have entered into employment agreements with certain of our executive officers, any of them could leave our employment at any time. We currently do not have "key person" insurance on any of our employees. The loss of the services of one or more of our current key employees might impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel, also will be critical to our success. There currently is a shortage of skilled individuals with substantial gene therapy experience, which is likely to continue. As a result, competition for skilled personnel, including in gene therapy research and capsid manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for individuals with similar skill sets. In addition, the failure to succeed in preclinical or clinical trials or applications for marketing approval may make it more challenging to recruit and retain qualified personnel. The inability to

recruit, or loss of services of certain executives, key employees, consultants or advisors, may impede the progress of our research, development and commercialization objectives.

Our strategic plan and associated workforce reductions may not result in anticipated savings, could result in total costs and expenses that are greater than expected and could disrupt our business.

In April 2022 and December 2022, we announced a reduction in workforce by approximately 35% and 18%, respectively, as part of a strategic plan designed to streamline our operating structure. We may not realize, in full or in part, the anticipated benefits, savings and improvements in our cost structure from our restructuring efforts due to unforeseen difficulties, delays or unexpected costs. If we are unable to realize the expected operational efficiencies and cost savings from the restructuring, our operating results and financial condition would be adversely affected. We also cannot guarantee that we will not have to undertake additional workforce reductions or restructuring activities in the future. Furthermore, our strategic restructuring plan and the Acquisition may be disruptive to our operations. For example, our workforce reductions and integration of AavantiBio's business and operations into ours could yield unanticipated consequences, such as attrition beyond planned staff reductions, or increase difficulties in our day-to-day operations. Our workforce reductions and the Acquisition could also harm our ability to attract and retain qualified management, scientific, clinical, manufacturing and sales and marketing personnel who are critical to our business. Any failure to attract or retain qualified personnel could prevent us from successfully developing and commercializing our product candidates in the future.

If we are unable to manage growth in the scale and complexity of our operations, our performance may suffer.

If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial and other systems and resources to manage our operations, continue our research and development activities and, in the longer term, build a commercial infrastructure to support commercialization of our current and future candidates and products that are approved for sale. Future growth would impose significant added responsibilities on members of management. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and any future product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

Our employees may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk of employee fraud or other misconduct, including intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. This could include violations of the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, other U.S. federal and state law, and requirements of non-U.S. jurisdictions, including the European Union Data Protection Directive. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, standards, regulations, guidance or codes of conduct. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions.

Enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

Our business and financial prospects could be affected by changes in health care spending and policy in the United States and abroad. We operate in a highly regulated industry and new laws or judicial decisions, or new interpretations of existing laws or decisions, related to health care availability, the method of delivery or payment for health care products and services could negatively impact our business, operations and financial condition.

For example, in the United States there is significant interest in promoting health care reform, as evidenced by the enactment of the Patient Protection and Affordable Care Act and the companion Health Care and Education Reconciliation Act, or the Health Care Reform Law. The Health Care Reform Law increased federal oversight of private health insurance plans and included a number of provisions designed to reduce Medicare expenditures and the cost of health care generally, to reduce fraud and abuse, and to provide access to increased health coverage.

The Health Care Reform Law also imposed substantial changes to the U.S. system for paying for health care, including programs to extend medical benefits to millions of individuals who have lacked insurance coverage. Generally, implementation of the Health Care Reform Law has thus far included significant cost-saving, revenue and payment reduction measures with respect to, for example, several government health care programs that might cover our products in the United States, should they be commercialized, including Medicaid and Medicare. Additional downward pricing pressure associated with the Health Care Reform Law includes that the Health Care Reform Law established and provided significant funding for a Patient-Centered Outcomes Research Institute to coordinate and fund Comparative Effectiveness Research, as those terms are defined in the Health Care Reform Law. While the stated intent of Comparative Effectiveness Research is to develop information to guide providers to the most efficacious therapies, outcomes of Comparative Effectiveness Research could influence the reimbursement or coverage for therapies that are determined to be less cost-effective than others. Should any of our products be approved for sale, but then determined to be less cost-effective than alternative therapies, the levels of reimbursement for these products, or the willingness to reimburse at all, could be adversely impacted.

In addition to legislative changes resulting from the passage of the Health Care Reform Law, other legislative changes have been proposed and adopted since the Health Care Reform Law was enacted. In August 2011, the Budget Control Act of 2011, among other things, led to aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which will remain in effect for the first half of 2032. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2029 unless additional Congressional action is taken. The Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, suspended the 2% Medicare sequester from May 1, 2020 through December 31, 2020, and extended the sequester through 2031. These Medicare sequester reductions were suspended through June 2022, with the full 2% cut resuming thereafter. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used. Indeed, under current legislation, the actual reductions in Medicare payments may vary up to 4%. The Consolidated Appropriations Act, which was signed into law by President Biden in December 2022, made several changes to sequestration of the Medicare program. Section 1001 of the Consolidated Appropriations Act delays the 4% Statutory Pay-As-You-Go Act of 2010 sequester for two years, through the end of calendar year 2024. Triggered by enactment of the American Rescue Plan Act of 2021, the 4% cut to the Medicare program would have taken effect in January 2023. The Act's health care offset title includes Section 4163, which extends the 2% Budget Control Act of 2011 Medicare sequester for six months into fiscal year 2032 and lowers the payment reduction percentages in fiscal years 2030 and 2031.

Since enactment of the Health Care Reform Law, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, or the TCJA, Congress repealed the "individual mandate." The repeal of this provision of the Health Care Reform Law, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Further, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the Health Care Reform Law is an essential and inseverable feature of the Health Care Reform Law, and therefore because the mandate was repealed as part of the TCJA, the remaining provisions of the Health Care Reform Law are invalid as well. The U.S. Supreme Court heard this case on November 10, 2020 and on June 17, 2021, dismissed this action after finding that the plaintiffs do not have standing to challenge the constitutionality of the statute. It is unclear how such litigation and other efforts to repeal and replace the Health Care Reform Law will impact the Health Care Reform Law and our business. Litigation and legislation over the Health Care Reform Law are likely to continue, with unpredictable and uncertain results.

Although the previous administration took actions to undermine or delay implementation of the Health Care Reform Law, President Biden rescinded those actions with the issuance of an Executive Order on January 28, 2021 which directs federal agencies to reconsider rules and other policies that limit Americans' access to health care, and consider actions that will protect and strengthen that access. Under this Executive Order, federal agencies are directed to re-examine policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the Health Care Reform Law that may reduce coverage or undermine the programs,

including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the Health Care Reform Law; and policies that reduce affordability of coverage or financial assistance, including for dependents. This Executive Order also directs the U.S. Department of Health and Human Services to create a special enrollment period for the Health Insurance Marketplace in response to the recent COVID-19 pandemic.

Current and future legislative efforts may limit the prices for our products, if and when they are licensed for marketing and that could materially impact our ability to generate revenues.

The prices of prescription pharmaceuticals have been the subject of considerable discussion in the United States. To date, there have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for products. In 2020, CMS issued an interim final rule implementing a most favored nation model for prices that would tie Medicare Part B payments for certain physician-administered pharmaceuticals to the lowest price paid in other economically advanced countries, effective January 1, 2021. That rule, however, has been subject to a nationwide preliminary injunction and, on December 29, 2021, CMS issued a final rule to rescind it. With issuance of this rule, CMS stated that it will explore all options to incorporate value into payments for Medicare Part B pharmaceuticals and improve beneficiaries' access to evidence-based care.

In addition, in October 2020, the HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program, or SIP, to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by the Pharmaceutical Research and Manufacturers of America, or PhRMA, but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. Nine states have passed laws allowing for the importation of drugs from Canada. Certain of these states have submitted Section 804 Importation Program proposals and are awaiting FDA approval. In January 2024, the FDA approved Florida's plan for Canadian drug importation. Further, the HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law.

Further, on November 20, 2020, the HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Medicare Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a new safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers, the implementation of which has been delayed until January 1, 2032 by the IRA.

The IRA has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028, and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years, but it does not apply to drugs and biologics that have been approved for a single rare disease or condition. Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $4,000 a year in 2024 and, thereafter beginning in 2025, at $2,000 a year.

The IRA includes a provision exempting orphan drugs from Medicare price negotiation but this exclusion has been interpreted by CMS in final guidance issued in July 2023 to apply only to those orphan drugs with an approved indication (or indications) for a single rare disease or condition. The final guidance clarifies that CMS will consider only active designations/approvals when evaluating a drug for the exclusion, such that designations/indications withdrawn before the selected drug publication date will not be considered. CMS also clarified that, if a drug loses its orphan drug exclusion status, the agency will use the earliest date of approval/licensure to determine whether the product is a qualifying single source drug subject to price negotiations.

In June 2023, Merck filed a lawsuit against HHS and CMS asserting that, among other things, the IRA's Drug Price Negotiation Program for Medicare constitutes an uncompensated taking in violation of the Fifth Amendment of the Constitution. Subsequently, a number of other parties, including the U.S. Chamber of Commerce and pharmaceutical companies, also filed lawsuits in various courts with similar constitutional claims against HHS and CMS. Litigation involving these and other provisions of the IRA will continue with unpredictable and uncertain results.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, if approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

It is likely that federal and state legislatures within the United States and foreign governments will continue to consider changes to existing health care legislation. We cannot predict the reform initiatives that may be adopted in the future or whether initiatives that have been adopted will be repealed or modified. The continuing efforts of the government, insurance companies, managed care organizations and other health care payors of to contain or reduce costs of health care may adversely affect:

- the demand for any product candidates for which we may obtain regulatory approval;
- our ability to set a price that we believe is fair for our products;
- our ability to obtain coverage and reimbursement approval for a product;
- our ability to generate revenue and achieve or maintain profitability; and
- the level of taxes that we are required to pay.

Finally, in the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of healthcare and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most European Union member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved.

In markets outside of the United States and the European Union, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action in the United States, the European Union or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

Our relationships with customers, physicians and third-party payors will be subject, directly or indirectly, to federal and state health care fraud and abuse laws, false claims laws, health information privacy and security laws, and other health care laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

If we obtain FDA approval for our current or future candidates and begin commercializing one or more of those products in the United States, our operations will be directly or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws and regulations, including, without limitation, the federal Health Care Program Anti-Kickback Statute, the federal civil and criminal laws and the Physician Payment Sunshine Act and regulations. These laws will impact, among other things, our proposed sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business. The laws that will affect our operations include, but are not limited to:

- the federal Health Care Program Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal health care program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers on the other. The Health Care Reform Law amended the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it;
- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent. The Health Care Reform Law provides and recent government cases against pharmaceutical and medical device manufacturers support the view that Federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the False Claims Act;
- HIPAA, which created new federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any health care benefit program, regardless of the payor (e.g., public or private);
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act; Other Modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and health care providers;

- federal transparency laws, including the federal Physician Payment Sunshine Act, that require certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the CMS information related to: (i) payments or other "transfers of value" made to physicians, other healthcare professionals and teaching hospitals and (ii) ownership and investment interests held by physicians and their immediate family members;
- state and foreign law equivalents of each of the above federal laws, state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other health care providers or marketing expenditures and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states; and
- state and foreign laws that govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment and the curtailment or restructuring of our operations.

The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. The shifting compliance environment and the need to build and maintain robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that we may run afoul of one or more of the requirements.

We are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies, contractual obligations and failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.

We are subject to data privacy and protection laws and regulations that apply to the collection, transmission, storage and use of personally-identifying information, which among other things, impose certain requirements relating to the privacy, security and transmission of personal information, including comprehensive regulatory systems in the United States, EU and UK. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Failure to comply with any of these laws and regulations could result in enforcement action against us, including fines, imprisonment of company officials and public censure, claims for damages by affected individuals, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects.

There are numerous U.S. federal and state laws and regulations related to the privacy and security of personal information. In particular, regulations promulgated pursuant to HIPAA establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information and ensure the confidentiality, integrity and availability of electronic protected health information. Determining whether protected health information has been handled in compliance with applicable privacy standards and our contractual obligations can be complex and may be subject to changing interpretation. These obligations may be applicable to some or all of our business activities now or in the future.

If we are unable to properly protect the privacy and security of protected health information, we could be found to have breached our contracts. Further, if we fail to comply with applicable privacy laws, including applicable HIPAA privacy and security standards, we could face civil and criminal penalties. HHS enforcement activity can result in financial liability and reputational harm, and responses to such enforcement activity can consume significant internal resources. In addition, state attorneys general are authorized to bring civil actions seeking either injunctions or damages in response to violations that threaten the privacy of state residents. We cannot be sure how these regulations will be interpreted, enforced or applied to our operations. In addition to the risks associated with enforcement activities and potential contractual liabilities, our ongoing efforts to comply with evolving laws and regulations at the federal and state level may be costly and require ongoing modifications to our policies, procedures and systems.

In 2018, California passed into law the California Consumer Privacy Act, or CCPA, which took effect on January 1, 2020 and imposed many requirements on businesses that process the personal information of California residents. Many of the CCPA's requirements are similar to those found in the General Data Protection Regulation, or GDPR, including requiring businesses to provide notice to data subjects regarding the information collected about them and how such information is used and shared, and providing data subjects the right to request access to such personal information and, in certain cases, request the erasure of such personal information. The CCPA also affords California residents the right to opt-out of "sales" of their personal information. The CCPA contains significant penalties for companies that violate its requirements. In November 2020, California voters passed a ballot initiative for the California Privacy Rights Act, or CPRA, which went into effect on January 1, 2023 and significantly expanded the CCPA to incorporate additional GDPR-like provisions including requiring that the use, retention, and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information, and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA also created a new enforcement agency – the California Privacy Protection Agency – whose sole responsibility is to enforce the CPRA and other California privacy laws, which will further increase compliance risk. The provisions in the CPRA may apply to some of our business activities. In addition to California, a number of other states have passed comprehensive privacy laws similar to the CCPA and CPRA. These laws are either in effect or will go into effect sometime before the end of 2026. Like the CCPA and CPRA, these laws create obligations related to the processing of personal information, as well as special obligations for the processing of "sensitive" data (which includes health data in some cases). Some of the provisions of these laws may apply to our business activities. There are also states that are strongly considering privacy laws that will go into effect in 2025 and beyond. Other states will be considering these laws in the future, and Congress has also been debating passing a federal privacy law. There are also states that are specifically regulating health information that may affect our business. These laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products.

Similar to the laws in the United States, there are significant privacy and data security laws that apply in Europe and other countries. The collection, use, disclosure, transfer, or other processing of personal data, including personal health data, regarding individuals who are located in the EEA, and the processing of personal data that takes place in the EEA, is regulated by the GDPR, which went into effect in May 2018 and which imposes obligations on companies that operate in our industry with respect to the processing of personal data and the cross-border transfer of such data. The GDPR imposes onerous accountability obligations requiring data controllers and processors to maintain a record of their data processing and policies. If our or our partners' or service providers' privacy or data security measures fail to comply with the GDPR requirements, we may be subject to litigation, regulatory investigations, enforcement notices requiring us to change the way we use personal data and/or fines of up to 20 million Euros or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, as well as compensation claims by affected individuals, negative publicity, reputational harm and a potential loss of business and goodwill.

The GDPR places restrictions on the cross-border transfer of personal data from the EU to countries that have not been found by the European Commission to offer adequate data protection legislation, such as the United States. There are ongoing concerns about the ability of companies to transfer personal data from the EU to other countries. In July 2020, the Court of Justice of the European Union, or the CJEU, invalidated the EU-U.S. Privacy Shield, one of the mechanisms used to legitimize the transfer of personal data from the EEA to the U.S. The CJEU decision also drew into question the long-term viability of an alternative means of data transfer, the standard contractual clauses, for transfers of personal data from the EEA to the U.S. While we were not self-certified under the Privacy Shield, this CJEU decision may lead to increased scrutiny on data transfers from the EEA to the U.S. generally and increase our costs of compliance with data privacy legislation as well as our costs of negotiating appropriate privacy and security agreements with our vendors and business partners.

Following the withdrawal of the UK from the EU, the UK Data Protection Act 2018 applies to the processing of personal data that takes place in the UK and includes parallel obligations to those set forth by GDPR. In relation to data transfers, both the UK and the EU have determined, through separate "adequacy" decisions, that data transfers between the two jurisdictions are in compliance with the UK Data Protection Act and the GDPR, respectively. The UK and the U.S. have also agreed to a U.S.-UK "Data Bridge", which functions similarly to the EU-U.S. Data Privacy Framework and provides an additional legal mechanism for companies to transfer data from the UK to the United States. In addition to the UK, Switzerland is also in the process of approving an adequacy decision in relation to the Swiss-U.S. Data Privacy Framework (which would function similarly to the EU-U.S. Data Privacy Framework and the U.S.-UK Data Bridge in relation to data transfers from Switzerland to the United States). Any changes or updates to these developments have the potential to impact our business.

Additionally, in October 2022, President Biden signed an executive order to implement the EU-U.S. Data Privacy Framework, which serves as a replacement to the EU-U.S. Privacy Shield. The European Commission initiated the process to adopt an adequacy decision for the EU-U.S. Data Privacy Framework in December 2022 and the European Commission adopted the adequacy decision on July 10, 2023. The adequacy decision permits U.S. companies who self-certify to the EU-U.S. Data Privacy Framework to rely on it as a valid data transfer mechanism for data transfers from the EU to the U.S.

However, some privacy advocacy groups have already suggested that they will be challenging the EU-U.S. Data Privacy Framework. If these challenges are successful, they may not only impact the EU-U.S. Data Privacy Framework, but also further limit the viability of the standard contractual clauses and other data transfer mechanisms. The uncertainty around this issue has the potential to impact our business internationally.

Following Brexit, the UK Data Protection Act 2018 applies to the processing of personal data that takes place in the United Kingdom and includes parallel obligations to those set forth by GDPR. In relation to data transfers, both the United Kingdom and the EU have determined, through separate "adequacy" decisions, that data transfers between the two jurisdictions are in compliance with the UK Data Protection Act and the GDPR, respectively. Any changes or updates to these adequacy decisions have the potential to impact our business.

Beyond GDPR, there are privacy and data security laws in a growing number of countries around the world. While many loosely follow GDPR as a model, other laws contain different or conflicting provisions. These laws will impact our ability to conduct our business activities, including both our clinical trials and the sale and distribution of commercial products, through increased compliance costs, costs associated with contracting and potential enforcement actions.

While we continue to address the implications of the recent changes to data privacy regulations, data privacy remains an evolving landscape at both the domestic and international level, with new regulations coming into effect and continued legal challenges, and our efforts to comply with the evolving data protection rules may be unsuccessful. It is possible that these laws may be interpreted and applied in a manner that is inconsistent with our practices. We must devote significant resources to understanding and complying with this changing landscape. Failure to comply with laws regarding data protection would expose us to risk of enforcement actions taken by data protection authorities in the EEA and elsewhere and carries with it the potential for significant penalties if we are found to be non-compliant. Similarly, failure to comply with federal and state laws in the United States regarding privacy and security of personal information could expose us to penalties under such laws. Any such failure to comply with data protection and privacy laws could result in government-imposed fines or orders requiring that we change our practices, claims for damages or other liabilities, regulatory investigations and enforcement action, litigation and significant costs for remediation, any of which could adversely affect our business. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our business, financial condition, results of operations or prospects.

We are subject to anti-corruption laws, as well as export control laws, customs laws, sanctions laws and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses, be precluded from developing manufacturing and selling certain products outside the United States or be required to develop and implement costly compliance programs, which could adversely affect our business, results of operations and financial condition.

Our operations are subject to anti-corruption laws, including the U.K. Bribery Act 2010, or Bribery Act, the U.S. Foreign Corrupt Practices Act, or FCPA, and other anti-corruption laws that apply in countries where we do business and may do business in the future. The Bribery Act, FCPA and these other laws generally prohibit us, our officers, and our employees and intermediaries from bribing, being bribed or making other prohibited payments to government officials or other persons to obtain or retain business or gain some other business advantage. Compliance with the FCPA, in particular, is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

We may in the future operate in jurisdictions that pose a high risk of potential Bribery Act or FCPA violations, and we may participate in collaborations and relationships with third parties whose actions could potentially subject us to liability under the Bribery Act, FCPA or local anti-corruption laws. In addition, we cannot predict the nature, scope or effect of future regulatory requirements to which our international operations might be subject or the manner in which existing laws might be administered or interpreted. If we expand our operations outside of the United States, we will need to dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate.

We are also subject to other laws and regulations governing our international operations, including regulations administered by the governments of the United Kingdom and the United States, and authorities in the European Union, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, collectively referred to as the Trade Control laws. In addition, various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to

those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

There is no assurance that we will be completely effective in ensuring our compliance with all applicable anti-corruption laws, including the Bribery Act, the FCPA or other legal requirements, including Trade Control laws. If we are not in compliance with the Bribery Act, the FCPA and other anti-corruption laws or Trade Control laws, we may be subject to criminal and civil penalties, disgorgement and other sanctions and remedial measures, and legal expenses, which could have an adverse impact on our business, financial condition, results of operations and liquidity. The Securities and Exchange Commission also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions. Any investigation of any potential violations of the Bribery Act, the FCPA, other anti-corruption laws or Trade Control laws by United Kingdom, U.S. or other authorities could also have an adverse impact on our reputation, our business, results of operations and financial condition.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any product candidate that we may develop.

We face an inherent risk of product liability exposure related to the testing of SGT-003 and any of our current and future candidates in preclinical studies and clinical trials and may face an even greater risk if we commercialize any product candidate that we may develop. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidate that we may develop;
- loss of revenue;
- substantial monetary awards to trial participants or patients;
- significant time and costs to defend the related litigation;
- withdrawal of clinical trial participants;
- the inability to commercialize any of our product candidates; and
- injury to our reputation and significant negative media attention.

Although we maintain product liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage each time we commence a clinical trial and if we successfully commercialize any product candidate. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and viruses and other biologic materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages. We also could incur significant costs associated with civil or criminal fines and penalties. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Although we maintain workers' compensation insurance for certain costs and expenses, we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, which have tended to become more stringent over time. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities.

Our internal computer systems, or those of our collaborators, contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development.

Despite the implementation of security measures, our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Such systems are also vulnerable to service interruptions or to security breaches from inadvertent or intentional actions by our employees, third-party vendors and/or business partners, or from cyber-attacks by malicious third parties. Cyber-attacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cyber-attacks could include the deployment of harmful malware, ransomware, denial-of-service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. Cyber-attacks also could include phishing attempts or e-mail fraud to cause payments or information to be transmitted to an unintended recipient.

While we are not aware of any such material system failure, accident, cyber-attack or security breach to date, if such an event were to occur and cause interruptions in our or our collaborators', contractors' or consultants' operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from preclinical studies or clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of our current and other future candidates could be delayed.

Risks related to our intellectual property

We heavily rely on certain in-licensed patents and other intellectual property rights in connection with our development of our Candidates and may be required to acquire or license additional patents or other intellectual property rights to continue to develop and commercialize our Candidates.

Our ability to develop and commercialize our product candidates is heavily dependent on licenses to patent rights and other intellectual property granted to us by third parties. In particular, we have licensed certain patents and patent applications from the University of Missouri, the University of Washington and others that are important or necessary to the development of SGT-003, our other Candidates and other elements of our gene transfer program. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, development and commercialization obligations, milestone payments, royalties and other obligations on us. If we fail to comply with our obligations under our agreements, we may be subject to damages, which may be significant, and the licensor may have the right to terminate the license, in which event we may not be able to develop or market product candidates or technologies covered by the license. In addition, certain of these license agreements are not assignable by us without the consent of the respective licensor, which may have an adverse effect on our ability to engage in certain transactions.

Under our existing license agreements, we do not have, and under future license agreements we may not have, the right to control the preparation, filing and prosecution of patent applications, or the maintenance, enforcement and defense of the patents and patent applications that we license from third parties. For example, under our inbound license agreements with the University of Missouri and the University of Washington, each of the applicable licensors controls the prosecution of patent applications and the maintenance of patents and patent applications. Therefore, we cannot be certain that the licensed patents and applications will be prosecuted, maintained, enforced and defended in a manner consistent with the best interests of our business. If our licensors fail to maintain, enforce or defend such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our product candidates that are the subject of such licensed rights could be adversely affected. For more information, see Part I, Item 1, "Business—Strategic partnerships and collaborations/licenses" of this Annual Report on Form 10-K.

Moreover, licenses to additional third-party intellectual property, technology and materials may be required for our development programs but may not be available in the future or may not be available on commercially reasonable terms. For example, third parties may claim that the constructs containing the gene or protein of interest and the AAV capsids we are developing for use in product candidates are covered by patents held by them. We believe that we would have valid defenses to any such claims; however, if any such claims were ultimately successful, we might require a license to continue to use and sell product candidates and such AAV capsids. Such licenses may not be available on commercially reasonable terms, or at all. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be

unwilling to assign our license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. Moreover, even if we are able to obtain such licenses, they may only be non-exclusive, which could permit competitors and other third parties to use the same intellectual property in competition with us.

We may collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. These institutions may provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the required timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

If we are unable to successfully obtain rights, or successfully challenge such rights, to any third-party intellectual property rights that are required for the development and commercialization of our Candidates, and such third-party intellectual property rights are successfully asserted against us, we may be liable for damages, which may be significant, and we may be required to cease the development and commercialization of our Candidates.

If we are unable to obtain and maintain patent protection for our Candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our Candidates may be adversely affected.

Our success depends, in large part, on our and our licensors' ability to seek, obtain, maintain, enforce and defend patent rights in the United States and other countries with respect to our Candidates and our future innovation related to our manufacturing technology. Our licensors and we have sought, and we intend to continue to seek, to protect our proprietary position by filing patent applications in the United States and, in at least some cases, one or more countries outside the United States related to our Candidates that are important to our business. However, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents or whether the claims of any issued patents will provide us with a competitive advantage.

Moreover, although we have pending patent applications in the United States and abroad, we cannot predict whether or in which jurisdictions the pending applications will result in issuance of patents that effectively protect any of our product candidates or will effectively prevent others from commercializing competitive products. Further, each of the provisional patent applications is not eligible to become an issued patent until, among other things, we file a non-provisional patent application within 12 months of the filing date of each provisional patent application. If we do not timely file a non-provisional patent application in respect of a provisional patent application, we may lose our priority date with respect to such provisional patent application and any patent protection on the inventions disclosed in such provisional patent application. While we intend to timely file non-provisional patent applications relating to our provisional patent applications, we cannot predict whether such future patent applications will result in the issuance of patents that effectively protect any of our product candidates or will effectively prevent others from commercializing competitive products.

We may not be able to file, prosecute, maintain, enforce, defend or license all patents that are necessary to our business.

The patent prosecution process is expensive, time-consuming and complex, and we and our licensors may not be able to file, prosecute, maintain, enforce, defend or license all necessary or desirable patents and patent applications at a reasonable cost or in a timely manner.

It is also currently unknown what claims may, if ever, issue from pending applications included in our patent rights. Additionally, certain of our in-licensed U.S. patent rights lack corresponding foreign patents or patent applications, and therefore we will be unable to obtain patent protection for our product candidates in certain jurisdictions. We or our licensors may not be able to obtain or maintain patent protection with respect to our Candidates.

Changes in either the patent laws or their interpretation in the United States and other countries may diminish our ability to protect our inventions, obtain, maintain and enforce our intellectual property rights, and more generally, could affect the value of our intellectual property rights or narrow the scope of our licensed patents or future owned patents.

It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach the agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Patent applications included in our current and future patent rights may not result in patents being issued that protect our product candidates, effectively prevent others from commercializing competitive products or otherwise provide any competitive advantage. In fact, patent applications may not issue as patents at all. Even assuming patents issue from patent applications in which we have rights, changes in either the patent laws or interpretation of the patent laws in the United States and other jurisdictions may diminish the value of our patents or narrow the scope of our patent protection.

Other parties have developed products that may be related or competitive to our own and such parties may have filed or may file patent applications, or may have received or may receive patents, claiming inventions that may overlap or conflict with those claimed in our patent applications or issued patents. We may not be aware of all third-party intellectual property rights potentially relating to our Candidates. In addition, we cannot provide any assurances that any of the inventions disclosed in our patent applications will be found to be patentable, including over third-party or our own prior art patents, publications or other disclosures, or will issue as patents. Even if our patent applications issue as patents, we cannot provide any assurances that such patents will not be challenged or ultimately held to be invalid or unenforceable. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and in other jurisdictions are typically not published until 18 months after filing, or, in some cases, at all. Therefore, we cannot know with certainty whether the inventors of our licensed patents and applications were the first to make the inventions claimed in those patents or pending patent applications, or that they were the first to file for patent protection of such inventions. Similarly, should we own any issued patents or patent applications in the future, we may not be certain that we were the first to file for patent protection for the inventions claimed in such patents or patent applications. Furthermore, given the differences in patent laws in the United States, Europe and other foreign jurisdictions, for example, the availability of grace periods for filing patent applications and what can be considered as prior art, we cannot make any assurances that any claims in our pending and future patent applications in the United States or other jurisdictions will issue, or if they do issue, whether they will issue in a form that provides us with any meaningful competitive advantage. Similarly, we cannot make any assurances that if the patentability, validity, enforceability or scope of our pending or future patents and patent applications in the United States or foreign jurisdictions are challenged by any third party, that the claims of such pending or future patents and patent applications will survive any such challenge in a form that provides us with any meaningful competitive advantage. For example, we are aware of certain third-party patents and publications related to certain microdystrophin constructs. While we believe that our owned or in-licensed patents and patent applications claim novel and non-obvious features of microdystrophin constructs that are not described in such third-party patents or publications, such third-party patents and publications may have earlier priority or publication dates and may be asserted as prior art against our owned or in-licensed patents and applications. Any such challenge, if successful, could limit or eliminate patent protection for our products and product candidates or otherwise materially harm our business. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights cannot be predicted with any certainty.

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if patent applications we license or may own in the future do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Any patents that we license or may own in the future may be challenged, narrowed, circumvented, or invalidated by third parties. Consequently, we do not know whether any of our product candidates will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative products in a non-infringing manner.

The degree of patent protection we require to successfully compete in the marketplace may be unavailable. We cannot provide any assurances that any of the patents or patent applications included in our patent rights include or will include claims with a scope sufficient to protect our product candidates or otherwise provide any competitive advantage. In addition, the laws of foreign countries may not protect our proprietary rights to the same extent as the laws of the United States. Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally twenty years after it is filed. Certain extensions may be available, however, the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent rights may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing products similar or identical to our product candidates, including biosimilar versions of such products.

Our licensed patents, and any patents we may own in the future, may be challenged, narrowed, invalidated or held unenforceable.

Even if we acquire patent protection that we expect should enable us to maintain some competitive advantage, third parties, including competitors, may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. In litigation, a competitor could claim that our in-licensed patents or any patents we may own in the future are not valid or enforceable for a number of reasons. If a court agrees, we would lose our rights to those challenged patents. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such proceedings could result in the revocation or cancellation of or amendment to our licensed patents and any patents we may own in the future in such a way that they no longer cover our product candidates.

Even if issued, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our current and future patent rights may be challenged in the courts or patent offices in the United States and abroad. For example, we may be subject to a third-party submission of prior art to the U.S. Patent and Trademark Office, or USPTO, challenging the validity of one or more claims of patents included in our patent rights. Such submissions may also be made prior to a patent's issuance, precluding the granting of a patent based on one of the pending patent applications included in our patent rights. We may become involved in opposition, derivation, revocation, reexamination, post-grant and *inter partes* review, or interference proceedings challenging one or more patents included in our patent rights. For example, competitors may claim that they invented the inventions claimed in patents or patent applications included in our patent rights, such as the microdystrophin we use in SGT-003, prior to the inventors of such patents or patent applications, or may have filed one or more patent applications before the filing of the patents or patent applications included in our patent rights. A competitor who can establish an earlier filing or invention date may also assert that we are infringing their patents and that we therefore cannot practice our technology related to our product candidates as claimed in the patents or patent applications included in our patent rights. Competitors may also contest patents or patent applications included in our patent rights by showing that the claimed subject matter was not patent-eligible, was not novel or was obvious or that the patent claims failed any other requirement for patentability or enforceability. In addition, we may in the future be subject to claims by our or our licensors' current or former employees or consultants asserting an ownership right in the patents or patent applications included in our patent rights as an inventor or co-inventor, as a result of the work they performed.

An adverse determination in any such submission or proceeding may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar therapeutics, without payment to us, or could limit the duration of the patent protection covering our product candidates. Such challenges may also result in our inability to manufacture or commercialize our product candidates without infringing third-party patent rights, and we may be required to obtain a license from third parties, which may not be available on commercially reasonable terms or at all, or we may need to cease the development, manufacture and commercialization of one or more of our product candidates. In addition, if the breadth or strength of protection provided by the patents and patent applications included in our patent rights is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates. Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us.

Even if they are unchallenged, the patents and pending patent applications included in our patent rights may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our patent rights by developing similar or alternative therapeutics in a non-infringing manner. For example, a third party may develop a competitive therapeutic that provides benefits similar to one or more of our product candidates but that uses a capsid or an expression construct that falls outside the scope of our patent protection. If the patent protection provided by the patents and patent applications we license or pursue with respect to our product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our product candidates could be negatively affected.

Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.

We currently depend, and will continue to depend, on our license, collaboration and other similar agreements. Further development and commercialization of our Candidates and platform technologies may require us to enter into additional license, collaboration or other similar agreements. The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, impact our ability to sublicense the relevant

intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

If any of our licenses or material relationships are terminated or breached, we may:

- lose our rights to develop and market our Candidates;
- lose patent protection for our Candidates;
- experience significant delays in the development or commercialization of our Candidates;
- not be able to obtain any other licenses on acceptable terms, if at all; or
- incur liability for damages.

These risks apply to any agreements that we may enter into in the future for our Candidates.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We have certain obligations under licensing agreements with third parties that include annual maintenance fees and payments that are contingent upon achieving various development, commercial and regulatory milestones. Pursuant to many of these license agreements, we are required to make milestone payments if certain development, regulatory and commercial sales milestones are achieved, and may have certain additional research funding obligations. Also, pursuant to the terms of many of these license agreements, when and if commercial sales of a licensed product commence, we must pay royalties to our licensors on net sales of the respective licensed products.

We have entered into, or plan to enter into, license agreements with third parties and may need to obtain additional licenses from one or more of these same third parties or from others to advance our research or allow our commercialization of our Candidates. It is possible that we may be unable to obtain such licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be required to expend significant time and resources to redesign Candidates or the methods for manufacturing them or to develop or license replacement products, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize our Candidates. We cannot provide any assurances that third-party patents or other intellectual property rights do not exist that might be enforced against our manufacturing methods, product candidates or any technologies we may develop, resulting in either an injunction prohibiting our manufacture or sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

In each of our existing license agreements, and we expect in our future agreements, patent prosecution of our licensed technology is controlled solely by the licensor, and we may be required to reimburse the licensor for their costs of patent prosecution. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. Further, in certain of our license agreements our licensors have the first right to bring any actions against any third party for infringing on the patents we have licensed. Our license agreements also require us to meet development thresholds to maintain the license, including establishing a set timeline for developing and commercializing product candidates. Disputes may arise regarding intellectual property subject to our licensing agreements, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our products or processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship or ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of licensed patented inventions.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize our Candidates. In spite of our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements, thereby resulting in disputes or litigation, which could cause us to incur substantial costs and distract management's time, and if we are unsuccessful, we could lose our ability to develop and commercialize products covered by these license agreements. If these licenses are ultimately terminated by the licensor, or if the underlying patents fail to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours.

Third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability and the ability of our future collaborators to develop, manufacture, market and sell our product candidates without infringing, misappropriating or otherwise violating the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We or our licensors may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our Candidates, including interference proceedings, post grant review and *inter partes* review before the USPTO. Our competitors or other third parties may assert infringement claims against us, alleging that, among other things, our therapeutics, manufacturing methods, formulations or administration methods are covered by their patents.

Given the vast number of patents in our field of technology, we cannot be certain or guarantee that a court would hold that any of our Candidates do not infringe an existing patent or a patent that may be granted in the future. Many companies and institutions have filed, and continue to file, patent applications related to gene therapy and related manufacturing methods. Some of these patent applications have already been allowed or issued and others may issue in the future. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed and additional patents granted in the future, as well as additional research and development programs expected in the future. Furthermore, because patent applications can take many years to issue, may be confidential for 18 months or more after filing and can be revised before issuance, there may be applications now pending that may later result in issued patents that may be infringed by the manufacture, use, sale or importation of our product candidates and we may or may not be aware of such patents. If a patent holder believes the manufacture, use, sale or importation of one of our product candidates infringes its patent, the patent holder may sue us even if we have licensed other patent protection for our product candidates. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant product revenue and against whom our licensed patent portfolio may therefore have no deterrent effect.

It is also possible that we have failed to identify relevant third-party patents or applications for which we may need a license to develop and commercialize our Candidates. For example, applications filed before November 29, 2000, and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to our product candidates because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our product candidates. In addition, we may be unaware of one or more issued patents that would be infringed by the manufacture, sale or use of a current or future product candidate, or we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our product candidates.

Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent or other intellectual property rights against us. For example, third parties may claim that gene or protein of interest, such as microdystrophin, or the AAV capsids we are developing for use in our Candidates are covered by patents held by them. Even if we believe such claim, or other intellectual property claims alleged by third parties, are without merit, there is no assurance that we would be successful in defending such claims. A court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could materially and adversely affect our ability to commercialize our Candidates covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance

that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. Similarly, there is no assurance that a court of competent jurisdiction would find that our product candidates did not infringe a third-party patent.

Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcome is uncertain. If we are found, or believe there is a risk that we may be found, to infringe, misappropriate or otherwise violate a third party's intellectual property rights, and we are unsuccessful in demonstrating that such intellectual property rights are invalid or unenforceable, we could be required or may choose to obtain a license from such third party to continue developing, manufacturing and marketing our product candidates. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing product candidate. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement, misappropriation or other violation of intellectual property rights, or claims that we have done so, could prevent us from manufacturing and commercializing our product candidates or force us to cease some or all of our business operations.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Litigation or other legal proceedings relating to intellectual property claims, with or without merit, is unpredictable and generally expensive and time-consuming. Competitors may infringe patents that we may own in the future or the patents of our licensing partners or we may be required to defend against claims of infringement. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing or misappropriating or successfully challenging our intellectual property rights. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our licensed patents and applications and any patents and patent applications we may own in the future. The USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable intellectual property law firms and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could have a material adverse effect on our business.

Some intellectual property that we have in-licensed may have been discovered through government-funded programs and thus may be subject to federal regulations such as "march-in" rights, certain reporting requirements, and a preference for U.S. manufacturing. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non-U.S. manufacturers.

Some of the intellectual property rights we have licensed, including such rights licensed from the University of Missouri, the University of Washington and the University of Florida, are stated to have been generated through the use of U.S. government funding and may therefore be subject to certain federal regulations. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh-Dole Act of 1980, or Bayh-Dole Act. These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive or non-exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention, (ii) government action is necessary to meet public health or safety needs or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in rights"). The U.S. government also has the right to take title to these inventions if we, or the applicable licensor, fail to disclose the invention to the government and fail to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us or the applicable licensor to expend substantial resources. In addition, the U.S. government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the United States. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply.

We may not be able to protect our intellectual property and proprietary rights throughout the world.

Filing, prosecuting, maintaining, enforcing and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States could be less extensive than those in the United States. Although our license agreements grant us worldwide rights, certain of our in-licensed U.S. patents lack corresponding foreign patents or patent applications. For example, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States even in jurisdictions where we and our licensors pursue patent protection. Consequently, we and our licensors may not be able to prevent third parties from practicing our inventions in all countries outside the United States, even in jurisdictions where we and our licensors pursue patent protection, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our inventions in jurisdictions where we and our licensors have not pursued and obtained patent protection to develop their own products and may export otherwise infringing products to territories where we have patent protection, but where enforcement is not as strong as it is in the United States. These products may compete with our product candidates and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property rights, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents, if pursued and obtained, or the marketing of competing products in violation of our intellectual property and proprietary rights generally. Proceedings to enforce our intellectual property and proprietary rights in foreign jurisdictions could (i) result in substantial costs and divert our efforts and attention from other aspects of our business, (ii) put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and (iii) provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of the discovery and development processes of our Candidates or technology platforms that involve proprietary know-how, information or technology that is not covered by patents. Aspects of our manufacturing process are protected by trade secrets. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets.

We seek to protect our proprietary know-how, trade secrets and processes, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our employees, consultants, scientific advisors, CROs, manufacturers and contractors. These agreements typically limit the rights of third parties to use or disclose our confidential information. However, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements, despite the existence generally of confidentiality agreements and other contractual restrictions. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary processes. Monitoring unauthorized uses and disclosures is difficult and we do not know whether the steps we have taken to protect our proprietary know-how and trade secrets will be effective. If any of our employees, collaborators, CROs, manufacturers, consultants, advisors and other third parties who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time-consuming, and the outcome is unpredictable. As a result, we could lose our trade secrets. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these security measures, they may still be breached, and we may not have adequate remedies for any breach.

In addition, our trade secrets may otherwise become known or be independently discovered by competitors. Competitors could purchase our product candidates, if approved, and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe, misappropriate or otherwise violate our intellectual property rights, design around our protected know-how and trade secrets, or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate such trade secrets, from using that technology or information to compete with us. If our trade secrets are not adequately protected so as to protect our market against competitors' products and technologies, our competitive position could be adversely affected.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Certain of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors, as well as our academic partners. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. An inability to incorporate such technologies or features would have a material adverse effect on our business and may prevent us from successfully commercializing our product candidates. Moreover, any such litigation or the threat of such litigation may adversely affect our ability to hire employees or contract with independent contractors. A loss of key personnel or their work product could hamper or prevent our ability to commercialize our product candidates. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. Moreover, even when we obtain agreements assigning intellectual property to us, the assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Moreover, individuals executing agreements with us may have preexisting or competing

obligations to a third party, such as an academic institution, and thus an agreement with us may be ineffective in perfecting ownership of inventions developed by that individual.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

Changes in either the patent laws or the interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes several significant changes to U.S. patent law. Prior to March 2013 in the United States, assuming that other requirements for patentability are met, the first to make the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith Act, the United States transitioned to a first inventor to file system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the invention. The Leahy-Smith Act also includes a number of significant changes that affect the way patent applications will be prosecuted and may also affect patent litigation. These include allowing third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent through various post-grant proceedings administered by the USPTO. The USPTO developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business as, among other reasons, the USPTO must still implement various regulations. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

The patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Two cases involving diagnostic method claims and “gene patents” have been decided by the U.S. Supreme Court. On March 20, 2012, the U.S. Supreme Court issued a decision in Mayo Collaborative Services v. Prometheus Laboratories, Inc., or Prometheus, a case involving patent claims directed to a process of measuring a metabolic product in a patient to optimize a drug dosage for the patient. According to the U.S. Supreme Court, the addition of well understood, routine or conventional activity such as “administering” or “determining” steps was not enough to transform an otherwise patent-ineligible natural phenomenon into patent-eligible subject matter. On July 3, 2012, the USPTO issued a guidance memo to patent examiners indicating that process claims directed to a law of nature, a natural phenomenon or a naturally occurring relation or correlation that do not include additional elements or steps that integrate the natural principle into the claimed invention such that the natural principle is practically applied and the patent claim amounts to significantly more than the natural principle itself should be rejected as directed to patent-ineligible subject matter. On June 13, 2013, the U.S. Supreme Court issued its decision in Association for Molecular Pathology v. Myriad Genetics, Inc., or Myriad, a case involving patent claims held by Myriad Genetics, Inc. relating to the breast cancer susceptibility genes BRCA1 and BRCA2. Myriad held that an isolated segment of naturally occurring DNA, such as the DNA constituting the BRCA1 and BRCA2 genes, is not patent-eligible subject matter, but that complementary DNA may be patent-eligible.

In 2014, the USPTO issued a guidance to its patent examiners for evaluating claims for patent subject matter eligibility under the relevant statute (35 U.S.C. § 101). This guidance was in response to a series of decisions from the U.S. Supreme Court on patent claims reciting judicial exceptions, including Abstract Ideas, Laws of Nature/Natural Principles, Natural Phenomena and/or Natural Products. Based on judicial decisions and public feedback, several supplements to this guidance and additional memoranda and materials have since been issued and are continually being issued, while the current eligibility guidance has been incorporated into the latest (10th) edition of the MPEP (Manual for Patent Examination Procedure), last revised in June 2020. The current subject matter eligibility guideline instructs USPTO examiners to follow a two-part test, set forth in the U.S. Supreme Court decisions Alice/Mayo, as the only test that should be used to evaluate the eligibility of claims under examination, including claims directed to natural products and principles including all naturally occurring nucleic acids. Certain claims of our licensed patents and patent applications contain, and any future patents we may obtain may contain, claims that relate to specific recombinant DNA sequences that are naturally occurring at least in part and, therefore, could be the subject of future challenges made by third parties. In addition, the current USPTO subject matter eligibility guidance and the constantly evolving case law, together with contemplated congressional action, could all impact our ability to pursue similar patent claims in patent applications we may prosecute in the future.

We cannot assure our stockholders that our efforts to seek patent protection for our product candidates will not be negatively impacted by the decisions described above, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what impact the U.S. Supreme Court’s decisions in Prometheus and Myriad may have on the ability of life science companies to obtain or enforce patents relating to their products in the future. These decisions,

the guidance issued by the USPTO and rulings in other cases or changes in USPTO guidance or procedures could have a material adverse effect on our existing patent rights and our ability to protect and enforce our intellectual property in the future.

Moreover, although the U.S. Supreme Court has held in Myriad that isolated segments of naturally occurring DNA are not patent-eligible subject matter, certain third parties could allege that activities that we may undertake infringe other gene-related patent claims, and we may deem it necessary to defend ourselves against these claims by asserting non-infringement and/or invalidity positions, or paying to obtain a license to these claims. In any of the foregoing or in other situations involving third-party intellectual property rights, if we are unsuccessful in defending against claims of patent infringement, we could be forced to pay damages or be subjected to an injunction that would prevent us from utilizing the patented subject matter.

If we do not obtain patent term extension for patents relating to our product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of our Candidates, one or more U.S. patents that we license or may own in the future may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process based on the first regulatory approval for a particular drug or biologic. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. In addition, to the extent we wish to pursue patent term extension based on a patent that we in-license from a third party, we would need the cooperation of that third party. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may be able to enter the market sooner.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition and our business may be adversely affected.

We have registered trademarks with the USPTO for the marks “SOLID BIOSCIENCES”, and “SOLID BIOSCIENCES” logo and registered marks in foreign jurisdictions for “SOLID BIOSCIENCES”, “SOLID GT” and “SOLID BIOSCIENCES” logo. Once registered, our trademarks or trade names may be challenged, infringed, diluted, tarnished, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement, dilution or tarnishment claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources.

Intellectual property rights and regulatory exclusivity rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make gene therapy products that are similar to our candidates but that are not covered by the claims of the patents that we license or may own in the future;
- we, or our current or future license partners or collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent applications that we license or may own in the future;

- we, or our current and future license partners or collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative products or duplicate any of our processes without infringing our owned or licensed intellectual property rights;
- others may circumvent our regulatory exclusivities, such as by pursuing approval of a competitive product candidate via the traditional approval pathway based on their own clinical data, rather than relying on the abbreviated pathway provided for biosimilar applicants;
- it is possible that our pending licensed patent applications or those that we may own in the future will not lead to issued patents;
- issued patents that we hold rights to now or in the future may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- others may have access to the same intellectual property rights licensed to us;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents or other intellectual property rights of others may have an adverse effect on our business; and
- we may choose not to file a patent for certain trade secrets or know how, and a third party may subsequently file a patent covering such intellectual property.

If approved, our product candidates that are licensed and regulated as biologics may face competition from biosimilars approved through an abbreviated regulatory pathway.

The Biologics Price Competition and Innovation Act of 2009, or BPCIA, was enacted as part of the Health Care Reform Law to establish an abbreviated pathway for the approval of biosimilar and interchangeable biological products. The regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as "interchangeable" based on its similarity to an approved biologic. Under the BPCIA, a reference biological product is granted 12 years of data exclusivity from the time of first licensure of the product, and the FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product In addition, the licensure of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still develop and receive approval of a competing biologic, so long as its BLA does not reply on the reference product, sponsor's data or submit the application as a biosimilar application.

In December 2022, Congress clarified through FDORA, that the FDA may approve multiple first interchangeable biosimilar biological products so long as the products are all approved on the same first day on which such a product is approved as interchangeable with the reference product and the exclusivity period may be shared amongst multiple first interchangeable products. More recently, in October 2023, the FDA issued its first interchangeable exclusivity determination under the BPCIA.

We believe that any of the product candidates we develop as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider the subject product candidates to be reference products for competing products, potentially creating the opportunity for biosimilar competition sooner than anticipated. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of the reference products in a way that is similar to traditional generic substitution for non-biological products will depend on a number of marketplace and regulatory factors that are still developing. Nonetheless, the approval of a biosimilar to our product candidates would have a material adverse impact on our business due to increased competition and pricing pressure.

Risks related to ownership of our common stock

Our executive officers, directors and principal stockholders maintain the ability to control or significantly influence all matters submitted to our stockholders for approval.

Our executive officers and directors and principal stockholders, in the aggregate, beneficially own shares representing a significant percentage of our capital stock. As a result, if these stockholders were to choose to act together, they would be able to control or significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control or significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets.

This concentration of voting power may:

- delay, defer or prevent a change in control;
- entrench our management and our Board of Directors; or
- delay or prevent a merger, consolidation, takeover or other business combination involving us on terms that other stockholders may desire.

A significant number of our total outstanding shares may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is performing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. Our outstanding shares of common stock may be freely sold in the public market at any time to the extent permitted by Rules 144 and 701 under the Securities Act of 1933, as amended, or the Securities Act, or to the extent such shares have already been registered under the Securities Act and are held by non-affiliates of ours. Moreover, holders of a substantial number of shares of our common stock have rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders.

In October 2020, in connection with the execution of our collaboration and license agreement with Ultragenyx, we issued and sold 521,719 shares of our common stock to Ultragenyx. For the ten-year period after date of such sale, subject to specified conditions, we have agreed to file a registration statement in order to register all or a portion of the shares sold to Ultragenyx.

In July 2019, December 2020 and January 2024, we completed private placements of shares of our common stock and pre-funded warrants to purchase shares of our common stock to several accredited investors. In December 2022, we also issued shares of our common stock in the Acquisition and in a related private placement to several accredited investors. We have filed registration statements covering the resale of these shares by the purchasers in these private placements and the stock consideration issued in the Acquisition, and have agreed to keep such registration statements effective until the date the shares covered by the respective registration statement have been sold or can be resold without restriction under Rule 144 of the Securities Act.

In addition, we have filed registration statements registering all shares of common stock that we may issue under our equity compensation plans. These shares can be freely sold in the public market upon issuance, subject to black-out periods and volume limitations applicable to affiliates.

We currently have on file with the SEC a universal shelf registration statement which allows us to offer and sell registered common stock, preferred stock, debt securities, depositary shares, warrants and/or units from time to time pursuant to one or more offerings at prices and terms to be determined at the time of sale.

The price of our common stock has been, and in the future is likely to be, volatile and fluctuate substantially, which could result in substantial losses for holders of our common stock.

Our stock price has been, and in the future is likely to be, volatile. The stock market in general and the market for biopharmaceutical or pharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their shares of common stock at or above the price they paid for their shares. The market price for our common stock may be influenced by many factors, including:

- our ability to achieve the anticipated benefits of the Acquisition and to successfully implement our proposed business strategy;
- results of or developments in preclinical studies and clinical trials of our Candidates or those of our competitors;
- the success of competitive products or technologies;
- the effect of public health emergencies or pandemics, such as the recent COVID-19 pandemic, on both the healthcare system and the patient population;
- regulatory or legal developments in the United States, the European Union and other countries;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates, or our clinical development programs and our commercialization efforts;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates;
- actual or anticipated changes in our development timelines;
- our ability to raise additional capital;
- our inability to obtain or delays in obtaining adequate product supply for any approved product or inability to do so at acceptable prices;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our product candidates;
- significant lawsuits, including patent or stockholder litigation;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of health care payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions;
- the liquidity for our stock and daily share volumes transacted;
- our ability to maintain our listing on the Nasdaq Global Select Market; and
- the other factors described in this "Risk Factors" section.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially.

In the past, following periods of volatility in the market price of a company's securities, securities class-action litigation often has been instituted against that company. We and certain of our executive officers and board members have previously been named as defendants in purported class action lawsuits. Any such litigation instituted against us could cause us to incur substantial costs to defend such claims and divert management's attention and resources.

An active trading market for our common stock may not be sustained.

Although our common stock is listed on the Nasdaq Global Select Market, given the limited trading history of our common stock, there is a risk that an active trading market for our shares may not continue to develop or be sustained. If an active market for our common stock does not continue to develop or is not sustained, it may be difficult for our stockholders to sell shares without depressing the market price for the shares, if at all.

We are a "smaller reporting company" and the reduced disclosure requirements applicable to smaller reporting companies may make our common stock less attractive to investors.

We are a smaller reporting company, and we will remain a smaller reporting company so long as the market value of our common stock held by non-affiliates is less than $250 million measured on the last business day of our second fiscal quarter, or our annual revenues are more than $100 million during the most recently completed fiscal year and the market value of our common stock held by non-affiliates is more than $700 million measured on the last business day of our second fiscal quarter. Smaller reporting companies are able to provide simplified executive compensation disclosure and have certain other reduced disclosure obligations, including, among other things, being permitted to provide only two years of audited financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations"; not being required to furnish a contractual obligations table in "Management's Discussion and Analysis of Financial Condition and Results of Operations"; and not being required to furnish a stock performance graph in our annual report.

We may choose to take advantage of some, but not all, of the available exemptions. We have taken advantage of reduced reporting burdens in our filings with the SEC. We cannot predict whether investors will find our common stock less attractive if we rely on certain or all of these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

We incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to new compliance initiatives.

As a public company, we incur significant legal, accounting and other expenses. Those expenses will increase if we do not remain a smaller reporting company. In addition, the Sarbanes-Oxley Act and rules subsequently implemented by the SEC and Nasdaq have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance.

Pursuant to Section 404, we are required to furnish a report by our management on our internal control over financial reporting. However, while we remain a smaller reporting company with less than $100 million in revenue, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

Provisions in our certificate of incorporation and our bylaws and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our certificate of incorporation and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our Board of Directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our Board of Directors. Among other things, these provisions:

- establish a classified Board of Directors such that not all members of our board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our Board of Directors;
- limit the manner in which stockholders can remove directors from the board;

- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our Board of Directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our Board of Directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our Board of Directors; and
- require the approval of the holders of at least two-thirds of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our certificate of incorporation or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, or the DGCL, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, is the only sole source of gain for an investment in our common stock.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for an investor for the foreseeable future.

Our certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for certain litigation that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for such disputes with us or our directors, officers or employees.

Our certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware is the exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim for breach of a fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the DGCL, our certificate of incorporation or our bylaws or (iv) any action asserting a claim governed by the internal affairs doctrine. We do not intend to have this choice of forum provision apply to, and this choice of forum provision will not apply to, actions arising under the Securities Act or the Exchange Act. The choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. Alternatively, if a court were to find the choice of forum provision contained in our certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions.

Item 1B. Unresolved Staff Comments.

None.

Item 1C. Cybersecurity

We have certain processes for assessing, identifying and managing cybersecurity risks, which are built into our information technology function and are designed to help protect our information assets and operations from internal and external cyber threats, as well as secure our networks, systems and data. Such processes include physical, procedural, and technical safeguards, employee training and incident simulations. We have the capacity to engage certain external parties, such as consultants, independent privacy assessors, computer security firms and risk management, peer companies, industry groups and governance experts, to enhance our cybersecurity oversight. We also consider the internal risk oversight programs of third-party service providers before engaging them to help protect us from any related vulnerabilities.

To help manage our material risks from cybersecurity threats and to help protect against, detect, and prepare to respond to cybersecurity incidents, we conduct periodic training for employees involved in our systems and processes that handle sensitive data. We also conduct on-boarding cybersecurity awareness assessments, cybersecurity training for all employees, and regular phishing email simulations for all employees. In addition, we use technology-based tools to help mitigate cybersecurity risks and to bolster our employee-based cybersecurity programs.

The Audit Committee of our Board of Directors provides oversight of our cybersecurity risk and provides regular updates to the Board of Directors regarding such oversight. The Audit Committee receives periodic updates from management regarding cybersecurity matters, and is notified between such updates regarding significant new cybersecurity threats or incidents

Our Senior Director of Information Technology's leads the operational oversight of company-wide cybersecurity strategy, policy, standards and processes and works across relevant departments to assess and help us prepare our employees to address cybersecurity risks. Our Senior Director of Information Technology has over sixteen years of experience in information technology, ten years of experience in information technology for life sciences companies, and a relevant bachelor's degree in information technology. We do not believe that there are currently any known risks from cybersecurity threats that are reasonably likely to materially affect us or our business strategy, results of operations or financial condition.

Item 2. Properties.

We lease our corporate headquarters, which consists of approximately 49,869 square feet of office, laboratory, research and development and manufacturing space in Charlestown, Massachusetts. The lease for our corporate headquarters has an initial term of approximately ten years that expires in 2032 with an option to extend the lease for an additional five years. In addition, we lease smaller laboratory and office space in North Carolina and Florida.

Item 3. Legal Proceedings.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

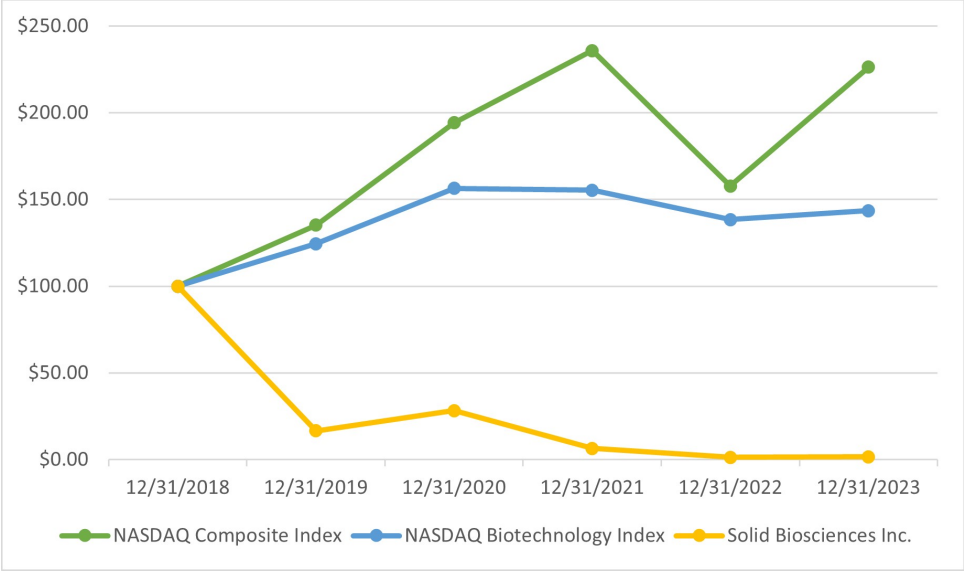
Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information

Our common stock has been publicly traded on the Nasdaq Global Select Market under the symbol "SLDB" since January 26, 2018 in connection with our initial public offering. Prior to that date, there was no established public trading market for our common stock.

The following graph compares the performance of our common stock to The Nasdaq Composite Index and to The Nasdaq Biotechnology Index from December 31, 2018 through December 31, 2023. The comparison assumes $100 was invested after the market closed on December 31, 2018 in our common stock and in each of the foregoing indices, and it assumes reinvestment of dividends, if any. The stock price performance included in this graph is not necessarily indicative of future stock price performance.

COMPARISON OF CUMULATIVE TOTAL RETURN
Among The Nasdaq Composite Index, The Nasdaq Biotechnology Index and Solid Biosciences Inc.



The performance graph in this Item 5 is not deemed to be "soliciting material" or to be "filed" with the SEC for purposes of Section 18 of the Exchange Act, or otherwise subject to the liabilities under that Section, and shall not be deemed incorporated by reference into any of our filings under the Securities Act or the Exchange Act, except to the extent we specifically incorporate it by reference into such a filing.

Holders

As of February 13, 2024, we had approximately 57 holders of record of our common stock. This number does not include beneficial owners whose shares were held in street name. The actual number of holders of our common stock is greater than this number of record holders and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers or held by other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Purchase of Equity Securities

We did not purchase any of our registered equity securities during the period covered by this Annual Report on Form 10-K.

Recent Sales of Unregistered Securities

We did not sell any securities, during the year ended December 31, 2023 that were not registered under the Securities Act of 1933, as amended, or the Securities Act, and that have not otherwise been described in a Quarterly Report on Form 10-Q or in a Current Report on Form 8-K.

Item 6. Reserved.

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Annual Report on Form 10-K, our actual results could differ materially from the results described, in or implied, by these forward-looking statements.

Unless otherwise indicated, all information in this Annual Report on Form 10-K gives effect to a 1-for-15 reverse stock split of our common stock that became effective on October 27, 2022, and all references to historical share and per share amounts give effect to the reverse stock split.

Overview

We are a life sciences company focused on advancing a portfolio of current and future gene therapy candidates, which we refer to collectively as our Candidates, including SGT-003 for the treatment of Duchenne muscular dystrophy, or Duchenne, SGT-501 for the treatment of Catecholaminergic polymorphic ventricular tachycardia, or CPVT, and additional assets for the treatment of cardiac and other diseases, at different stages of development, with varying levels of investment. We are advancing our diverse pipeline across rare neuromuscular and cardiac diseases, bringing together experts in science, technology, disease management and care. Patient-focused and founded by those directly impacted by Duchenne, our mission is to improve the daily lives of patients living with these devastating diseases.

Solid was purpose-built to advance the best science and accelerate the discovery and development of treatments that may benefit all patients with Duchenne. As Solid expands to bring meaningful treatments to patients living with other neuromuscular and cardiac diseases, the values and guiding principles that drive us continue. Our corporate vision is to build an innovation platform enabling the discovery and development of high-value genetic medicines for neuromuscular and cardiac diseases by integrating internal capabilities, including a vector core, use of validated animal models, optimized expression cassettes, novel capsids and regulatory expertise, and collaborations with leaders in related clinical and research fields. Our mission, which guides our operations, is to treat and change the course of neuromuscular and cardiac diseases at all stages. Underscoring this mission, our disease-focused business model is founded on the following fundamental principles:

- identify and develop meaningful therapies for patients with neuromuscular and cardiac diseases;
- bring together the leading experts in neuromuscular and cardiac diseases, science, technology, disease management and care; and
- be guided by the needs of these patients.

On December 2, 2022, we completed our acquisition of AavantiBio, Inc., or AavantiBio, a privately held gene therapy company focused on transforming the lives of patients with Friedreich's ataxia, or FA, and rare cardiomyopathies, or the Acquisition. Upon the consummation of the Acquisition, we acquired AavantiBio's gene therapy programs, AVB-202-TT for the treatment of FA and AVB-401 for the treatment of BAG3-mediated dilated cardiomyopathy ("DCM"), additional assets for the treatment of cardiac diseases, platform technologies and know-how related thereto.

Our Operations

We are focused on developing transformative treatments to improve the lives of patients with rare neuromuscular and cardiac diseases. Our current programs are all designed to treat these diseases with gene transfer products. Gene transfer, a type of gene therapy, is designed to address diseases caused by mutated genes through the delivery of functional versions of those genes, called transgenes. The transgenes are then utilized by the body to produce proteins that are absent or not functional prior to treatment, potentially offering long-lasting clinical benefit. In addition to a transgene, our gene transfer candidates include a viral capsid or vector (a protein shell utilized as a vehicle to deliver a transgene to cells in the body) and a promoter (a specialized DNA sequence that directs cells to produce the protein in specific tissues). The capsid is modified to no longer self-replicate yet still retain its ability to introduce new genetic material directly into patients' cells. Adeno-associated virus, or AAV, capsids have been approved for use to deliver transgenes to patients, including via systemic delivery. The use of AAV capsids to deliver gene therapies has also been extensively studied by third parties in human clinical trials for multiple disease indications, and in certain of these trials AAV was delivered systemically to the patient.

Due to our significant research and development expenditure, licensing and patent investment, and general administrative costs associated with our operations, we have generated substantial operating losses in each period since our inception. Our net losses were $96.0 million, $86.0 million and $72.2 million for the years ended December 31, 2023, 2022

and 2021, respectively. As of December 31, 2023, we had an accumulated deficit of $658.8 million. We expect to incur significant expenses and operating losses for the foreseeable future.

As we seek to develop and commercialize our Candidates, we anticipate that our expenses will increase significantly and that we will need substantial additional funding to support our continuing operations. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of public or private equity financings, debt financings or other sources, which may include licensing agreements or strategic collaborations. We may be unable to raise additional funds or enter into such agreements or arrangements when needed on favorable terms, if at all. If we fail to raise capital or enter into such agreements as and when needed, we may have to significantly delay, scale back or discontinue the development or commercialization of our Candidates.

Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses or determine when or if we will be able to achieve or maintain profitability. Even if we are able to generate revenue from product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

As of December 31, 2023, we had cash, cash equivalents, and available-for-sale securities of $123.6 million, excluding restricted cash of $1.8 million. In January 2024, we sold to investors in a private placement, or the January 2024 Private Placement, an aggregate of 16,973,103 shares of our common stock at a price of $5.53 per share, and, to one investor in lieu of shares, pre-funded warrants to purchase 2,712,478 shares of our common stock, at a price of $5.529 per pre-funded warrant. We received approximately $104.0 million of aggregate net proceeds, after deducting offering costs. We believe that our cash, cash equivalents, and available-for-sale securities as of December 31, 2023, together with the net proceeds from the January 2024 Private Placement, will enable us to fund our operating expenses and capital expenditure requirements into 2026. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently anticipate.

Financial operations overview

Revenue

Collaboration revenue

There was no collaboration revenue for the year ended December 31, 2023. Collaboration revenue was $8.1 million for the year ended December 31, 2022. We recognized this revenue related to research services and cost reimbursement from the collaboration and license agreement, or the Collaboration Agreement, with Ultragenyx Pharmaceutical Inc., or Ultragenyx. No other research and development has commenced under the Collaboration Agreement.

Product revenue

We have not generated any product revenue to date and do not expect to generate any product revenue from the sale of our products, if approved, for the foreseeable future, if ever. If our development efforts for our Candidates are successful and result in marketing approval, we may generate product revenue in the future from product sales.

Operating expenses

We classify our operating expenses into two categories: research and development, and general and administrative expenses. Personnel costs, including salaries, benefits, bonuses and equity-based compensation expense, comprise a significant component of each of these expense categories. We allocate expenses associated with personnel costs based on the nature of work associated with these resources.

Research and development expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our discovery efforts, and the development of SGT-003 and other Candidates and include:

- expenses incurred under agreements with third parties, including contract research organizations, or CROs, that conduct research and preclinical activities on our behalf, as well as contract manufacturing organizations, or CMOs, that manufacture SGT-003 and other Candidates for use in our preclinical studies and clinical trials;

- salaries, benefits and other related costs, including equity-based compensation expense, for personnel engaged in research and development functions;
- costs of outside consultants, engaged to assist in our research and development activities, including their fees, equity-based compensation and related travel expenses;
- costs of laboratory supplies and acquiring, developing and manufacturing preclinical study and clinical trial materials;
- costs incurred in seeking regulatory approval of SGT-003 and other Candidates;
- expenses incurred under our intellectual property licenses; and
- facility-related research and development expenses, which include direct depreciation costs and allocated expenses for rent and maintenance of facilities and other operating costs.

Research and development activities are central to our business model. We are still in the early stages of development of our Candidates. Product candidates in later stages of clinical development generally have higher development costs than those in preclinical development or in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect that our research and development expenses will continue to increase for the foreseeable future if and as we conduct clinical trials for SGT-003, initiate clinical trials for our other Candidates and continue to identify and develop additional candidates.

We typically use our employee and infrastructure resources across our product candidates. We track outsourced development costs and milestone payments made under our licensing arrangements by product candidates, but we do not allocate personnel costs, license payments made under our licensing arrangements or other internal costs to product candidates on a program-specific basis. These costs are included in unallocated research and development expenses in the table below.

The following table summarizes our research and development expenses by product candidates for the respective periods:

	For the Year Ended December 31,	
(in thousands)	**2023**	**2022**
SGT-001	$ 3,432	$ 24,844
SGT-003	20,856	9,995
SGT-501	3,200	-
Other development programs	7,439	3,450
Unallocated research and development expenses		
Personnel related expenses	24,968	24,883
External expenses	16,668	15,248
Total unallocated research and development expenses	41,636	40,131
Total research and development expenses	$ 76,563	$ 78,420

We cannot determine with certainty the duration, costs and timing of clinical trials of SGT-003 or other Candidates, or if, when or to what extent we will generate revenue from the commercialization and sale of any of our product candidates for which we obtain marketing approval or our other research and development expenses. We may never succeed in obtaining marketing approval for any of our product candidates. The duration, costs and timing of clinical trials and development of our candidates will depend on a variety of factors, including:

- the scope, rate of progress, expense and results of any clinical trials of our Candidates and other research and development activities that we may conduct;
- the imposition of regulatory restrictions on clinical trials, including full and partial clinical holds and the time and activities required to lift any such holds;
- uncertainties in clinical trial design and patient enrollment or drop out or discontinuation rates;
- significant and changing government regulation and regulatory guidance;
- potential additional studies or clinical trials requested by regulatory agencies;
- the timing and receipt of any marketing approvals; and
- the expense of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights.

General and administrative expenses

General and administrative expenses consist primarily of salaries and other related costs, including equity-based compensation, for personnel in our executive, finance, business development and administrative functions. General and administrative expenses also include legal fees relating to patent and corporate matters, professional fees for accounting, auditing, tax and consulting services, insurance costs, travel expenses, acquisition costs, and facility-related expenses, which include direct depreciation costs and allocated expenses for rent and maintenance of office facilities and other operating costs.

We expect that our general and administrative expenses will increase in the future as we support our research and development activities and activities related to our INSPIRE Duchenne trial and any planned or future clinical trials for and potential commercialization of our Candidates.

Restructuring charges

In April 2022, we implemented changes to our corporate strategy. In connection with the changes to corporate operations, we reduced headcount by approximately 35 percent.

In November 2022, we approved a plan designed to streamline our operating structure in connection with the Acquisition. In connection with the plan, we reduced headcount by approximately 18 percent in December 2022.

Other income (expense), net

Other income (expense), net consists of interest income earned on our cash, cash equivalents, available-for-sale securities, amortization of investment premium or accretion of investment discount.

Income taxes

We account for income taxes using an asset and liability approach, which requires recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements but have not been reflected in taxable income. A valuation allowance is established to reduce deferred tax assets to their estimated realizable value.

We account for uncertainty in income taxes recognized in the financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Critical accounting policies and use of estimates

Our management's discussion and analysis of financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities, costs and expenses and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates.

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements appearing at the end of this Annual Report on Form 10-K, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements and that involve a significant level of estimation uncertainty.

Revenue recognition

As discussed in Note 2 to our consolidated audited financial statements, under Accounting Standards Codification, or ASC 606, Revenue from Contracts with Customers, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services.

To determine the appropriate amount of revenue to be recognized for arrangements determined to be within the scope of ASC 606, we perform the following five steps: (i) identification of the contract(s) with the customer, (ii) identification of the promised goods or services in the contract and determination of whether the promised goods or services are performance obligations, (iii) measurement of the transaction price, (iv) allocation of the transaction price to the performance obligations, and (v) recognition of revenue when (or as) we satisfy each performance obligation. We only apply the five-step model to contracts when it is probable that the entity will collect consideration it is entitled to in exchange for the goods or services it transfers to the customer.

When optional goods or services are offered, we assess the options to determine whether the options grant the customer a material right. This determination includes whether the option is priced at an amount that the customer would not have received without entering into the contract. If we conclude the option conveys a material right, it is accounted for as a separate performance obligation. In identifying performance obligations in a contract, we identify those promises that are distinct. Promised goods or services are considered distinct when the customer can benefit from the goods or services on their own, or together with readily available resources, and the goods or services are separately identifiable from other promises in the contract. If a promise is not distinct, it is combined with other promises in the contract until the combined group of promises is capable of being distinct.

We estimate the transaction price based on the amount of consideration we expect to receive for transferring the promised goods or services in the contract. The consideration may include both fixed consideration and variable consideration. At the inception of each arrangement that includes variable consideration, we evaluate the amount of the potential payments and the likelihood that the payments will be received. If it is probable that a significant revenue reversal would not occur, the variable consideration is included in the transaction price. For contracts that include sales-based royalties for licensed compounds, we recognize revenue at the date when the related sales occur. Finally, we determine whether the contract contains a significant financing component by analyzing the promised consideration relative to the standalone selling price of the promised goods and services and the timing of payment relative to the transfer of the promised goods and services. At each reporting date, we reassess the transaction price and probability of achievement of the performance obligations and the associated constraints on transaction price. If necessary, we adjust the transaction price, recording a cumulative catch-up based on progress for the amount that was previously constrained.

Revenue is recognized when (or as) control of a performance obligation is transferred to the customer. When combined performance obligations contain a promised license and related services or other promises, management judgment is required to determine the appropriate timing of revenue recognition. In doing so, we must identify the predominant promise or promises in the contract to determine whether revenue is recognized at a point in time or over time. If over time, we must determine the appropriate measure of progress. If a license is deemed to be the predominant promise in a performance obligation, we must determine the nature of the license, whether functional or symbolic intellectual property, to conclude whether point-in-time or over-time revenue recognition is most appropriate. The determination of functional or symbolic intellectual property requires an assessment of whether the customer is able to exploit and benefit from the license in its

current condition, or if the utility of the license is dependent on or influenced by our ongoing activities or being associated with us.

At each reporting date, we calculate the measure of progress for the performance obligations transferred over time. The calculation generally uses an input measure based on costs incurred to-date relative to estimated total costs to complete the transfer of the performance obligation. The measurement of progress is then used to calculate the total revenue earned, including any cumulative catch-up adjustment.

Accrued research and development expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advanced payments. We make estimates of our accrued expenses as of each balance sheet date in our consolidated financial statements based on facts and circumstances known to us at that time. Examples of estimated accrued research and development expenses include fees paid to:

- CROs in connection with performing research activities on our behalf and conducting clinical trials and preclinical studies on our behalf;
- vendors in connection with preclinical development activities;
- vendors related to product manufacturing and development and distribution of clinical and preclinical supplies; and
- third parties under our intellectual property licenses.

We base our expenses related to preclinical studies and clinical trials on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple CROs that conduct and manage preclinical studies and clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing fees, we estimate the time period over which services will be performed, and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or amount of prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in us reporting amounts that are too high or too low in any particular period.

Equity-based compensation

We have equity plans under which we make equity awards to employees, directors and non-employees. We measure all stock options and other stock-based awards granted to employees, directors and non-employees based on the fair value on the date of the grant and recognize compensation expense of those awards, over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. We apply the straight-line method of expense recognition to all awards with only service-based vesting conditions. We have not issued any awards with performance-based vesting conditions. For stock-based awards granted to non-employees, compensation expense is recognized over the period during which services are rendered by such non-employees until completed.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. We historically have been a private company and lack company-specific historical and implied volatility information. Therefore, we estimate our expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expect to continue to do so until such time as we have adequate historical data regarding the volatility of our own traded stock price. For options with service-based vesting conditions and options granted to non-employees, the expected term of our stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that we have never paid cash dividends and do not expect to pay any cash dividends in the foreseeable future, if ever.

Results of operations

Comparison of the years ended December 31, 2023 and 2022

The following table summarizes our results of operations for the years ended December 31, 2023 and 2022:

	For the Year Ended December 31,		Increase	%
	2023	2022	(decrease)	Change
(in thousands)				
Collaboration revenue - related party	$ —	$ 8,094	$ (8,094)	(100)%
Operating expenses:				
Research and development	76,563	78,420	(1,857)	(2)%
General and administrative	27,752	28,948	(1,196)	(4)%
Restructuring expense	(63)	7,178	(7,241)	(101)%
Total operating expenses	104,252	114,546	(10,294)	(9)%
Loss from operations	(104,252)	(106,452)	2,200	2%
Other income, net:				
Interest income, net	7,142	2,616	4,526	173%
Gain on acquisition	—	18,236	(18,236)	(100)%
Other income (expense)	1,095	(381)	1,476	(387)%
Total other income, net	8,237	20,471	(12,234)	(60)%
Net loss	$ (96,015)	$ (85,981)	$ (10,034)	(12)%

Collaboration revenue

There was no collaboration revenue for the year ended December 31, 2023, compared to $8.1 million of collaboration revenue for the year ended December 31, 2022. The decrease in collaboration revenue of $8.1 million was related to the completion of research and development services contemplated under the Collaboration Agreement during the second quarter of fiscal year 2022, resulting in the recognition of the remaining deferred revenue, which was recorded at the time the Collaboration Agreement was executed. No other research and development has commenced under the Collaboration Agreement.

Research and development expenses

(in thousands)	For the Year Ended December 31, 2023	2022	Increase (decrease)	% Change
SGT-001	$ 3,432	$ 24,844	$ (21,412)	(86)%
SGT-003	20,856	9,995	10,861	109%
SGT-501	3,200	-	3,200	100%
Other development programs	7,439	3,450	3,989	116%
Unallocated research and development expenses				
Personnel related expenses	24,968	24,883	85	0%
External expenses	16,668	15,248	1,420	9%
Total unallocated research and development expenses	41,636	40,131	1,505	4%
Total research and development expenses	$ 76,563	$ 78,420	$ (1,857)	(2)%

Research and development expenses for the year ended December 31, 2023 were $76.6 million, compared to $78.4 million for the year ended December 31, 2022. The decrease of $1.8 million in research and development expenses was primarily related to a $21.4 million decrease in costs for SGT-001 due to our decision to prioritize development of SGT-003, offset by an $11.0 million increase in manufacturing, clinical, and study related costs for SGT-003, a $4.0 million increase in costs for other development programs, a $3.2 million increase in costs for SGT-501, and a $1.4 million increase in external expenses.

General and administrative expenses

General and administrative expenses were $27.8 million for the year ended December 31, 2023, compared to $28.9 million for the year ended December 31, 2022. The decrease of $1.1 million was primarily related to a $1.6 million decrease in legal fees due to acquisition related costs occurring in 2022, a $1.0 million decrease in insurance premiums, a $0.4 million decrease in business development research, and a $0.1 million decrease in charitable contributions, offset by a $1.0 million increase in personnel related costs, a $0.4 million increase in IT related costs, a $0.3 million increase in recruiting costs, and a $0.3 million increase in facilities costs.

Restructuring charges

During the year ended December 31, 2023, restructuring charges were $(0.1) million compared to $7.2 million for the year ended December 31, 2022. The restructuring charges were related to severance and other employee-related costs in connection with the restructurings that occurred in April 2022 and December 2022. We paid $3.7 million during the year ended December 31, 2023 and $3.3 million during the year ended December 31, 2022.

Other income (expense)

Other income (expense) was $8.2 million and $20.5 million for the years ended December 31, 2023 and 2022, respectively. The decrease in other income was primarily related to the gain recorded in connection with the Acquisition of $18.2 million during the year ended December 31, 2022, partially offset by a $4.5 million increase in interest income primarily related to available-for-sale securities included within our portfolio, and a $1.5 million increase in other income primarily related to our sublease of space at our Massachusetts facility.

Results of operations—Years ended December 31, 2022 and 2021

Discussion and analysis of the year ended December 31, 2022 compared to the year ended December 31, 2021 is included in Part II, Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our Annual Report on Form 10-K for the year ended December 31, 2022 as filed with the SEC on March 23, 2023 (the “2022 Form 10-K”).

Liquidity and capital resources

Sources of liquidity

To date, we have financed our operations primarily through the sale of redeemable preferred units and member units, the sale of common stock and prefunded warrants to purchase shares of our common stock in private placements and the sale of common stock in our initial public offering and a follow-on public offering, and sales of common stock under our "at-the-market offering" sales agreement, dated March 13, 2019 and as amended on August 16, 2021, by and between us and Jefferies LLC, or Jefferies, or the ATM Sales Agreement. Through December 31, 2023, we raised an aggregate of $144.6 million of gross proceeds from our sales of preferred units prior to the completion of our initial public offering, and an aggregate of $546.8 million of net proceeds from the sale of our common stock through public offerings, including our IPO and follow-on public offering, private placements, the ATM Sales Agreement, and pursuant to the stock purchase agreement with Ultragenyx, as detailed in the following paragraphs.

On March 13, 2019, we entered into the ATM Sales Agreement, which was amended in August 2021, under which we may offer and sell, from time to time, shares of our common stock having aggregate gross proceeds of up to $75.0 million through Jefferies as sales agent. Any such sales being made by any method that is deemed an "at-the-market offering" as defined in Rule 415 promulgated under the Securities Act. We pay Jefferies a commission of up to 3% of the gross proceeds of any sales of common stock pursuant to the ATM Sales Agreement. During the years ended December 31, 2023 and 2022, we sold 602,030 and 0 shares of common stock, respectively, pursuant to the ATM Sales Agreement resulting in net proceeds of $3.0 million and $0, respectively. During the three months ended December 31, 2023, we sold 182,030 shares pursuant to the ATM Sales Agreement.

On March 23, 2021, we issued and sold in a public offering 1,666,666 shares of our common stock at a price per share of $86.25, including the full exercise by the underwriters of an option to purchase additional shares of common stock. We received net proceeds of approximately $134.9 million after deducting underwriting discounts and commissions and offering expenses.

On December 2, 2022, we issued and sold 10,638,290 shares of our common stock at a price per share of $7.05 in a private placement, or the December 2022 Private Placement, which closed immediately following the Acquisition. We received $72.6 million of net proceeds from the December 2022 Private Placement after deducting placement agent fees.

As of December 31, 2023, we had cash, cash equivalents and available-for-sale securities of $123.6 million, excluding restricted cash of $1.8 million, and had no debt outstanding.

On January 11, 2024, we issued and sold 16,973,103 shares of our common stock at a price per share of $5.53 and, to one investor in lieu of shares of common stock, pre-funded warrants to purchase 2,712,478 shares of common stock at a price of $5.529 per pre-funded warrant, in the January 2024 Private Placement. We received approximately $104.0 million of net proceeds from the January 2024 Private Placement after deducting offering costs.

Cash flows

The following table summarizes our sources and uses of cash for each of the periods presented:

	For the Year Ended December 31,	
	2023	2022
(in thousands)		
Net cash used in operating activities	$ (94,180)	$ (97,977)
Net cash provided by investing activities	9,689	59,157
Net cash provided by financing activities	3,122	74,831
Net (decrease) increase in cash, cash equivalents and restricted cash	$ (81,369)	$ 36,011

Operating activities

During the year ended December 31, 2023, operating activities used $94.2 million of cash, primarily resulting from our net loss of $96.0 million offset by non-cash charges of $8.2 million due primarily to equity-based compensation of $7.6 million and depreciation and impairment expense of $3.0 million, partially offset by amortization of discount on available-for sale-securities of $2.4 million. Net cash used by changes in our operating assets and liabilities was $6.3 million which included a decrease of $9.0 million in accrued other current and non-current liabilities, and a decrease in accounts payable of $0.7 million, partially offset by a decrease in prepaid and other non-current assets of $3.4 million.

During the year ended December 31, 2022, operating activities used $98.0 million of cash, primarily resulting from our net loss of $86.0 million and non-cash charges of $8.3 million due primarily to the gain on acquisition of $18.2 million, offset by equity-based compensation of $7.5 million and depreciation expense of $2.4 million. Net cash used by changes in our operating assets and liabilities was $3.7 million which included a decrease in deferred revenue of $8.1 million, a decrease in accounts receivable of $0.1 million as a result of the Ultragenyx Collaboration Agreement, and a decrease in accounts payable of $5.2 million due to the timing of payments, partially offset by an increase in prepaid and other non-current assets of $3.7 million and a decrease in accrued other liabilities and non-current liabilities of $5.8 million.

Investing activities

During the year ended December 31, 2023, investing activities provided cash of $9.7 million, consisting primarily of the sale of available-for-sale securities of $128.6 million, partially offset by net purchases of available-for-sale securities of $117.4 million and the purchase of property and equipment of $1.5 million primarily related to the corporate headquarters lease.

During the year ended December 31, 2022, investing activities provided cash of $59.2 million, consisting primarily of the sale of available-for-sale securities of $212.8 million, net cash received upon the closing of the Acquisition of $31.5 million, and cash proceeds from the sale of property and equipment of $0.6 million, partially offset by net purchases of available-for-sale securities of $182.8 million and the purchase of property and equipment of $3.0 million primarily related to our corporate headquarters lease.

Financing activities

During the year ended December 31, 2023, net cash provided by financing activities was $3.1 million as a result of proceeds from the issuance of common stock of $3.0 million and from the issuance of shares of $0.1 million under the Company's employee stock purchase plan.

During the year ended December 31, 2022, net cash provided by financing activities was $74.8 million as a result of the December 2022 Private Placement.

A discussion of changes in our cash flow from the year ended December 31, 2021 to the year ended December 31, 2022 can be found in Part II, Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations” of the 2022 Form 10-K.

Funding requirements

We expect our expenses to increase substantially in connection with our ongoing development activities related to our Candidates. In addition, we have incurred and expect to continue to incur costs associated with operating as a public company. We expect that our expenses will increase substantially if and as we:

- enroll patients in our INSPIRE Duchenne trial and advance clinical development of SGT-003;
- advance our other Candidates into clinical trials;
- continue research and preclinical development of our Candidates and adjacent technologies such as assays;
- seek to identify additional candidates;
- engage in regulatory interactions with the FDA and other regulatory authorities;
- submit regulatory filings relating to the development of our Candidates and seek marketing approvals for our Candidates that successfully complete clinical trials, if any;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- arrange manufacturing for larger quantities of our product candidates for preclinical and clinical development and potential commercialization;
- maintain, expand, protect and enforce our intellectual property portfolio;
- hire and retain additional clinical, quality control and scientific personnel;
- build out new facilities or expand existing facilities to support our activities;
- acquire or in-license other drugs, drug candidates, technologies and intellectual property;
- fund a portion of the development or commercialization of products in collaboration with Ultragenyx pursuant to the Collaboration Agreement; and
- add operational, financial and management information systems and personnel.

As of December 31, 2023, we had cash, cash equivalents and available-for-sale securities of $123.6 million, excluding restricted cash of $1.8 million. Based on our current operating plan, we believe that our cash, cash equivalents and available-for-sale securities as of December 31, 2023, together with the net proceeds from our January 2024 Private Placement, will be sufficient to fund our operating expenses and capital requirements into 2026. As a result, in order to continue to operate our business beyond that time, we will need to raise additional funds. However, there can be no assurance that we will be able to generate funds on terms acceptable to us, on a timely basis, or at all. In addition, we have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently anticipate.

Because of the numerous risks and uncertainties associated with the development of our Candidates and because the extent to which we may enter collaborations with third parties for development of our product candidates is unknown, we are

unable to estimate the timing and amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. Our future capital requirements will depend on many factors, including:

- the progress, costs and results of the INSPIRE Duchenne trial and any future clinical trials of our Candidates;
- the costs, timing and outcome of regulatory review of our Candidates;
- the scope, progress, results and costs of discovery, laboratory testing, manufacturing, preclinical development and clinical trials for our Candidates;
- the costs associated with manufacturing and use of third-party manufacturers;
- the revenue, if any, received from commercial sale of our Candidates, should any of our Candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining, defending and enforcing our intellectual property rights and defending intellectual property-related claims;
- the outcome of any lawsuits filed against us;
- the terms of our current and any future license agreements and collaborations;
- the success of our collaboration with Ultragenyx;
- our ability to establish and maintain additional strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the payment or receipt of milestones, royalties and other collaboration-based revenues, if any;
- the extent to which we acquire or in-license other candidates, technologies and intellectual property; and
- if and as we need to adapt our business in response to public health emergencies or pandemics, such as the recent COVID-19 pandemic, and collateral consequences related thereto.

We are supplying, and expect to continue to supply, our ongoing and future clinical development programs with drug produced at a cGMP compliant facility located at one of our CMOs. We intend to establish the capability and capacity to supply Candidates at commercial scale from multiple sources.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval for any product candidates or generate revenue from the sale of any products for which we may obtain marketing approval. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for many years, if ever. Accordingly, we will need to obtain substantial additional funds to achieve our business objectives.

Adequate additional funds may not be available to us on acceptable terms, or at all. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity securities, our existing stockholders' ownership interest may be diluted. Any debt or preferred equity financing, if available, may involve agreements that include restrictive covenants that may limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, which could adversely impact our ability to conduct our business, and may require the issuance of warrants, which could potentially dilute existing stockholders' ownership interests.

If we raise additional funds through licensing agreements and strategic collaborations with third parties, we may have to relinquish valuable rights to our technology, future revenue streams, research programs, or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds, we may be required to delay, limit, reduce and/or terminate development of our product candidates or any future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual obligations and commitments

We lease certain office space, lab space and lab equipment in Massachusetts, North Carolina and Florida. These leases are used for our continuing operations. For a description of our lease obligations, refer to Note 11 to our consolidated financial statements appearing at the end of this Annual Report on Form 10-K.

Under various agreements with third-party licensors, we have agreed to make milestone payments and pay royalties to third parties based on specified milestones. For a description of our license agreements, see "Business—Strategic partnerships and collaborations/licenses" and see Note 13 of our consolidated financial statements appearing at the end of this Annual Report on Form 10-K.

We enter into contracts in the normal course of business with CROs and CMOs for clinical trials, preclinical research studies and testing, manufacturing and other services and products for operating purposes. These contracts are cancelable by us upon prior notice of 30 days.

Recently issued accounting pronouncements

We have reviewed all recently issued standards and have determined that, other than as disclosed in Note 2 to our consolidated financial statements appearing at the end of this Annual Report on Form 10-K, such standards will not have a material impact on our consolidated financial statements or do not otherwise apply to our operations.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

We are exposed to market risk related to changes in interest rates. As of December 31, 2023, our cash equivalents consisted of money market accounts that have contractual maturities of less than 90 days from the date of acquisition. As of December 31, 2023, our investments consisted of treasury bills that have contractual maturities of less than one year. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of our portfolio, an immediate 10% change in market interest rates would not have a material impact on the fair market value of our investment portfolio or on our financial position or results of operations.

Inflation Risk

Inflation generally affects us by increasing our cost of labor and research, manufacturing and development costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations in the last three years. However, our operations may be adversely affected by the inflation in the future.

Item 8. Financial Statements and Supplementary Data.

The information required by this Item 8 is included at the end of this Annual Report on Form 10-K beginning on page F-1.

Item 9. Changes in and Disagreements With Accountants on Accounting and Financial Disclosure.

Not applicable.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer (our principal executive officer and principal financial officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2023. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, refers to controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that such information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to a company's management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating our disclosure controls and procedures, management recognizes that disclosure controls and procedures, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the objectives of the disclosure controls and procedures are met. Additionally, in designing disclosure controls and procedures, our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible disclosure controls and procedures. The design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, controls may become inadequate because of changes in conditions, or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a control system, misstatements due to error or fraud may occur and not be detected.

Based on the evaluation of our disclosure controls and procedures, our Chief Executive Officer and Chief Financial Officer concluded that, as of December 31, 2023, our disclosure controls and procedures were effective at the reasonable assurance level.

Management's Annual Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Management conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2023 based on the framework in *Internal Control-Integrated Framework* (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). Based on that evaluation, management has concluded that the Company's internal control over financial reporting was effective as of December 31, 2023.

As a non-accelerated filer and a “smaller reporting company”, as defined in Rule 12b-2 under the Exchange Act, our independent registered public accounting firm is not required to issue an attestation report on the internal control over financial reporting.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the three months ended December 31, 2023 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

(b) Director and Officer Trading Arrangements

None of our directors or officers adopted or terminated a Rule 10b5-1 trading arrangement or a non-Rule 10b5-1 trading arrangement (as defined in Item 408(c) of Regulation S-K) during the fourth quarter of 2023.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

Except to the extent provided below, the information required under this Item 10 is incorporated by reference to our definitive proxy statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission not later than 120 days after the end of the fiscal year ended December 31, 2023.

We have adopted a Code of Business Conduct and Ethics, or the Code of Conduct, that applies to our directors, executive officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. The Code of Conduct is available on the Investor Relations portion of our website, www.solidbio.com. The nominating and corporate governance committee of our Board of Directors is responsible for overseeing the Code of Conduct and must approve any waivers of the Code of Conduct for employees, executive officers and directors. In addition, we intend to post on our website all disclosures that are required by law or the listing standards of Nasdaq concerning any amendments to, or waivers of, any provision of the Code of Conduct.

Item 11. Executive Compensation.

The information required under this Item 11 is incorporated by reference to our definitive proxy statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission not later than 120 days after the end of the fiscal year ended December 31, 2023.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The information required under this Item 12 is incorporated by reference to our definitive proxy statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission not later than 120 days after the end of the fiscal year ended December 31, 2023.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required under this Item 13 is incorporated by reference to our definitive proxy statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission not later than 120 days after the end of the fiscal year ended December 31, 2023.

Item 14. Principal Accountant Fees and Services.

The information required under this Item 14 is incorporated by reference to our definitive proxy statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission not later than 120 days after the end of the fiscal year ended December 31, 2023.

PART IV

Item 15. Exhibits and Financial Statement Schedules.

(1) Financial Statements:

(2) Financial Statement Schedules:

All financial statement schedules have been omitted because they are not applicable, not required or the information required is shown in the financial statements or the notes thereto.

(3) Exhibits. The following is a list of exhibits filed as part of this Annual Report on Form 10-K.

Exhibit Number	Description
2.1	Agreement and Plan of Merger, dated as of September 29, 2022, by and among Solid Biosciences Inc., Greenland Merger Sub LLC, AavantiBio, Inc. and, solely in his capacity as the Equityholder Representative, Doug Swirsky (incorporated by reference to Exhibit 2.1 to the Current Report on Form 8-K filed on September 30, 2022).
3.1	Certificate of Incorporation of Solid Biosciences Inc., as amended (incorporated by reference to Exhibit 4.1 to the Registration Statement on Form S-8 filed on December 2, 2022).
3.2	Bylaws of Solid Biosciences Inc. (incorporated by reference to Exhibit 4.2 to the Registration Statement on Form S-8 filed on January 29, 2018).
4.1	Specimen Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Registration Statement on Form S-1 filed on December 29, 2017).
4.2	Description of the Company's Securities Registered under Section 12 of the Exchange Act (incorporated by reference to Exhibit 4.2 to the Annual Report on Form 10-K filed on March 23, 2023).
10.1†	Amended and Restated Registration Rights Agreement dated March 29, 2017 by and among Solid Biosciences, LLC and certain investors (incorporated by reference to Exhibit 10.17 to the Registration Statement on Form S-1 filed on December 29, 2017).
10.2†	Solid Biosciences, LLC Amended and Restated Equity Incentive Plan and form of unit restriction agreement (incorporated by reference to Exhibit 10.7 to the Annual Report on Form 10-K filed on March 29, 2018).
10.3†	Solid Biosciences Inc. 2018 Omnibus Incentive Plan (incorporated by reference to Exhibit 99.1 to the Registration Statement on Form S-8 filed on January 29, 2018).
10.4†	Form of Incentive Stock Option Agreement under 2018 Omnibus Incentive Plan. (incorporated by reference to Exhibit 10.7 to the Annual Report on Form 10-K filed on March 13, 2019).
10.5†	Form of Nonqualified Stock Option Agreement under 2018 Omnibus Incentive Plan. (incorporated by reference to Exhibit 10.8 to the Annual Report on Form 10-K filed on March 13, 2019).
10.6†	Form of Restricted Stock Agreement under 2018 Omnibus Incentive Plan (incorporated by reference to Exhibit 10.9 to the Registration Statement on Form S-1 filed on December 29, 2017).
10.7*#	Exclusive Patent License Agreement, dated as of October 16, 2015, by and between Solid GT, LLC and the University of Washington.
10.8*#	License Agreement, dated as of October 15, 2015, by and between Solid GT, LLC and The Curators of the University of Missouri.
10.9†	Form of Indemnification Agreement for Directors and Officers (incorporated by reference to Exhibit 10.16 to the Registration Statement on Form S-1 filed on December 29, 2017).
10.10†	Summary of Non-Employee Director Compensation Program (incorporated by reference to Exhibit 10.12 to the Annual Report on Form 10-K filed on March 23, 2023).
10.11	Securities Purchase Agreement, dated July 25, 2019, by and among the Company and the other parties thereto (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on July 26, 2019).
10.12	Form of Pre-Funded Warrant to Purchase Common Stock to be issued pursuant to the Securities Purchase Agreement (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on July 26, 2019).
10.13	Registration Rights Agreement, dated July 25, 2019, by and among the Company and the other parties thereto (incorporated by reference to Exhibit 10.3 to the Current Report on Form 8-K filed on July 26, 2019).
10.14†	Consulting Agreement, dated as of November 19, 2020, by and between Solid Biosciences Inc. and Danforth Advisors, LLC. (incorporated by reference to Exhibit 10.24 to the Annual Report on Form 10-K filed on March 15, 2021).

10.15*†	Executive Chair Agreement, effective January 1, 2022, by and between Solid Biosciences Inc. and Ian F. Smith, as amended by the First Amendment to Executive Chair Agreement, effective September 30, 2022, and Second Amendment to the Executive Chair Agreement, effective January 1, 2024
10.16†	Amended and Restated 2020 Equity Incentive Plan (incorporated by reference to Exhibit 99.1 to the Current Report on Form 8-K filed on December 1, 2022).
10.17†	Form of Stock Option Agreement under the 2020 Equity Incentive Plan (incorporated by reference to Exhibit 10.4 to the Quarterly Report on Form 10-Q filed on August 6, 2020).
10.18†	Form of Restricted Stock Unit Agreement under the 2020 Equity Incentive Plan (incorporated by reference to Exhibit 10.28 to the Annual Report on Form 10-K filed on March 14, 2022).
10.19#	Collaboration and License Agreement, dated as of October 22, 2020, by and between the Company and Ultragenyx Pharmaceutical Inc. (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed on November 5, 2020).
10.20	Stock Purchase Agreement, dated as of October 22, 2020, by and between the Company and Ultragenyx Pharmaceutical Inc. (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed on November 5, 2020).
10.21#	Investor Agreement, dated as of October 22, 2020, by and between the Company and Ultragenyx Pharmaceutical Inc. (incorporated by reference to Exhibit 10.3 to the Quarterly Report on Form 10-Q filed on November 5, 2020).
10.22#	First Amendment, dated as of October 9, 2020, to the Exclusive Patent License by and between the Company and the University of Washington (incorporated by reference to Exhibit 10.4 to the Quarterly Report on Form 10-Q filed on November 5, 2020).
10.23	Securities Purchase Agreement, dated December 10, 2020, by and among the Company and the other parties thereto (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on December 11, 2020).
10.24	Registration Rights Agreement, dated December 10, 2020, by and among the Company and the other parties thereto (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on December 11, 2020).
10.25#	First Amendment, dated as of January 27, 2021, to the Exclusive Patent License by and between the Company and the Curators of the University of Missouri (incorporated by reference to Exhibit 10.36 to the Annual Report on Form 10-K filed on March 14, 2021).
10.26	Lease, dated June 15, 2021, between Solid Biosciences Inc. and Hood Park LLC (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed on August 16, 2021).
10.27†*	Amended and Restated 2021 Employee Stock Purchase Plan.
10.28†	Form of Nonstatutory Inducement Stock Option Agreement (incorporated by reference to Exhibit 10.4 to the Quarterly Report on Form 10-Q filed on August 16, 2021).
10.29†	Form of Inducement Restricted Stock Unit Award Agreement (incorporated by reference to Exhibit 99.5 to the Registration Statement on Form S-8 filed on August 16, 2021).
10.30*	Amended and Restated Sales Agreement, dated March 13, 2024, by and between the Company and Jefferies LLC.
10.31	Form of Parent Support Agreement (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on September 30, 2022).
10.32	Form of Support and Joinder Agreement (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on September 30, 2022).
10.33	Securities Purchase Agreement, dated September 29, 2022, by and among Solid Biosciences Inc. and the persons party thereto (incorporated by reference to Exhibit 10.3 to the Current Report on Form 8-K filed on September 30, 2022).
10.34	Registration Rights Agreement, dated September 29, 2022, by and among Solid Biosciences Inc. and the persons party thereto (incorporated by reference to Exhibit 10.4 to the Current Report on Form 8-K filed on September 30, 2022).

10.35†	Employment Agreement, dated September 29, 2022, by and between Solid Biosciences Inc. and Alexander Cumbo (incorporated by reference to Exhibit 10.5 to the Current Report on Form 8-K filed on September 30, 2022).
10.36†	Executive Transition and Separation Agreement, dated September 29, 2022, by and between Solid Biosciences Inc. and Ilan Ganot (incorporated by reference to Exhibit 10.6 to the Current Report on Form 8-K filed on September 30, 2022).
10.37†	Consulting Agreement, dated September 29, 2022, by and between Solid Biosciences Inc. and Ilan Ganot (incorporated by reference to Exhibit 10.7 to the Current Report on Form 8-K filed on September 30, 2022).
10.38†	Executive Transition and Separation Agreement, dated September 29, 2022, by and between Solid Biosciences Inc. and Erin Powers Brennan (incorporated by reference to Exhibit 10.8 to the Current Report on Form 8-K filed on September 30, 2022).
10.39†	Consulting Agreement, dated September 29, 2022, by and between Solid Biosciences Inc. and Erin Powers Brennan (incorporated by reference to Exhibit 10.9 to the Current Report on Form 8-K filed on September 30, 2022).
10.40	Employment Agreement, dated September 29, 2022, by and between Solid Biosciences Inc. and David Tyronne Howton (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed on May 11, 2023).
10.41	Executive Transition and Separation Agreement, dated as of May 22, 2023, between Solid Biosciences Inc. and Carl Morris (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed on August 14, 2023).
10.42	Consulting Agreement dated as of July 14, 2023, between Solid Biosciences Inc. and PHDL Consulting LLC (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed on August 14, 2023)
10.43	Employment Agreement, dated October 2, 2023, by and between Solid Biosciences Inc. and Gabriel Brooks (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed on November, 8 2023).
10.44#	Standard Exclusive License Agreement With Know-How (Agreement No. A19110), dated as of March 17, 2020, by and between AavantiBio, Inc. and University of Florida Research Foundation, Incorporated, as amended on August 23, 2022 (incorporated by reference to Exhibit 10.42 to the Annual Report on Form 10-K filed as on March 23, 2023).
10.45#	Standard Exclusive License Agreement With Know-How (Agreement No. A19111), dated as of March 17, 2020, by and between AavantiBio, Inc. and University of Florida Research Foundation, Incorporated, as amended on May 25, 2021 and August 23, 2022 (incorporated by reference to Exhibit 10.43 to the Annual Report on Form 10-K filed on March 23, 2023).
10.46	Form on Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 to the Current Report on Form 8-K filed on January 8, 2024).
10.47	Form of Securities Purchase Agreement, dated January 8, 2024, by and among Solid Biosciences Inc. and the other parties thereto (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on January 8, 2024).
10.48	Form of Registration Rights Agreement, dated January 8, 2024, by and among Solid Biosciences Inc. and the other parties thereto (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on January 8, 2024).
10.49*†	2024 Inducement Stock Incentive Plan.
10.50*†	Form of Non-Statutory Stock Option Agreement under the 2024 Inducement Stock Incentive Plan.
10.51*†	Form of Restricted Stock Unit Agreement under the 2024 Inducement Stock Incentive Plan.
10.52*†	Form of Restricted Stock Unit Agreement under the 2020 Equity Incentive Plan.
10.53*##	Patent License Agreement, dated as of March 10, 2016, by and between Solid GT, LLC and the Regents of the University of Michigan.
10.54*##	Life Technologies Cell Line License Agreement, dated as of November 20, 2016, by and between Solid Biosciences, LLC and Life Technologies Corporation.
10.55*##	License Agreement, dated as of June 23, 2016, by and between Solid GT, LLC and President and Fellows of Harvard College.
10.56*##	License Agreement, dated as of August 3, 2017, by and between Solid Biosciences, LLC and President and Fellows of Harvard College.
21.1*	Subsidiaries of Solid Biosciences Inc.

23.1*	Consent of PricewaterhouseCoopers LLP, Independent Registered Public Accounting Firm.
31.1*	Certification of Chief Executive Officer of the Registrant Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Chief Financial Officer of the Registrant Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1**	Certification of Chief Executive Officer of the Registrant Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2**	Certification of Chief Financial Officer of the Registrant Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
97.1*	Dodd-Frank Compensation Recovery Policy
101.INS	Inline XBRL Instance Document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document With Embedded Linkbase Documents
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101).

* Filed herewith.
** Furnished herewith.
† Indicates management contract or compensatory plan.
# Certain portions of this exhibit have been omitted because they are not material and contain information that the Registrant customarily and actually treats as private or confidential.
Filed with this Annual Report on Form 10-K solely for the purpose of transitioning this previously-filed exhibit, which is the subject of expiring confidential treatment orders, to the rules governing the filing of redacted exhibits under Regulation S-K Item 601(b)(10)(iv) pursuant to the SEC's CF Disclosure Guidance: Topic 7. Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

Item 16. Form 10-K Summary.

Not applicable.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended, the Registrant has duly caused this Report to be signed on its behalf by the undersigned, thereunto duly authorized**.**

SOLID BIOSCIENCES INC.

Date: March 13, 2024

By: /s/ Alexander Cumbo

Alexander Cumbo
President, Chief Executive Officer and Director
(Principal Executive Officer)

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this Report has been signed below by the following persons on behalf of the Registrant in the capacities and on the dates indicated.

/s/ Alexander Cumbo Alexander Cumbo	President, Chief Executive Officer and Director *(Principal Executive Officer)*	March 13, 2024
/s/ Kevin Tan Kevin Tan	Chief Financial Officer *(Principal Financial and Accounting Officer)*	March 13, 2024
/s/ Ian F. Smith Ian F. Smith	Chairman of the Board	March 13, 2024
/s/ Martin Freed Martin Freed	Director	March 13, 2024
/s/ Ilan Ganot Ilan Ganot	Director	March 13, 2024
/s/ Clare Kahn Clare Kahn	Director	March 13, 2024
/s/ Georgia Keresty Georgia Keresty	Director	March 13, 2024
/s/ Adam Koppel Adam Koppel	Director	March 13, 2024
/s/ Sukumar Nagendran Sukumar Nagendran	Director	March 13, 2024
/s/ Rajeev Shah Rajeev Shah	Director	March 13, 2024
/s/ Adam Stone Adam Stone	Director	March 13, 2024
/s/ Lynne Sullivan Lynne Sullivan	Director	March 13, 2024

Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of Solid Biosciences Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Solid Biosciences Inc. and its subsidiaries (the "Company") as of December 31, 2023 and 2022, and the related consolidated statements of operations, of comprehensive loss, of stockholders' equity and of cash flows for each of the three years in the period ended December 31, 2023, including the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2023 and 2022, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2023 in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits of these consolidated financial statements in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion**.**

Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matters

The critical audit matter communicated below is a matter arising from the current period audit of the consolidated financial statements that was communicated or required to be communicated to the audit committee and that (i) relates to accounts or disclosures that are material to the consolidated financial statements and (ii) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

External Research and Development Costs

As described in Note 2 to the consolidated financial statements, research and development costs are expensed as incurred. Research and development expenses include salaries, equity-based compensation and benefits of employees, third-party license fees and other operational costs related to the Company's research and development activities, including allocated facility-related expenses and external costs of outside vendors engaged to conduct both preclinical studies and clinical trials. The Company has entered into various research and development contracts with research institutions and other companies. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. Management records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, management analyzes progress of the studies, including the phase or completion of events, invoices received and contracted costs. As disclosed by management, the majority of service providers invoice the Company in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advanced

payments. The Company's research and development expense for the year ended December 31, 2023 was $76.6 million, a majority of which relates to external research and development costs.

The principal consideration for our determination that performing procedures relating to external research and development costs is a critical audit matter is a high degree of auditor effort in performing procedures related to the Company's external research and development costs.

Addressing the matter involved performing procedures and evaluating audit evidence in connection with forming our overall opinion on the consolidated financial statements. These procedures included, among others, testing external research and development costs on a sample basis by agreeing relevant information, including overall contract value, amounts incurred to date, and percentage of completion amounts to the (i) underlying agreements with outside vendors engaged to conduct preclinical studies and clinical trials, (ii) purchase orders, (iii) invoices received, (iv) underlying payments made for expenses incurred on the contracts, and (v) external confirmations or communications obtained by management from outside vendors.

/s/ PricewaterhouseCoopers LLP
Boston, Massachusetts
March 13, 2024

We have served as the Company's auditor since 2017.

SOLID BIOSCIENCES INC.
CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share data)

	December 31,	
	2023	**2022**
Assets		
Current assets:		
Cash and cash equivalents	$ 74,015	$ 155,384
Available-for-sale securities	49,625	58,338
Prepaid expenses and other current assets	6,094	5,916
Total current assets	129,734	219,638
Operating lease, right-of-use asset	26,539	28,949
Property and equipment, net	6,624	9,657
Other non-current assets	209	175
Restricted cash	1,833	1,833
Total assets	$ 164,939	$ 260,252
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,032	$ 3,238
Accrued expenses	10,137	16,691
Operating lease liabilities	1,855	1,897
Finance lease liabilities	469	668
Other current liabilities	24	14
Total current liabilities	14,517	22,508
Operating lease liabilities, excluding current portion	22,707	24,279
Finance lease obligations, excluding current portion	1,234	1,703
Other non-current liabilities	-	96
Total liabilities	38,458	48,586
Commitments and Contingencies (Note 12)		
Stockholders' Equity:		
Preferred stock, $0.001 par value; 10,000,000 shares authorized at December 31, 2023 and December 31, 2022; no shares issued and outstanding at December 31, 2023 and December 31, 2022	—	—
Common stock, $0.001 par value; 60,000,000 shares authorized at December 31, 2023 and December 31, 2022; 20,386,606 shares issued and outstanding at December 31, 2023 and 19,556,732 shares issued and outstanding at December 31, 2022	20	20
Additional paid-in capital	785,199	774,452
Accumulated other comprehensive income (loss)	15	(68)
Accumulated deficit	(658,753)	(562,738)
Total stockholders' equity	126,481	211,666
Total liabilities and stockholders' equity	$ 164,939	$ 260,252

The accompanying notes are an integral part of these consolidated financial statements.

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(In thousands, except share and per share data)

	Year Ended December 31,		
	2023	**2022**	**2021**
Collaboration revenue - related party	$ —	$ 8,094	$ 13,620
Operating expenses:			
Research and development	76,563	78,420	58,739
General and administrative	27,752	28,948	27,135
Restructuring expense	(63)	7,178	—
Total operating expenses	104,252	114,546	85,874
Loss from operations	(104,252)	(106,452)	(72,254)
Other income, net:			
Interest income, net	7,142	2,616	64
Gain on acquisition	—	18,236	—
Other income (expense)	1,095	(381)	2
Total other income, net	8,237	20,471	66
Net loss	$ (96,015)	$ (85,981)	$ (72,188)
Net loss per share, basic and diluted	$ (4.83)	$ (10.10)	$ (10.14)
Weighted average common stock outstanding, basic and diluted	19,884,007	8,512,089	7,118,024

The accompanying notes are an integral part of these consolidated financial statements.

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(In thousands)

	Year Ended December 31,		
	2023	**2022**	**2021**
Net loss	$ (96,015)	$ (85,981)	$ (72,188)
Other comprehensive income (loss):			
Unrealized gain (loss) on available-for-sale securities	83	(23)	(45)
Comprehensive loss	$ (95,932)	$ (86,004)	$ (72,233)

The accompanying notes are an integral part of these consolidated financial statements.

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS STOCKHOLDERS' EQUITY
(In thousands except for share data)

	Common Stock	Amount	Additional paid in capital	Accumulated other comprehensive income (loss)	Accumulated Deficit	Total Equity
Balance at December 31, 2020	5,803,487	$ 6	$ 536,649	$ —	$ (404,569)	$ 132,086
Equity-based compensation	—	—	13,373	—	—	13,373
Sale of common stock, net of issuance costs of $8,872	1,666,666	1	134,877	—	—	134,878
Exercise of common stock options	775	—	41	—	—	41
Issuance of common stock in connection with employee stock purchase plan	2,978	—	66	—	—	66
Vesting of restricted stock units	27,946	—	—	—	—	—
Forfeiture of restricted stock awards	(1,947)	—	—	—	—	—
Unrealized gain on available-for-sale securities	—	—	—	(45)	—	(45)
Net loss	—	—	—	—	(72,188)	(72,188)
Balance at December 31, 2021	7,499,905	7	685,006	(45)	(476,757)	208,211
Equity-based compensation	—	—	7,537	—	—	7,537
Sale of common stock, net of issuance costs of $2,449	10,638,290	11	72,540	—	—	72,551
Exercise of pre-funded warrants	—	—	22	—	—	22
Issuance of common stock in connection with employee stock purchase plan	29,130	1	180	—	—	181
Vesting of restricted stock units	35,149	—	—	—	—	—
Issuance of shares in connection with acquisition	1,354,258	1	9,167	—	—	9,168
Unrealized loss on available-for-sale securities	-	—	—	(23)	—	(23)
Net loss	-	—	—	—	(85,981)	(85,981)
Balance at December 31, 2022	19,556,732	20	774,452	(68)	(562,738)	211,666
Equity-based compensation	—	—	7,625	—	—	7,625
Vesting of restricted stock units	183,951	—	—	—	—	—
Sale of common stock net of issuance costs of $76	602,030	—	2,974	—	—	2,974
Issuance of common stock in connection with employee stock purchase plan	43,893	—	148	—	—	148
Unrealized gain on available-for-sale securities	—	—	—	83	—	83
Net loss	—	—	—	—	(96,015)	(96,015)
Balance at December 31, 2023	20,386,606	$ 20	$ 785,199	$ 15	$ (658,753)	$ 126,481

The accompanying notes are an integral part of these consolidated financial statements.

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)

	Year Ended December 31,		
	2023	**2022**	**2021**
Operating activities:			
Net loss	$ (96,015)	$ (85,981)	$ (72,188)
Adjustments to reconcile net loss to net cash used in operating activities:			
Net amortization of (discount)/premium on available-for-sale securities	(2,408)	233	1,117
Equity-based compensation expense	7,625	7,537	13,373
Depreciation and amortization expense	2,581	2,408	2,964
Loss on sale of property and equipment	374	—	92
Gain on lease termination	—	(249)	(81)
Gain on acquisition	—	(18,236)	—
Changes in operating assets and liabilities:			
Prepaid expenses and other current and non-current assets	3,436	3,695	(9,247)
Accounts receivable - related party	—	110	(110)
Accounts payable	(764)	(5,246)	1,209
Accrued expenses and other current and non-current liabilities	(9,009)	5,832	(2,255)
Deferred revenue - related party, current and non-current	-	(8,080)	(12,638)
Net cash used in operating activities	(94,180)	(97,977)	(77,764)
Investing activities:			
Purchases of property and equipment	(1,515)	(3,015)	(1,281)
Acquisition of business, net of cash received	—	31,523	—
Proceeds from sale of property and equipment	—	600	—
Proceeds from sales and maturities of available-for-sale securities	128,632	212,811	51,444
Purchases of available-for-sale securities	(117,428)	(182,762)	(141,249)
Net cash provided by (used in) investing activities	9,689	59,157	(91,086)
Financing activities:			
Proceeds from issuance of common stock, net of issuance costs	2,974	72,551	134,878
Proceeds from financing liabilities	—	2,143	—
Proceeds from exercise of stock options	—	—	41
Proceeds from exercise of pre-funded warrants	—	22	—
Employee stock purchases and withholdings	148	181	66
Repayment of financing liabilities	—	(66)	—
Net cash provided by financing activities	3,122	74,831	134,985
Net (decrease) increase in cash, cash equivalents and restricted cash	(81,369)	36,011	(33,865)
Cash, cash equivalents, and restricted cash at beginning of period	157,217	121,206	155,071
Cash, cash equivalents, and restricted cash at end of period	$ 75,848	$ 157,217	$ 121,206
Supplemental disclosure of non-cash investing and financing activities:			
Issuance of common stock in acquisition	$ —	$ 9,168	$ —
Right-of-use assets obtained in exchange for operating lease liabilities	$ 410	$ 29,126	$ —
Decrease in right-of-use asset and lease liability due to lease termination	$ (252)	$ (464)	$ (1,233)
Decrease in property, plant and equipment due to asset exchange	$ (950)	$ —	$ —
Property and equipment included in accounts payable and accruals	$ 76	$ 527	$ 104

The accompanying notes are an integral part of these consolidated financial statements.

1. Nature of the Business and Basis of Presentation

Nature of Business

Solid Biosciences Inc. was organized in March 2013 under the name SOLID Ventures Management, LLC and operated as a Delaware limited liability company until immediately prior to the effectiveness of its registration statement on Form S-1 on January 25, 2018, at which time it completed a statutory corporate conversion into a Delaware corporation and changed its name to Solid Biosciences Inc. (the "Company" or "Solid"). On December 2, 2022, the Company completed its acquisition of AavantiBio, Inc. ("AavantiBio"), a privately held gene therapy company focused on transforming the lives of patients with Friedreich's ataxia ("FA") and rare Cardiomyopathies (the "Acquisition"). Upon the consummation of the Acquisition, the Company acquired AavantiBio's gene therapy programs, AVB-202-TT for FA and AVB-401 for BAG3 mediated dilated cardiomyopathy, as well as additional assets for the treatment of other cardiac diseases, platform technologies and know-how related thereto. AavantiBio is a wholly owned subsidiary of the Company.

The Company is a life sciences company focused on advancing a portfolio of current and future gene therapy candidates (collectively, "Candidates"), including SGT-003 for the treatment of Duchenne muscular dystrophy ("Duchenne"), SGT-501 for the treatment of Catecholaminergic polymorphic ventricular tachycardia ("CPVT"), and additional assets for the treatment of cardiac and other diseases, at different stages of development with varying levels of investment. The Company is advancing its diverse pipeline across rare neuromuscular and cardiac diseases, bringing together experts in science, technology, disease management and care. Patient-focused and founded by those directly impacted by Duchenne, Solid's mission is to improve the daily lives of patients living with these devastating diseases.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on licenses, protection of proprietary technology, dependence on key personnel, compliance with government regulations and the need to obtain additional financing to fund operations. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical studies and clinical trials and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance and reporting capabilities.

The Company's candidates are in development. There can be no assurance that the Company's research and development will be successfully completed, that adequate protection for the Company's intellectual property will be obtained, that any products developed will obtain necessary government regulatory approval or that any approved products will be commercially viable. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from, among others, other pharmaceutical and biotechnology companies. In addition, the Company is dependent upon the services of its employees, partners and consultants.

On October 27, 2022, the Company effected a reverse stock split of its outstanding shares of common stock at a ratio of one-for-15 pursuant to a certificate of amendment to its certificate of incorporation filed with the Secretary of State of the State of Delaware. The reverse stock split was reflected on the Nasdaq Stock Market ("Nasdaq") beginning with the opening of trading on October 28, 2022. Pursuant to the reverse stock split, every 15 shares of the Company's issued and outstanding shares of common stock were automatically combined into one issued and outstanding share of common stock, without any change in the par value per share of the common stock. The reverse stock split reduced the authorized number of shares of common stock from 300,000,000 to 20,000,000 and, pursuant to the certificate of amendment, such reduced authorized number of shares of common stock was subsequently multiplied by three, such that following the reverse stock split the Company has 60,000,000 shares of common stock authorized. The reverse stock split affected all issued and outstanding shares of the Company's common stock, and the respective numbers of shares of common stock underlying the Company's outstanding stock options, outstanding restricted stock units, outstanding warrants and the Company's equity incentive plans were proportionately adjusted. All share and per share amounts of the common stock included in the accompanying consolidated financial statements have been retrospectively adjusted to give effect to the reverse stock split for all periods presented, including reclassifying an amount equal to the reduction in par value to additional paid-in capital.

Liquidity

The accompanying consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business. Through December 31, 2023, the Company has funded its operations primarily with the proceeds from the sale of redeemable preferred units and member units as well as the sale of common stock and prefunded warrants to purchase shares of its common stock in private placements and the sale of common stock in its initial public offering and follow-on public offering in March 2021 and under its at-the-market sales agreement.

On September 29, 2022, the Company entered into the securities purchase agreement, pursuant to which, on December 2, 2022, the Company issued an aggregate of 10,638,290 shares of the Company's common stock in a private placement. The private placement closed immediately following the closing of the Acquisition on December 2, 2022. The Company received net proceeds from the private placement of $72,551.

During the year ended December 31, 2023, the Company issued 602,030 shares of its common stock, pursuant to the Company's "at-the-market offering" sales agreement, dated March 13, 2019, as amended on August 16, 2021, between the Company and Jefferies LLC (the "ATM Sales Agreement"). During the year ended December 31, 2023, the Company received net proceeds of $2,974 from the sale of shares pursuant to the ATM Sales Agreement.

On January 11, 2024, the Company issued and sold in a private placement 16,973,103 shares of the Company's common stock at a price per share of $5.53 and, to one investor in lieu of shares of common stock, pre-funded warrants to purchase 2,712,478 shares of common stock at a price of $5.529 per pre-funded warrant (the "January 2024 Private Placement"). The Company received approximately $104,034 of net proceeds from the January 2024 Private Placement after deducting offering costs.

In accordance with Accounting Standards Codification ("ASC") 205-40, Going Concern, the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date the financial statements are issued. As of December 31, 2023, the Company had an accumulated deficit of $658,753. During the years ended December 31, 2023, 2022 and 2021, the Company incurred a net loss of $96,015, $85,981 and $72,188, respectively. The Company used $94,180 of cash in operations for the year ended December 31, 2023. The Company expects to continue to generate operating losses in the foreseeable future. Based upon its current operating plan, the Company expects that its cash, cash equivalents and available-for-sale securities of $123,640, excluding restricted cash of $1,833, as of December 31, 2023, together with the net proceeds from the January 2024 Private Placement, will be sufficient to fund its operating expenses and capital expenditure requirements for at least twelve months from the date of issuance of these financial statements. However, the Company has based this estimate on assumptions that may prove to be wrong, and its operating plan may change as a result of many factors currently unknown to it. As a result, the Company could deplete its capital resources sooner than it currently expects. The Company expects to finance its future cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances or licensing arrangements. If the Company is unable to obtain funding, the Company would be forced to delay, reduce or eliminate some or all of its research and development programs, preclinical and clinical testing or commercialization efforts, which could adversely affect its business prospects.

The accompanying consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP"). The accompanying consolidated financial statements include the accounts of the Company and its wholly owned or controlled subsidiaries. All intercompany accounts and transactions have been eliminated.

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of the Company's consolidated financial statements in conformity with GAAP requires management to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of revenues and expenses during the reporting periods. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, estimates related to revenue recognition, the recognition of research and development expenses and equity-based compensation. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from the Company's estimates.

Cash Equivalents

The Company considers all short-term, highly liquid investments with original maturities of 90 days or less at acquisition date to be cash equivalents.

Restricted Cash

The Company held restricted cash of $1,833 in a restricted bank account as a security deposit for lease of the Company's facilities as of December 31, 2023 and December 31, 2022. The Company has included restricted cash of $1,833 as a non-current asset as of December 31, 2023 and December 31, 2022. A reconciliation of the amounts of cash and cash equivalents and restricted cash from the cash flow statement to the balance sheet is as follows:

	December 31, 2023	December 31, 2022	December 31, 2021
Cash and cash equivalents	$ 74,015	$ 155,384	$ 119,136
Restricted cash, non-current	1,833	1,833	2,070
Cash and cash equivalents and restricted cash	$ 75,848	$ 157,217	$ 121,206

Available-for-Sale Securities

Available-for-sale securities consist of investments with original maturities greater than 90 days at acquisition date. The Company has classified its investments with maturities beyond one year as short term, based on their highly liquid nature and because such available-for-sale securities represent the investment of cash that is available for current operations.

The Company classifies all of its investments as available-for-sale securities. The Company's investments are measured and reported at fair value using quoted prices in active markets for similar securities. Unrealized gains and losses on available-for-sale debt securities are reported as a separate component of stockholders' equity. The cost of debt securities sold is determined on a specific identification basis, and realized gains and losses are included in other income (expense) within the consolidated statement of operations. If any adjustment to fair value reflects a decline in the value of the investment that the Company considers to be "other than temporary," the Company reduces the investment to fair value through a charge to the consolidated statement of operations. No such adjustments were necessary during the periods presented.

Concentration of Credit Risk and of Significant Suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash, cash equivalents and available-for-sale securities. Periodically, the Company maintains deposits in accredited financial institutions in excess of federally insured limits. The Company maintains each of its cash, cash equivalents and available-for-sale securities balances with high-quality and accredited financial institutions and accordingly, such funds are not exposed to significant credit risk. The Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company is dependent on third-party manufacturers to supply products for research and development activities of its programs, including clinical and preclinical testing. These programs could be adversely affected by a significant interruption in the supply of such drug substance products.

Fair Value Measurements

Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities or other inputs that are observable or can be corroborated by observable market data.

- Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company's cash equivalents and available-for-sale securities are carried at fair value, determined according to the fair value hierarchy described above. See Note 5, *Fair Value of Financial Assets and Liabilities*, for additional information. The carrying values of the Company's accounts payable and accrued expenses and other current liabilities approximate their fair value due to the short-term nature of these liabilities.

Leases

At inception of a contract, the Company determines if a contract meets the definition of a lease. A lease is a contract, or part of a contract, that conveys the right to control the use of identified property, plant, or equipment (an identified asset) for a period of time in exchange for consideration. The Company determines if the contract conveys the right to control the use of an identified asset for a period of time. The Company assesses throughout the period of use whether the Company has both of the following: (1) the right to obtain substantially all of the economic benefits from use of the identified asset and (2) the right to direct the use of the identified asset. This determination is reassessed if the terms of the contract are changed. Leases are classified as operating or finance leases based on the terms of the lease agreement and certain characteristics of the identified asset. Right-of-use assets and lease liabilities are recognized at the lease commencement date based on the present value of the minimum future lease payments. Adjustments to the right-of-use asset may be required for items such as lease prepayments or incentives received. The Company's policy is to not record leases with an original term of twelve months or less on the consolidated balance sheets. The Company recognizes lease expense for these short-term leases on a straight-line basis over the lease term. Certain lease agreements include rental payments that are adjusted periodically for inflation or other variables. In addition to rent, the leases may require the Company to pay additional amounts for taxes, insurance, maintenance and other expenses, which are generally referred to as non-lease components. Such adjustments to rental payments and variable non-lease components are treated as variable lease payments and recognized in the period in which the obligation for these payments was incurred. Variable lease components and variable non-lease components are not measured as part of the right-of-use asset and liability. Only when lease components and their associated non-lease components are fixed are they accounted for as a single lease component and recognized as part of a right-of-use asset and liability. Total contract consideration is allocated to the combined fixed lease and non-lease components.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over the useful life of the asset. Laboratory equipment is depreciated over five years. Computer equipment is depreciated over three years. Computer software is depreciated over two years. Furniture and office equipment are depreciated over five years. Leasehold improvements are amortized over the shorter of the lease term or the estimated useful life of the related asset. Expenditures for repairs and maintenance of assets are charged to expense as incurred. Upon retirement or sale, the cost and related accumulated depreciation of assets disposed of are removed from the accounts and any resulting gain or loss is included in loss from operations. Equipment under a finance lease is stated at fair value at the inception of the lease less accumulated depreciation and is depreciated over the remaining lease term or the estimated useful life of the equipment.

Impairment of Long-Lived Assets

Long-lived assets, comprised of property and equipment, to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset over its fair value, determined based on discounted cash flows.

Revenue Recognition

The Company recognizes revenue in accordance with ASC Topic 606, Revenue from Contracts with Customers ("ASC 606"). Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine the appropriate amount of revenue to be recognized for arrangements determined to be within the scope of ASC 606, the Company performs the following five steps: (i) identification of the contract(s) with the customer; (ii) identification of the promised goods or services in the contract and determination of whether the promised goods or services are performance obligations; (iii) measurement of the transaction price; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

As part of the accounting for these arrangements, the Company must use significant judgment to determine: a) the number of performance obligations based on the determination under step (ii) above and whether those performance obligations are distinct from other performance obligations in the contract; b) the transaction price under step (iii) above; and c) the standalone selling price for each performance obligation identified in the contract for the allocation of transaction price in step (iv) above. The Company uses judgment to determine whether milestones or other variable consideration, except for royalties, should be included in the transaction price as described further below. The transaction price is allocated to each performance obligation on a relative stand-alone selling price basis, for which the Company recognizes revenue as or when the performance obligations under the contract are satisfied. In determining the stand-alone selling price of a license to the Company's proprietary technology or a material right provided by a customer option, the Company considers market conditions as well as entity-specific factors, including those factors contemplated in negotiating the agreements as well as internally developed estimates that include assumptions related to the market opportunity, estimated development costs, probability of success and the time needed to commercialize a product candidate pursuant to the license. In validating its estimated stand-alone selling prices, the Company evaluates whether changes in the key assumptions used to determine its estimated stand-alone selling prices will have a significant effect on the allocation of arrangement consideration between performance obligations.

The Company estimates the transaction price based on the amount of consideration the Company expects to be received for transferring the promised goods or services in the contract. The consideration may include both fixed consideration and variable consideration. At the inception of each arrangement that includes variable consideration, the Company evaluates the amount of the potential payments and the likelihood that the payments will be received. The Company utilizes either the most likely amount method or expected value method to estimate the transaction price based on which method better predicts the amount of consideration expected to be received. If it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur, the variable consideration is included in the transaction price.

Performance obligations are promised goods or services in a contract to transfer a distinct good or service to the customer. Promised goods or services are considered distinct when: (i) the customer can benefit from the good or service on its own or together with other readily available resources and (ii) the promised good or service is separately identifiable from other promises in the contract. In assessing whether promised goods or services are distinct, the Company considers factors such as the stage of development of the underlying intellectual property, the capabilities of the customer to develop the intellectual property on their own and whether the required expertise is readily available.

For performance obligations which consist of licenses and other promises, the Company utilizes judgment to assess the nature of the combined performance obligation in order to determine whether the combined performance obligation is satisfied over time or at a point in time. Up-front payments and fees are recorded as deferred revenue upon receipt or when due until the Company performs its obligations under these arrangements. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as current portion of deferred revenue in the accompanying consolidated balance sheets. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion. Amounts are recorded as accounts receivable when the Company's right to consideration is unconditional. Amounts recognized as revenue, but not yet received or invoiced are generally recognized as contract assets.

Exclusive Licenses

If the license granted in the arrangement is determined to be distinct from the other promises or performance obligations identified in the arrangement, which generally include research and development services, the Company recognizes revenue from non-refundable, upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license In assessing whether a license is distinct from the other promises, the Company considers relevant facts and circumstances of each arrangement, including the research and development capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can benefit from the license for its intended purpose without the receipt of the remaining promise, whether the value of the license is dependent on the unsatisfied promise, whether there are other vendors that could provide the remaining promise, and whether it is separately identifiable from the remaining promise. For licenses that are combined with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. The measure of progress, and thereby periods over which revenue should be recognized, are subject to estimates by management and may change over the course of the arrangement.

Research and Development Services

The promises under the Company's collaboration and license agreements generally include research and development services to be performed by the Company on behalf of the collaboration partner. For performance obligations that include research and development services, the Company generally recognizes revenue allocated to such performance obligations based on an appropriate measure of progress. The Company utilizes judgment to determine the appropriate method of measuring progress for purposes of recognizing revenue, which is generally an input measure, such as costs incurred.

Milestone Payments

At the inception of each arrangement that includes milestone payments based on certain events, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. The Company evaluates factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the particular milestone in making this assessment. There is considerable judgment involved in determining whether it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur. At the end of each subsequent reporting period, the Company reevaluates the probability of achievement of all milestones subject to constraint and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment. If a milestone or other variable consideration relates specifically to the Company's efforts to satisfy a single performance obligation or to a specific outcome from satisfying the performance obligation, the Company generally allocates the milestone amount entirely to that performance obligation once it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur.

Royalties

For arrangements that include sales-based royalties, including milestone payments based on a level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of its licensing arrangements.

Collaboration Revenue

The Company analyzes its collaboration arrangements to assess whether they are within the scope of ASC 808, *Collaborative Arrangements* ("ASC 808") to determine whether such arrangements involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities. This assessment is performed throughout the life of the arrangement based on changes in the responsibilities of all parties in the arrangement. For collaboration arrangements within the scope of ASC 808 that contain multiple elements, the Company first determines which elements of the collaboration are deemed to be within the scope of

ASC 808 and those that are more reflective of a vendor-customer relationship and therefore within the scope of ASC 606. For elements of collaboration arrangements that are accounted for pursuant to ASC 808, an appropriate recognition method is determined and applied consistently, generally by analogy to ASC 606. For those elements of the arrangement that are accounted for pursuant to ASC 606, the Company applies the five-step model described above.

Costs Associated with License and Collaborative Arrangements

All costs associated with license and collaborative arrangements are expensed as incurred and recorded in research and development expense in the consolidated statements of operations.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development expenses include salaries, equity-based compensation and benefits of employees, third-party license fees and other operational costs related to the Company's research and development activities, including allocated facility-related expenses and external costs of outside vendors engaged to conduct both preclinical studies and clinical trials. Non-refundable pre-payments for goods or services that will be used or rendered for future research and development activities are deferred and capitalized. Such amounts are recognized as expense as the goods or services are delivered or the related services are performed, or until it is no longer expected that the goods will be delivered or the services rendered.

The Company may in-license the rights to develop and commercialize product candidates. For each in-license transaction the Company evaluates whether it has acquired processes or activities along with inputs that would be sufficient to constitute a "business" as defined under GAAP. A "business" as defined under GAAP consists of inputs and processes applied to those inputs that have the ability to create outputs. Although businesses usually have outputs, outputs are not required for an integrated set of activities to qualify as a business. When the Company determines that it has not acquired sufficient processes or activities to constitute a business, any up-front payments, as well as milestone payments, are immediately expensed as acquired research and development in the period in which they are incurred.

Research Contract Costs and Accruals

The Company has entered into various research and development contracts with research institutions and other companies. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies, including the phase or completion of events, invoices received and contracted costs. Estimates based on available information are made in determining the accrual balances at the end of any reporting period. Actual results could differ from the Company's estimates; however, the Company's historical accrual estimates have not been materially different from the actual costs.

Patent Costs

All patent-related costs incurred for filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

Equity-Based Compensation

The Company measures all stock options and other stock-based awards granted to employees, directors and non-employees based on the fair value on the date of the grant and recognizes compensation expense of those awards, over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. The Company applies the straight-line method of expense recognition to all awards with only service-based vesting conditions. The Company has not issued any awards with performance-based vesting conditions.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. The Company historically has been a private company and lacks company-specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. For options with service-based vesting conditions and options granted to non-employees, the expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

The fair value for restricted stock units ("RSU") was calculated using the closing price of the Company's common stock on the date of grant.

The Company classifies stock-based compensation expense in its consolidated statement of operations in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

Income Taxes

Income taxes are accounted for under the asset and liability method. Under the asset and liability method, deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date. The Company records valuation allowances to reduce deferred income tax assets to the amount that is more likely than not to be realized. The Company determines whether it is more likely than not that a tax position will be sustained upon examination. If it is not more likely than not that a position will be sustained, no amount of benefit attributable to the position is recognized. The tax benefit to be recognized of any tax position that meets the more likely than not recognition threshold is calculated as the largest amount that is more than 50% likely of being realized upon resolution of the contingency.

Prior to January 25, 2018, the Company had not been subject to U.S. federal income taxes as the Company was organized as a limited liability company. As such, the taxable income or loss was passed through to and included in the tax returns of the members. Since January 25, 2018, the Company's income has since been subject to U.S. federal, state, local, and foreign income taxes and taxed at the prevailing corporate tax rates.

Segment Data

The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions. The Company's singular focus is on developing treatments through gene therapy and other means for patients with neuromuscular and cardiac diseases. All of the Company's tangible assets are held in the United States.

Comprehensive Loss

Comprehensive loss includes net loss, as well as other changes in stockholders' equity that result from transactions and economic events other than those with members. The Company's only element of other comprehensive income (loss) in all periods presented was unrealized gains (losses) from available-for-sale securities.

Net Loss per Share

The Company follows the two-class method when computing net loss per share, as the Company has issued shares that meet the definition of participating securities. The two-class method determines net loss per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed.

Basic net loss per share is computed by dividing the net loss by the weighted average number of shares of common stock and pre-funded warrants outstanding for the period. Diluted net loss is computed by adjusting net loss to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net loss per share is computed by dividing the diluted net loss by the weighted average number of shares of common stock and pre-funded warrants outstanding for the period, including potential dilutive shares of common stock assuming the dilutive effect of common stock equivalents.

The Company's preferred stock could entitle the holders of such shares to participate in dividends and not require the holders of such shares to participate in losses of the Company. Accordingly, in periods in which the Company reports a net loss, such losses are not allocated to such participating securities. In periods in which the Company reports a net loss, diluted net loss per share is the same as basic net loss per share, since dilutive shares of common stock are not assumed to have been issued if their effect is anti-dilutive. As of the years ended December 31, 2023, 2022, and 2021, there was no preferred stock issued or outstanding with any contractual rights.

Contingencies

Loss contingency provisions are recorded if the potential loss from any claim, asserted or unasserted, or legal proceeding, is considered probable and the amount can be reasonably estimated, or a range of loss can be determined. These accruals represent the Company's best estimate of probable loss. Disclosure also is provided when it is reasonably possible that a loss will be incurred or when it is reasonably possible that the amount of a loss will exceed the recorded provision. The Company reviews the status of each significant matter and assesses its potential financial exposure. Significant judgment is required in both the determination of probability and the determination as to whether an exposure is reasonably estimable. Because of uncertainties related to these matters, accruals are based only on the best information available at the time. As additional information becomes available, the Company reassesses the potential liability related to pending claims and may change its estimates. These changes in the estimates of the potential liabilities could have a material impact on the Company's consolidated results of operations and financial position.

Business Combinations

The Company's consolidated financial statements include the operations of acquired businesses after the completion of the acquisitions. The Company accounts for acquired businesses using the acquisition method of accounting. Application of this method of accounting requires that (i) identifiable assets acquired (including identifiable intangible assets) and liabilities assumed be measured and recognized at fair value as of the acquisition date, and (ii) the excess of the purchase price over the net fair value of identifiable assets acquired and liabilities assumed be recorded as goodwill. Acquired in-process research and development ("IPR&D") is recognized at fair value and initially characterized as an indefinite-lived intangible asset, irrespective of whether the acquired IPR&D has an alternative future use. Transaction costs are expensed as incurred. Amounts assigned to goodwill and other identifiable intangible assets are based on independent appraisals or internal estimates.

Recently Issued Accounting Pronouncements

In December 2023, the Financial Accounting Standards Board issued Accounting Standards Update ("ASU") No. 2023-09, "Income Taxes (Topic 740): Improvements to Income Tax Disclosures." This ASU updates income tax disclosure requirements primarily by requiring specific categories and greater disaggregation within the rate reconciliation and disaggregation of income taxes paid by jurisdiction. This ASU is effective for annual periods beginning after December 15, 2024 and is applicable to the Company's fiscal year beginning January 1, 2025, with early application permitted. The Company is currently evaluating the impact of adopting this ASU on its consolidated financial statements and disclosures.

3. Collaborations

Ultragenyx Collaboration

Collaboration Agreement

On October 22, 2020, the Company entered into the Collaboration Agreement with Ultragenyx Pharmaceutical Inc. ("Ultragenyx ") to focus on the development and commercialization of new gene therapies for Duchenne. The Company granted Ultragenyx an exclusive worldwide license for any pharmaceutical product that expresses the Company's proprietary microdystrophin construct from AAV8 and variants thereof in clade E for the treatment of Duchenne and other diseases resulting from the lack of functional dystrophin (the "Licensed Products"). The Company retains exclusive rights to all other uses of its microdystrophin proteins, including under its SGT-003 program.

The Company has conducted certain research and development activities with respect to the development of the Licensed Products, and concluded such activities as were contemplated under the Collaboration Agreement during the second quarter of 2022, resulting in the recognition of the remaining deferred revenue recorded at the time the Collaboration Agreement was executed, related to the upfront payment received from Ultragenyx. The Company may conduct additional research and development activities in collaboration with Ultragenyx from time to time in the future. Ultragenyx reimbursed the Company for personnel and out-of-pocket costs that the Company incurred in conducting such activities.

In addition, Ultragenyx granted to the Company an exclusive Development Option or Income Share Option (each as defined and described below) exercisable in the Company's sole discretion one time per Licensed Product. After the date of first achievement of clinical proof of concept, Ultragenyx will provide to the Company a data package with respect to the relevant

Licensed Product. The Company will use the data package to determine whether to exercise the corresponding Development Option or Income Share Option with respect to such Licensed Product.

With respect to each Licensed Product for which the Company has not exercised the Development Option or Income Share Option the Company will be entitled to milestone payments of up to $25,000 in the aggregate for each such Licensed Product that achieves specified development milestones and $65,000 in the aggregate for each such Licensed Product that achieves specified regulatory milestones. With respect to each Licensed Product for which the Company has not exercised the Income Share Option, the Company will also be entitled to milestone payments of up to $165,000 in the aggregate for each Licensed Product that achieves specified annual worldwide net sales milestones. For Licensed Products for which the Company has not exercised the Development Option or Income Share Option, Ultragenyx will pay the Company tiered royalties on a Licensed Product-by-Licensed Product and country-by-country basis ranging from a low double-digit percentage to a mid-teens percentage based on Ultragenyx's annual worldwide net sales of such Licensed Products.

For each Licensed Product for which Ultragenyx decides to initiate a registrational trial in humans, the Company will have the option to fund 30% of the development costs in the United States and European Union for such Licensed Product and forgo the development and regulatory milestones (the "Development Option") and receive tiered royalties on a Licensed Product-by-Licensed Product and country-by-country basis ranging from a mid-teens percentage to a low twenties percentage based on Ultragenyx's annual worldwide net sales of each such Licensed Product.

For each Licensed Product for which the Company exercises the Development Option, the Company may also elect to share 30% of the net income and net losses on net sales of such Licensed Product in the United States and European Union (the "Income Share Option"). For Licensed Products for which the Company has exercised the Income Share Option, the Company will not be entitled to milestone payments and Ultragenyx will pay the Company tiered royalties on a Licensed Product-by-Licensed Product and country-by-country basis ranging from a mid-teens percentage to a low twenties percentage based on Ultragenyx's annual net sales of each such Licensed Product outside of the United States and European Union.

The Company may only exercise an Income Share Option if neither the Company nor any of its affiliates is then developing or commercializing a product that is competitive with the Licensed Product that is subject to such option. If the Company or any of its affiliates subsequently develops or commercializes a product that is competitive with a Licensed Product for which the Company has exercised an Income Share Option, then the Company and Ultragenyx will no longer share the net income and net losses on net sales of such Licensed Product and such Licensed Product will be treated as if the Company had exercised the Development Option with respect to such Licensed Product.

Following the Company's exercise of the Development Option or Income Share Option with respect to a Licensed Product, the Company also has the right to cease participation in the sharing of development costs and sharing in net income and net losses on net sales, as applicable, for such Licensed Product by written notice to Ultragenyx. Upon such notice, the Company will no longer share in the development costs and net income and net losses on net sales of such Licensed Product, as applicable, and will be eligible to receive payments on milestones achieved after the opt-out for such Licensed Product and royalties at the rates applicable to Licensed Products for which the Company has not exercised the Development Option or Income Share Option, as described above.

The Collaboration Agreement continues on a country-by-country and Licensed Product-by-Licensed Product basis until the expiration of all payment obligations under the agreement. With respect to any Licensed Product for which the Company has exercised an Income Share Option, the Collaboration Agreement continues until there are no longer sales of such Licensed Product in the United States or Europe. Either party has the right to terminate the agreement if the other party has materially breached in the performance of its obligations under the agreement and such breach has not been cured within the applicable cure period. Ultragenyx may also terminate the Collaboration Agreement in its sole discretion upon 90 days' prior written notice to the Company.

Stock Purchase Agreement

In connection with the execution of the Collaboration Agreement, Ultragenyx and the Company also entered into a stock purchase agreement (the "Stock Purchase Agreement") on the Effective Date, pursuant to which the Company issued and sold 521,719 shares of its common stock (the "Shares") to Ultragenyx at a price of $76.6695 per share for an aggregate purchase price of approximately $40,000. The Stock Purchase Agreement contains customary representations, warranties and covenants of each of the parties thereto. Following the sale of the Shares, Ultragenyx beneficially owned approximately 14.45% of the Company's outstanding common stock. As of December 31, 2023, Ultragenyx beneficially owned approximately 2.6% of the Company's outstanding common stock.

Investor Agreement

In connection with the consummation of the transactions contemplated by the Stock Purchase Agreement, the Company and Ultragenyx entered into an Investor Agreement (the "Investor Agreement") on the Effective Date. Pursuant to the terms of

the Investor Agreement, Ultragenyx agreed that, so long as it holds at least 10% of the Company's outstanding common stock, the Shares will be subject to a voting agreement, such that until the earliest to occur of certain specified events, and subject to specified conditions, Ultragenyx will, and will cause its permitted transferees to, vote in accordance with the recommendation of the Company's Board of Directors with respect to specified matters.

Accounting Treatment

The Company concluded that the Collaboration Agreement and the Stock Purchase Agreement should be combined and treated as a single arrangement for accounting purposes as the agreements were entered into contemporaneously and in contemplation of one another.

The Company assessed this arrangement in accordance with ASC 606 and concluded that the contract counterparty, Ultragenyx, is a customer. The Company identified the following promises in the Collaboration Agreement that were evaluated under the scope of ASC 606: (1) an exclusive worldwide license to the Licensed Products; (2) an obligation to perform research and development services; and (3) an obligation to participate in a joint steering committee. The Company assessed the promised goods and services to determine if they are distinct. Based on this assessment, the Company determined that Ultragenyx cannot benefit from the promised goods and services separately from the others as they are highly interrelated and therefore not distinct. Due to the early stage of the Licensed Products, the research and development services could not be performed by another party. The Company's skill-set, knowledge and expertise are required to conduct the research and development services and the research and development services are expected to involve significant further development of the Licensed Products. Accordingly, the promised goods and services represent one combined performance obligation and the entire transaction price will be allocated to that single combined performance obligation.

The Company determined the transaction price under ASC 606 at the inception of the Collaboration Agreement to be $22,513, which represents the excess proceeds from the equity investment under the Stock Purchase Agreement, when measured at fair value after taking into consideration a discount for lack of marketability, plus the estimated reimbursement of research and development costs, which represents variable consideration. The Company included the estimated reimbursement of research and development costs in the transaction price at the inception of the arrangement because the Company is required to perform research and development services and the contract requires Ultragenyx to reimburse the Company for costs incurred. Also, since the related revenue would be recognized only as the costs are incurred, the Company determined it is not probable that a significant reversal of cumulative revenue would occur. The Company evaluated how much variable consideration related to development and regulatory milestones, and the Company's potential exercise of its Development Option or Income Share Option per Licensed Product, to include in the transaction price using the most likely amount approach and concluded that no amount should be included in the transaction price due to the high degree of uncertainty and risk associated with these potential payments. The Company also determined that royalties and sales milestones relate solely to the license of intellectual property and are therefore excluded from the transaction price under the sales- or usage-based royalty exception of ASC 606. Revenue related to these royalties and sales milestones will only be recognized when the associated sales occur, and relevant thresholds are met.

The Company determined that revenue under the Collaboration Agreement should be recognized over time as Ultragenyx simultaneously receives the benefit from the Company as the Company performs under the single performance obligation over time. The Company will recognize revenue for the single performance obligation using a cost-to-cost input method as the Company has concluded it best depicts the research and development and joint steering committee participation services performed. Under this method, the transaction price is recognized over the contract's entire performance period, using costs incurred relative to total estimated costs to determine the extent of progress towards completion.

Ultragenyx was a related party because Ultragenyx was one of the Company's significant stockholders as of December 31, 2021 and continued to be a stockholder as of December 31, 2022. However, Ultragenyx is not a significant stockholder as of December 31, 2023. The Company did not recognize any related party collaboration revenue associated with its collaboration with Ultragenyx during the year ended December 31, 2023. The Company recognized $8,094 and $13,620 related party collaboration revenue during the year ended December 31, 2022 and 2021, respectively. Further, the Company has made no payments to Ultragenyx during the years ended December 31, 2023 and 2022. There are no amounts due from Ultragenyx as of December 31, 2023 and December 31, 2022. The amount received is deferred as a contract liability on the Company's consolidated balance sheet as the performance obligation has been fully satisfied as of December 31, 2022.

During the year ended December 31, 2023, the Company had no changes in related party collaboration receivables or contract liabilities. The following table presents changes in the balances of the Company's related party collaboration receivables and contract liabilities during the year ended December 31, 2022:

	Balance as of December 31, 2021	Additions	Deductions	Balance as of December 31, 2022
Related party collaboration receivable	$ 110	$ 14	$ (124)	$ —
Contract liabilities:				
Deferred revenue	8,080	—	(8,080)	—

The changes in the related party collaboration receivables balance during the year ended December 31, 2022 are the result of amounts owed to the Company for research and development services provided, offset by the collections received from Ultragenyx.

There was no deferred revenue related to Collaboration Agreement during the years ended December 31, 2023 and 2022. Additionally, there was no related party collaboration receivables during the years ended December 31, 2023 and 2022.

Costs incurred relating to the Collaboration Agreement consist of internal and external research and development costs, which primarily include salaries and benefits, lab supplies, preclinical research studies, clinical studies, consulting services, and commercial development. These costs are included in research and development expenses in the Company's consolidated statement of operations during the years ended December 31, 2022 and 2021. There was no costs incurred during the year ended December 31, 2023.

4. Acquisition

On September 29, 2022, the Company entered into an Agreement and Plan of Merger with AavantiBio. The Acquisition closed on December 2, 2022 and was announced on December 5, 2022. This acquisition allows the Company to add to its pipeline of assets. The Company acquired AavantiBio for a total purchase price of $9,169, including (i) $1 in cash and (ii) 1,354,258 shares of its common stock, par value $0.001 per share with a fair value of $9,168 to AavantiBio equityholders. The price per share of the Company's common stock used in the calculation of the purchase price is based on the closing price of Solid's common stock on the Nasdaq Global Select Market on December 2, 2022, which was $6.77.

The Acquisition was accounted for as a business combination in which the Company, as the accounting acquirer, recorded the assets acquired and liabilities assumed from AavantiBio at their fair values as of the acquisition date. The Company recognized a gain on the purchase of AavantiBio of $18,236 as the net assets acquired of $27,405 were greater than the purchase price of $9,169. Prior to recognizing the gain, the Company reassessed the measurement and recognition of identifiable assets acquired, and liabilities assumed and concluded that the valuation procedures and resulting measures were appropriate, in all material respects. The Company believes that its ability to negotiate a purchase price lower than the fair market value of the acquired net assets was due to a combination of factors, including the then prevailing market conditions and the uncertain future macroeconomic environment. The Company believes the seller, as a smaller, less well capitalized company, was motivated to complete the transaction under the terms described above as growing economic uncertainty and a rising interest rate environment negatively impacted their ability to raise additional capital.

The Company incurred acquisition related costs of $4,870, which are included in general and administrative expenses in the Company's consolidated statements of comprehensive loss for the fiscal year ended December 31, 2022.

The fair value is determined utilizing the fair value hierarchy as described in *Note 2, Summary of Significant Accounting Policies,* and *Note 5, Fair Value of Financial Assets and Liabilities*.

5. Fair Value of Financial Assets and Liabilities

The following tables present information about the Company's assets and liabilities that are measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values:

	Fair Value Measurements as of December 31, 2023:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ —	$ 62,398	$ —	$ 62,398
Available-for-sale securities	—	49,625	—	49,625
	$ —	$ 112,023	$ —	$ 112,023

	Fair Value Measurements as of December 31, 2022:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ —	$ 69,374	$ —	$ 69,374
Available-for-sale securities	—	58,338	—	58,338
	$ —	$ 127,712	$ —	$ 127,712

As of December 31, 2023 and December 31, 2022 the fair values of the Company's cash equivalents and available-for-sale securities were determined using Level 2 inputs. During the year ended December 31, 2023 and December 31, 2022, there were no transfers between Level 1, Level 2 and Level 3, respectively.

The fair value of the Company's cash, restricted cash, accounts payable, accrued expenses and other current liabilities approximate their carrying value due to their short-term maturities.

6. Available-for-Sale Securities

As of December 31, 2023, the fair value of available-for-sale debt securities by type of security was as follows:

	December 31, 2023			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Investments:				
Treasury bills	$ 49,610	$ 15	$ —	$ 49,625
	$ 49,610	$ 15	$	$ 49,625

As of December 31, 2022, the fair value of available-for-sale debt securities by type of security was as follows:

	December 31, 2022			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Investments:				
Treasury bills	$ 34,780	$ —	$ (30)	$ 34,750
Corporate bond securities	23,626	—	(38)	23,588
	$ 58,406	$	$ (68)	$ 58,338

The estimated fair value and amortized cost of the Company's available-for-sale securities by contractual maturity are summarized as follows:

	December 31, 2023	
	Amortized Cost	Fair Value
Due in one year or less	$ 49,610	$ 49,625
Total available-for-sale securities	$ 49,610	$ 49,625

	December 31, 2022	
	Amortized Cost	Fair Value
Due in one year or less	$ 58,406	$ 58,338
Total available-for-sale securities	$ 58,406	$ 58,338

The average maturity of the Company's available-for-sale securities as of December 31, 2023 and December 31, 2022 was approximately 0.4 years and 0.5 years, respectively.

7. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following:

	December 31,	
	2023	2022
Prepaid research and development expenses	$ 3,980	$ 2,913
Prepaid expenses and other assets	2,114	3,003
	$ 6,094	$ 5,916

8. Property and Equipment

Property and equipment consisted of the following:

	December 31,	
	2023	2022
Furniture and fixtures	$ 936	$ 868
Laboratory equipment	16,137	16,416
Leasehold improvements	481	384
Computer equipment	842	677
Computer software	553	553
Construction in process	410	1,715
	19,359	20,613
Less accumulated depreciation	12,735	10,956
	$ 6,624	$ 9,657

Depreciation expense was $2,581, $2,408 and $2,964 for the years ended December 31, 2023, 2022 and 2021, respectively. The Company recognized an impairment loss of $374, $0, and $92 for the years ended December 31, 2023, 2022 and 2021, respectively.

9. Accrued Expenses

Accrued expenses and other current liabilities consisted of the following:

	December 31,	
	2023	2022
Accrued research and development	$ 2,614	$ 3,033
Accrued compensation	5,948	8,370
Accrued other	1,575	5,288
	$ 10,137	$ 16,691

10. Equity-Based Compensation

Equity Incentive Plans

In connection with the closing of the Company's initial public offering, the Company's Board of Directors and stockholders approved the 2018 Omnibus Incentive Plan (the "2018 Plan"), which provides for the reservation of 333,400 shares of common stock for equity awards. On June 16, 2020, the Company's stockholders approved the 2020 Equity Incentive Plan (as amended or restated, the "2020 Plan") which consisted of, at the time of approval, (i) 200,000 shares of common stock and (ii) additional shares of common stock (up to 325,268) as is equal to (i) the number of shares reserved under the 2018 Plan that remain available for grant under the 2018 Plan as of immediately prior to the date the 2020 Plan was approved by the Company's stockholders and (ii) the number of shares subject to awards granted under the 2018 Plan which awards expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right.

As of the effective date of the 2020 Plan, no further awards will be made under the 2018 Plan. Any options or awards outstanding under the 2018 Plan remain outstanding and effective and are governed by their existing terms.

On June 16, 2021, the Company's stockholders approved an amendment to the 2020 Plan to reserve an additional 466,666 shares of common stock for issuance under the plan.

On December 1, 2022, the Company's stockholders approved an amendment and restatement of the 2020 Plan to (i) increase the number of shares of common stock reserved for issuance under the plan by 866,666 shares to 1,533,333 shares, subject to adjustment in the event of stock splits and other similar events, (ii) provide for an annual increase, to be added on the first day of each fiscal year during the term of the plan, beginning with the fiscal year ending December 31, 2023, of 5% of the number of shares of common stock outstanding on the first day of such fiscal year or a lesser number of shares determined by the Company's Board of Directors, (iii) provide that up to 1,858,601 shares of common stock may be granted as "incentive stock options" under the plan, (iv) extend the term of the plan to December 1, 2032 and (v) revise certain provisions of the plan relating to the Company's Board of Directors' ability to delegate authority to make awards under the plan.

At December 31, 2023, 738,778 shares remained available for future issuance under the 2020 Plan. Under the 2020 Plan, stock options may not be granted at less than fair value on the date of grant.

2021 Employee Stock Purchase Plan

The 2021 Employee Stock Purchase Plan (the "ESPP") was adopted by the Company's Board of Directors on April 14, 2021, approved by the stockholders on June 16, 2021, and became effective on June 16, 2021. The first offering period under the ESPP commenced on September 1, 2021.

On June 6, 2023, the Company's stockholders approved an amendment and restatement of the ESPP to (i) increase the number of shares of common stock reserved for issuance under the ESPP from 73,525 to 473,525 and (ii) provide for an annual increase to be added on the first day of each fiscal year, beginning with the fiscal year ending December 31, 2024 and ending with the fiscal year ending December 31, 2033, equal to the least of (a) 293,597 shares of common stock, (b) one percent (1%) of the outstanding shares of common stock on such date and (c) the number of shares of common stock determined by the Board of Directors. The Company amended and restated the ESPP on November 12, 2023 to provide for 24-month offering periods.

The number of shares of the Company's common stock reserved for issuance under the ESPP is 473,525 shares. At December 31, 2023, 397,546 shares remained available for future issuance under the ESPP.

Stock Options

The following table summarizes the Company's stock option activity for the year ended December 31, 2023:

	Number of Options	Weighted Average Exercise Price	Remaining Contractual Life (in years)
Outstanding at December 31, 2022	1,433,968	$ 36.22	8.88
Granted	1,141,449	5.38	
Exercised	—	—	
Expired	(54,941)	81.84	
Forfeitures	(260,804)	15.07	
Outstanding at December 31, 2023	2,259,672	$ 21.93	8.20
Vested and expected to vest as of December 31, 2023	2,259,672	$ 21.93	8.20
Exercisable at December 31, 2023	616,133	$ 57.99	7.69

At December 31, 2023, the Company had an aggregate of $8,949 of unrecognized equity-based compensation cost related to stock options outstanding which is expected to be recognized over a weighted average period of 2.8 years. The intrinsic value of stock options outstanding as of December 31, 2023 and 2022 was $863 and $0, respectively. The intrinsic value of stock options exercisable as of December 31, 2023 and 2022 was $27 and $0, respectively. No stock options were exercised during the year ended December 31, 2023.

The fair value of each option award is estimated on the date of grant using the Black-Scholes option-pricing model using the assumptions noted in the following table for the years ended December 31:

	2023	2022	2021
Expected volatility	121.8% - 129.60%	118.5% - 130.3%	115.5% - 123.9%
Expected dividends	0.0%	0.0%	0.0%
Expected term (in years)	5.31 - 6.25	5.31 - 6.25	5.10 - 6.25
Risk-free rate	3.5% - 4.9%	1.4% - 3.9%	0.4% - 1.4%

The weighted average fair value of options to purchase shares of common stock granted during the year ended December 31, 2023 and 2022 was $4.76 and $7.59, respectively.

Restricted Stock Units

In 2023, 2022 and 2021, the Company's Board of Directors issued restricted stock units to employees. Restricted stock unit grants typically vest over one to four years.

The following table summarizes the Company's restricted stock unit activity for the year ended December 31, 2023:

	Units	Weighted-Average Grant Date Fair Value
Unvested at December 31, 2022	512,557	$ 8.39
Granted	666,516	5.46
Vested	(183,951)	7.64
Forfeitures	(117,941)	7.96
Outstanding at December 31, 2023	877,181	$ 6.37
Unvested as of December 31, 2023	877,181	$ 6.37

At December 31, 2023, the Company had an aggregate of $4,293 of unrecognized equity-based compensation cost related to restricted stock units outstanding. The unrecognized expense for the restricted stock units is expected to be recognized over a weighted average period of 2.59 years.

Restricted Common Stock

In connection with the Company's statutory corporate conversion on January 25, 2018, all restricted Series B and D common units were converted into restricted shares of common stock. As of December 31, 2021, there were no remaining restricted shares of common stock outstanding.

No restricted common shares vested during the years ended December 31, 2023 and 2022. The aggregate intrinsic value of restricted common shares that vested during the year ended December 31, 2021 was $199.

At December 31, 2023, the Company had an aggregate of $0 of unrecognized equity-based compensation related restricted shares of common stock.

The Company recorded equity-based compensation expense related to all of its share-based awards to employees and non-employees in the following captions within its consolidated statements of operations for the years ended December 31, 2023, 2022, and 2021:

	For the Year Ended December 31,		
	2023	2022	2021
Research and development expenses	$ 3,094	$ 2,756	$ 6,289
General and administrative expenses	4,531	4,781	7,084
	$ 7,625	$ 7,537	$ 13,373

11. Leases

The Company has operating leases for laboratory and office space in Massachusetts, North Carolina and Florida.

In June 2021, the Company entered into a lease with Hood Park LLC ("Landlord"), pursuant to which the Company leases approximately 49,869 square feet of office, laboratory, research and development and manufacturing space located in Charlestown, Massachusetts ("Premises"). The Company relocated its corporate headquarters to the Premises in June 2022. The initial term of the lease commenced in June 2022 when the construction of the lessor assets was substantially completed and continues for a ten-year period, unless earlier terminated. The lease provides the Company with an option to extend the lease for an additional five-year term. The Company and the Landlord were each obligated to undertake certain improvements prior to the commencement of the lease, and significant improvements were completed as of June 2022. The monthly lease payment is approximately $305 with annual escalation of approximately 3%. The lease includes a $10,223 construction allowance which is considered a lease incentive and included within the right-of-use asset. The Company was required to post a customary letter of credit in the amount of $1,833, subject to decrease on a set schedule, as a security deposit pursuant to the lease.

During the year ended December 31, 2022, the Company recorded a failed sales-leaseback transaction related to certain lab equipment. The related financing liabilities are recorded on the Company's consolidated balance sheets within financing liabilities. In connection with this transaction, the Company also recorded a cash inflow within the financing activities under proceeds from financing liabilities of $2,143.

As of December 31, 2023, minimum future lease payments for these operating and finance leases were as follows:

	Finance Leases	Operating Leases
2024	$ 810	$ 4,396
2025	651	4,110
2026	—	4,123
2027	—	4,239
Thereafter	—	21,482
Total	1,461	38,350
Less: Imputed Interest	(242)	13,788
Total Lease Liabilities	$ 1,703	$ 24,562

The Company recorded rent expense of $5,255, $3,881 and $2,568 for the years ended December 31, 2023, 2022, and 2021, respectively.

Short-term lease and variable lease costs were not material for the year ended December 31, 2023 and 2022.

The supplemental disclosure of cash flow information related to the Company's leases and the weighted average remaining lease term and weighted average discount rate of the Company's leases are as follows:

	For the Year Ended December 31, 2023	For the Year Ended December 31, 2022	For the Year Ended December 31, 2021
Other information			
Cash paid for amounts included in the measurement of lease liabilities	$ 5,310	$ 2,840	$ 2,408
Operating lease liabilities arising from obtaining right-of-use-assets	$ 410	$ 29,126	$ 310
Finance lease liabilities arising from obtaining right-of-use assets	$ —	$ —	$ —
Weighted-average remaining lease term (in years)			
Operating lease	8.5	9.3	1.0
Finance lease	1.8	2.5	2.3
Weighted-average discount rate			
Operating lease	10.7%	10.6%	11.9%
Finance lease	22.8%	21.3%	10.7%

12. Commitments and Contingencies

Letter of Credit

The Company had an outstanding letter of credit in the amount of $1,833 at December 31, 2023 and 2022, which was required as a condition of the Company's office and laboratory leases.

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters, including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with its executive officers and members of its Board of Directors that require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as executive officers or directors. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnification arrangements.

The Company does not believe that the outcome of any claims under indemnification arrangements will have a material effect on its financial position, results of operations or cash flows, and it has not accrued any liabilities related to such obligations in its consolidated financial statements as of December 31, 2023 and 2022.

Legal Proceedings

The Company may periodically become subject to legal proceedings and claims arising in connection with ongoing business activities, including claims or disputes related to patents that have been issued or that are pending in the field of research on which the Company is focused. The Company is not aware of any material legal proceedings or claims as of December 31, 2023.

13. License Agreements

University of Washington License Agreement

In 2015, the Company entered into a license agreement with the University of Washington, acting through UW CoMotion, under which the Company obtained an exclusive, royalty-bearing, sublicensable, worldwide license under a patent application owned by the University of Washington relating to novel micro-dystrophins and all patents claiming priority to such patent to develop, manufacture, and commercialize products for use in the treatment of Duchenne and related disease indications caused by a lack of functional dystrophin. The Company has the right to grant sublicenses to third parties contingent upon written approval by the University of Washington prior to executing such sublicense, which approval may not be unreasonably withheld.

In consideration for the rights granted by the agreement, the Company paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. The Company is required to reimburse the University of Washington for costs incurred in applying for, prosecuting and maintaining patents and pay up to an aggregate of approximately $1,000 upon the achievement of certain milestones. In October 2017, the first milestone was achieved under this agreement. The milestone payment was recorded as a research and development expense in the fourth quarter of 2017. In October 2020, the license agreement was amended such that the Company was required to pay the University of Washington $375 in connection with the execution of the Collaboration Agreement. This payment was recorded as a research and development expense in the fourth quarter of 2020. The license agreement was also amended such that the Company is required to pay an aggregate of approximately $3,400 upon the achievement of certain milestones. There were no milestones achieved during the years ended December 31, 2023, 2022, and 2021. The Company must also pay royalties of a low single digit percentage of future sales by the Company and its sublicensees of products developed under the licensed patent rights. In addition, the Company must pay an annual maintenance fee until certain milestones are achieved, at which time a minimum annual royalty requirement will replace such maintenance fee and will apply to the Company and its sublicensees.

The license agreement remains in effect until the expiration of the last-to-expire patent licensed under the agreement. The Company may terminate the agreement at any time upon providing sixty days' written notice to the University of Washington. The University of Washington may terminate the agreement upon the Company's uncured, material breach of the agreement or if the Company enters into an insolvency-related event.

The Company recorded research and development expense in the amount of $41, $96, and $60 for the years ended December 31, 2023, 2022, and 2021, respectively, under the agreement.

The University of Missouri License Agreement

In 2015, the Company entered into a license agreement with the Curators of the University of Missouri (the "University of Missouri"), a public corporation of Missouri, under which the Company obtained an exclusive, royalty-bearing, sublicensable, worldwide license under certain patent and patent applications owned by the University of Missouri relating to a novel synthetic microdystrophin gene to make, sell and distribute products for use in the treatment of Duchene and related disease indications resulting from a lack of functional dystrophin.

In consideration for the rights granted by the agreement, the Company paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. The Company is required to reimburse the University of Missouri for costs incurred in applying for, prosecuting and maintaining the licensed patents and pay up to an aggregate of approximately $1,000 upon the achievement of certain milestones for each product developed based on the licensed patents. In October 2017, the first milestone was achieved under this agreement. The milestone payment was recorded as a research and development expense in the fourth quarter of 2017.

Under the agreement, in the event the Company grants a sublicenses to another party, the Company is required to pay the University of Missouri a percentage of the consideration received. The license agreement was amended such that the Company was required to pay the University of Missouri $750 in 2021 and $1,300 in 2022 as a result of the execution of the Collaboration Agreement with Ultragenyx in October 2020. These amounts were recorded as a research and development expense in the fourth quarter of 2020. The Company paid $750 in February 2021 and $1,300 in February 2022. The license agreement was also amended such that the Company is required to pay an aggregate of approximately $1,900 upon the achievement of certain milestones.

There were no milestones achieved during the years ended December 31, 2023, 2022, and 2021. The Company must pay a royalty of a low single digit percentage of future sales or by its sublicensees of products developed using the licensed patents. In addition, the Company must pay an annual maintenance fee until certain milestones are achieved, after which time a minimum annual royalty will replace such maintenance fee.

Under the agreement, the Company granted the University of Missouri a non-exclusive, royalty-free, irrevocable, paid-up license, with the right to grant sublicenses to non-profit, academic, educational or governmental institutions, to practice and use improvements made by the Company using the licensed patent rights, solely for non-commercial research purposes.

The license agreement remains in effect until the expiration of the last-to-expire patent or the abandonment of the last to be abandoned patent application licensed under the agreement. The University of Missouri may terminate the agreement, or render the license granted thereunder non-exclusive, in individual countries if the Company's sublicensees fail to achieve certain milestones. The Company may terminate the license agreement at any time upon providing six months' written notice to the University of Missouri and paying a termination fee. Each of the University of Missouri and the Company may also terminate the agreement for an uncured default or breach of the agreement by the other party. The Company's ability to cure such breach only applies to the first two notices of such breach provided by the University of Missouri, and thereafter, the University of Missouri may terminate the agreement for the Company's default or breach of the agreement upon thirty days' written notice without an opportunity to cure such default or breach.

The Company recorded research and development expense in the amount of $132, $133, and $195 for the years ended December 31, 2023, 2022, and 2021, respectively, under the agreement.

The University of Michigan License Agreement

In 2016, the Company entered into a license agreement with the Regents of the University of Michigan, (the "University of Michigan"), a constitutional corporation of Michigan, under which the Company obtained an exclusive, royalty-bearing, sublicensable, worldwide license to make, sell and distribute products under certain patents owned by the University of Michigan related to microdystrophin and utrophin spectrin-like nucleic acid sequences for any use that, but for this agreement, would comprise an infringement of a valid claim included in the licensed patent rights.

In consideration for the rights granted by the agreement, the Company paid a one-time license fee and a separate fee to cover past patent prosecution costs, which the Company recorded as a research and development expense in 2016. The Company was required to reimburse the University of Michigan for costs incurred in applying for, prosecuting and maintaining patents, and pay up to an aggregate of approximately $1,000 upon the achievement of certain milestones. There were no milestones achieved during the years ended December 31, 2023, 2022, and 2021. The Company was also required to pay a royalty of a low single digit percentage on future sales by the Company or its sublicensees of products developed using the licensed rights, with a minimum annual royalty after certain milestones are achieved. In addition, the Company was required to pay an annual maintenance fee in any year in which the minimum annual royalty is not reached.

Under the agreement, the University of Michigan reserved for itself and its affiliates the right to use the licensed rights for non-commercial research, public service, internal and educational purposes and the right to grant the same limited non-commercial rights to other non-profit research institutions.

The Company recorded research and development expense in the amount of $0, $0 and $37 for the years ended December 31, 2023, 2022, and 2021, respectively, under the agreement.

University of Florida License Agreements

In 2020, AavantiBio entered into license agreements with the University of Florida Research Foundation, Inc. (“UFRF”). Broadly, the agreements relate to FA. The Company acquired the agreements in connection with the Acquisition. In 2023, the Company entered into an additional license agreement with UFRF. Under each agreement the Company obtained an exclusive, royalty-bearing, sublicensable, world-wide license to certain patents and patent applications and a royalty-bearing non-exclusive license under the know-how, to make, have made, use, see, have sold, import and export licensed products. UFRF retains the right to practice the patent rights and know-how for internal non-commercial research, including research sponsored by commercial entities, and educational purposes.

In consideration for the rights granted under each agreement, AavantiBio paid a one-time non-refundable license fee. In connection with each agreement, the Company is required to pay an annual license maintenance fee until the first commercial sale of a licensed product after which time a minimum annual royalty will replace such maintenance fees. Under each agreement, the Company is required to reimburse UFRF for costs incurred in applying for, prosecuting and maintaining patents, pay up to an aggregate of approximately $2,900 upon the achievement of certain intellectual property, clinical and regulatory milestones for each licensed product under the agreement, and pay a low, single digit royalty on annual net sales by us and our sublicensees of licensed products on a licensed-product-by-licensed product basis. For any licensed product covered by both of these agreements, the Company is only obligated to make one payment for each milestone achieved and royalty payment due. Prior to the Acquisition, AavantiBio paid a single milestone fee related to the agreements of $50. Under each agreement, in the event the Company grants a sublicense to another party, the Company is required to pay UFRF a percentage of the consideration received.

Under each agreement, the Company has the right to grant sublicenses to third parties through multiple tiers, to the extent we are in compliance with our diligence obligations under the agreement and that sublicensee is subject to the terms of such agreement.

Under each agreement, the Company is obligated to use commercially reasonable efforts to develop and commercialize products covered by the licensed patent rights or know-how and to achieve certain regulatory and commercialization milestones within estimated time periods. Under the agreement entered in 2023, the Company agreed to pay to UFRF cumulative sales milestones of up to $8,500 upon achievement of specified milestone events and tiered royalties on worldwide net sales in the low-to-mid-single digits.

Under each agreement, UFRF controls the prosecution and maintenance of the licensed patents in consultation with the Company and at the Company's expense. In countries in which the Company has not requested prosecution or maintenance of licensed patents in a particular country or jurisdiction, the license granted to such patent rights will terminate in such country or jurisdiction. The Company has the first right to enforce such licensed patents at our expense.

Each of the agreements terminates on a licensed product-by-licensed product basis on the later of: (i) expiration of the patent rights covering such licensed product or (ii) ten (10) years from the first commercial sale of such licensed product. After five years, the Company may terminate an agreement for any reason giving advance written notice and reason for termination. UFRF may terminate an agreement for our uncured default or breach of the agreement. UFRF may immediately terminate an agreement if we bring or assist others in bringing a patent challenge against of the licensed patent rights. If UFRF sends the Company a written demand to terminate a sublicense agreement due to such sublicensee bringing or assisting a patent challenge, UFRF may terminate such agreement if we do not terminate the license with such sublicensee.

Maugeri License Agreement

On June 29, 2023, the Company entered into a license agreement (the "Maugeri License Agreement"), with ICS Maugeri S.p.A. SB ("Maugeri"), to focus on the development and commercialization of cardiac-related products by the Company based on Maugeri’s inventions. Pursuant to the Maugeri License Agreement, Maugeri granted us an exclusive worldwide sublicensable license in certain Maugeri patent rights, including existing patent rights, and those in any improvements or know-how made in performance of the Maugeri License Agreement, and a non-exclusive worldwide sublicensable license in certain Maugeri know-how, including existing know-how, and on any improvement thereto, in each case, subject to certain conditions, that is necessary or reasonably useful to develop the licensed products under the terms of the Maugeri License Agreement. The Company will conduct certain activities agreed to by the parties with respect to the research and development of licensed products. A condition precedent to the effectiveness of the Maugeri License Agreement was regulatory review in Italy, which was completed in the third quarter of 2023 and, upon the completion of the condition precedent, the Maugeri License Agreement became effective.

The Company paid Maugeri an upfront license fee of €1,500, which was recorded as research and development expense during the second quarter of 2023. Additionally, the Company agreed to cumulative developmental, regulatory, and commercial milestone payments of up to €15,000, cumulative sales milestone payments of up to €15,000, upon achievement of specified milestone events, and tiered royalties on worldwide net sales in the low-to-mid-single-digits.

The Maugeri License Agreement continues until the latest expiry of (i) the last valid claim (as defined in the Maugeri License Agreement), (ii) regulatory exclusivity, and (iii) all payment obligations. Either party may terminate the Maugeri License Agreement for the other party's uncured material breach. The Company may also terminate the Maugeri License Agreement in its sole discretion upon 60 days' prior written notice to Maugeri and payment of a fee.

Other Agreements

The Company has committed to make potential future milestone payments and pay legal fees to third parties as part of licensing and development programs. The agreements generally required an upfront license fee and, under each agreement, the Company may be required to pay annual maintenance fees, royalties, milestone payments and sublicensing fees. Each license agreement is generally cancelable by the Company, given appropriate prior written notice. At December 31, 2023, potential future milestone payments under these agreements totaled an aggregate of $12,000. None of these milestones were assessed to be probable as of December 31, 2023.

14. Net Loss per Share

Basic and diluted net loss per share were calculated as follows:

The numerator for basic and diluted net loss per share is as follows:

	For the Year Ended December 31,		
	2023	2022	2021
Net loss	$ (96,015)	$ (85,981)	$ (72,188)

The denominator is as follows:

	For the Year Ended December 31,		
	2023	2022	2021
Weighted average common stock outstanding, basic and diluted	19,884,007	8,512,089	6,974,136
Weighted average pre-funded warrants to purchase common stock	—	—	143,888
Total	19,884,007	8,512,089	7,118,024

Net loss per share, basic and diluted is as follows:

	For the Year Ended December 31,		
	2023	2022	2021
Net loss per share, basic and diluted	$ (4.83)	$ (10.10)	$ (10.14)

The following potential common stock equivalents, presented based on amounts outstanding at each period end, were excluded from the calculation of diluted net loss per share for the periods indicated because including them would have had an anti-dilutive effect:

	For the Year Ended December 31,		
	2023	2022	2021
Options to purchase shares of common stock	2,259,672	1,433,968	400,842
Unvested restricted stock units	877,181	512,557	33,979
	3,136,853	1,946,525	434,821

15. Income Taxes

The Company recorded no tax benefit for the years ended December 31, 2023 and 2022 for the net operating losses incurred due to its uncertainty of realizing a benefit from those items.

A reconciliation of income taxes computed using the U.S. federal statutory rate to that reflected in operations as of December 31, 2023 and 2022 is as follows:

	December 31, 2023	December 31, 2022
Income tax computed at federal statutory tax rate	21.0%	21.0%
State taxes, net of federal benefit	6.6%	7.4%
Permanent differences	(0.5)%	(1.4)%
Bargain purchase gain	0.0%	4.5%
Tax credits	7.0%	11.5%
Change in deferred tax rate	0.2%	0.1%
Stock compensation cancelations	(1.0)%	(1.9)%
Impact of ownership change	(108.5)%	0.0%
Other items	0.4%	(0.6)%
Valuation allowance	74.8%	(40.6)%
	0.0%	0.0%

The Company established deferred tax assets and liabilities on identified book to tax temporary differences as of the date of conversion to a C-corporation. Deferred income taxes reflect the net tax effects of these temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Significant components of the Company's net deferred tax assets as of December 31, 2023 and 2022 are as follows:

	December 31, 2023	December 31, 2022
Deferred tax assets:		
Tax loss carryforwards	$ 16,622	$ 70,965
Tax credit carryforwards	7,537	46,786
Deferred expenses	6,755	7,174
Accrued expenses	1,506	1,901
Stock compensation	8,496	7,839
Intangible assets	57,336	36,553
Depreciation	672	116
Other	80	166
Total deferred tax assets	99,004	171,500
Valuation allowance	(91,705)	(163,566)
Deferred tax liabilities:		
Right-of-use asset	(7,299)	(7,934)
Depreciation	—	—
Total deferred tax liabilities	(7,299)	(7,934)
Net deferred taxes	$ —	$ —

As of December 31, 2023, the Company has federal net operating loss carryforwards of $59,392 which may be available to offset future taxable income and do not expire but are limited in their usage to an annual deduction equal to 80% of annual taxable income. In addition, as of December 31, 2023, the Company has state net operating loss carryforwards of approximately $66,402 which may be available to offset future taxable income, of which $64,032 begins to expire in 2032 and $2,371 has unlimited carryforward. The Company also had federal and state tax credits of $6,599 and $1,187, respectively, which may be used to offset future tax liability and each of which begin to expire in 2042.

The Company's ability to utilize these federal and state carryforwards may be limited in the future if the Company experiences an ownership change pursuant to Internal Revenue Code Section 382. Ownership changes, as defined in the Internal Revenue Code, including those resulting from the issuance of common stock in connection with the Company's public offerings, may limit the amount of net operating loss and tax credit carryforwards that can be utilized to offset future taxable income or tax liability. The Company completed a study to assess whether a change of control has occurred under Section 382, and it was determined that all net operating loss carryforwards and credits generated before December 2, 2022 are limited. As a result, the carryfowards before the ownership change date of December 2, 2022 are not available for utilization and have been written off. The carryfowards as of December 31, 2023 were generated after the ownership change of December 2, 2022.

A valuation allowance is recorded against deferred tax assets if it is more likely than not that some or all of the deferred tax assets will not be realized. The Company has evaluated the positive and negative evidence bearing upon the realizability of the deferred tax assets. The Company concluded, in accordance with the applicable accounting standards, that it is more

likely than not that the Company will be unable to realize the benefit of its deferred tax assets. Accordingly, the Company has recorded a full valuation allowance against its deferred tax assets.

The following table presents the changes in the balance of the Company's deferred income tax asset valuation allowance:

	December 31, 2023	December 31, 2022
Valuation allowance at beginning of year	$ 163,566	$ 128,570
(Decreases) increases recorded to income tax provision	(71,861)	34,996
Valuation allowance at end of year	$ 91,705	$ 163,566

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending income tax examinations. The Company's C-Corporation tax years beginning with the year ended December 31, 2019 are open under statute. Any tax credit or net operating loss carryforward can be adjusted in future periods after the respective year of generation's statute of limitation has closed.

As of December 31, 2023 and 2022, the Company did not have unrecognized tax benefits. The Company recognizes interest and penalties related to income taxes as a component of income tax expense. As of December 31, 2023 and 2022, no interest and penalties have been recorded.

16. Defined Contribution Plan

The Company has a defined contribution savings plan under Section 401(k) of the Internal Revenue Code. The plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pretax basis. Company contributions to the plan may be made at the discretion of the Company's Board of Directors. The Company made $626, $527, and $241 of contributions during the years ended December 31, 2023, 2022, and 2021, respectively.

17. Restructuring

April 2022 Plan

In April 2022, the Company implemented changes to its corporate strategy to prioritize the advancement of its then-key programs, SGT-001 and SGT-003. In connection with the changes to corporate operations, the Company reduced headcount by approximately 35 percent. During the year ended December 31, 2022, the Company recorded and paid aggregate restructuring charges of $1,520 related to severance and other employee related costs in connection with the changes to its corporate strategy. The Company does not expect to incur any additional significant costs associated with this restructuring.

November 2022 Plan

In November 2022, the Company's Board of Directors approved a plan to reduce the Company's workforce by approximately 18 percent. These reductions were completed by December 5, 2022. This plan was designed to streamline the Company's operating structure following the Acquisition. The Company recorded a restructuring charge in the fourth quarter of 2022 of $5,658 related to the reduction in force, consisting of severance and other employee termination benefits. The Company paid $3,669 of this amount during the year ended December 31, 2023. The Company expects that approximately the remaining $252 will be paid by the first quarter of 2024.

The following table shows the total amount incurred and the liability related to the associated restructuring plans for the years ended December 31, 2023, 2022 and 2021:

One-Time Employee Termination Benefits	April 2022	November 2022
Accrued restructuring charges as of December 31, 2021	$ —	$ —
Accrual recorded as a result of restructuring charges	1,520	5,658
Amounts paid during the period	(1,520)	(1,737)
Accrued restructuring charges as of December 31, 2022	$ —	$ 3,921
Accrual recorded as a result of restructuring charges	—	—
Amounts paid during the period	—	(3,669)
Accrued restructuring charges as of December 31, 2023	$ —	$ 252

18. Subsequent Events

On January 11, 2024, the Company issued and sold 16,973,103 shares of the Company's common stock at a price per share of $5.53 and, to one investor in lieu of shares of common stock, pre-funded warrants to purchase 2,712,478 shares of common stock at a price of $5.529 per pre-funded warrant, in the January 2024 Private Placement. The Company received approximately $104,034 of net proceeds from the January 2024 Private Placement after deducting offering costs.

Exhibit 10.7

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

EXCLUSIVE PATENT LICENSE AGREEMENT

This exclusive patent license agreement ("Agreement") is dated and effective as of the date of last signature (the "Effective Date"), and is made between the University of Washington, a public institution of higher education and an agency of the state of Washington, acting through UW CoMotion ("University"), and Solid GT, LLC, a limited liability company under the laws of the state of Delaware ("Company"), (individually "Party" or collectively "Parties").

Background

Certain inventions related to Novel micro-dystrophins under muscle-specific promoters for the treatment of Duchenne Muscular Dystrophy were made in the laboratory of Dr. Jeffrey Chamberlain ("Principal Investigator");

As assignee of the inventions, University owns the patents and patent applications as listed in Section A1 "Licensed Patents" of Exhibit A "Exclusive Patent License Schedule" and University has the right to license to others certain rights to such patents and patent applications;

Whereas University and Company entered into a confidentiality agreement with the effective date of June 16th, 2014 with University reference number 35111A;

Whereas University and Company entered into an exclusive option agreement for Licensed Patents with the effective date of February 5, 2015 with University reference number 36201A;

Company desires that University grant it an exclusive license to use, develop, and commercialize the inventions claimed in the Licensed Patents; and

University is willing to grant a license on the terms set forth below.

The Parties therefore agree as follows:

1. Definitions.

For purposes of interpreting this Agreement, the following terms have the following meanings ascribed to them:

1.1."Assignment" means (A) the sale by Company of all but no less than all of its assets to an arm's length Third Party, (B) the sale, transfer, or exchange by the shareholders, partners, or equity owners of Company of a majority interest in Company to an arm's length Third Party, or (C) the merger of Company into an arm's length Third Party.

1.2.“Assignment Consideration” means all consideration received by Company for an Assignment.

1.3.“Confidential Information” means any information or materials (biological, chemical, or otherwise) of the Parties not generally known to the public, including any information comprised of those materials, and including without limitation the inventions covered by the Licensed Patents and Company’s business plans or reports. Confidential Information does not include any information that:

1.3.1. is or becomes part of the public domain through no fault of receiving Party;

1.3.2. is known to receiving Party prior to the disclosure by the disclosing Party, as evidenced by documentation;

1.3.3. is publicly released as authorized under this Agreement by University, its employees or agents;

1.3.4. is subsequently obtained by a Party from a Third Party who is authorized to have such information; or

1.3.5. is independently developed by a Party without reliance on any portion of the Confidential Information received from the disclosing Party and without any breach of this Agreement as evidenced by documentation.

1.4.“Event of Force Majeure” means an unforeseeable act that wholly prevents a Party from performing one or more of its material duties under this Agreement and that is outside of the reasonable control of the Party. An Event of Force Majeure includes acts of war or of Nature, insurrection and riot, and labor strikes. An Event of Force Majeure does not mean a Party’s inability to obtain a Third Party’s consent to any act or omission.

1.5.“Field of Use” means Treatment of Duchenne Muscular Dystrophy and related disease indications caused by a lack of functional dystrophin.

1.6.“Licensed Patents” means the patents and patent applications (including all provisional, nonprovisional, and PCT patent applications, and all national stage and foreign equivalents of the foregoing, accordingly) listed in Section A1 “Licensed Patents” of attached Exhibit A “Exclusive Patent License Schedule”, all divisionals and continuations of these patent applications, all patents issuing from these applications, divisionals, and continuations and any reissues, reexaminations, supplementary protection certificates and extensions of these patents, and any corresponding foreign applications or patents thereof. Claims in continuations-in-part applications are included in Licensed Patents only to the extent such claims are supported by a patent or patent application set forth in Section A1 “Licensed Patents” of Exhibit A “Exclusive Patent License Schedule” to benefit from the priority date of such patent or patent application and to the extent such claims are not encumbered by Third Party rights.

1.7.“Licensed Product” means any product or good that is used, made by, made for, sold, transferred, offered for sale, imported or otherwise disposed of during the term of this

Agreement and for which use, manufacture, sale, transfer is covered by one or more Valid Claims of the Licensed Patents.

1.8.“Net Sales” means the gross amount invoiced or otherwise received by Company or Sublicensee for sales, leases, and other dispositions of Licensed Products less (i) all trade, quantity, and cash discounts actually allowed, (ii) all credits and allowances actually granted due to rejections, returns, billing errors, and retroactive price reductions, (iii) duties, and (iv) excise, sale and use taxes, and equivalent taxes to the extent not reimbursable. On sales of Licensed Products by Company to Sublicensees or on sales made in other than an arm’s length transactions, the value of the Net Sales attributed to such transaction shall be that which would have been received in an arm’s length transaction, based on sales of like quantity and quality products on or about the time of this transaction.

1.9.“Patent Expenses” means all reasonable costs (including attorneys’ and application fees) incurred by University to apply for, prosecute, enforce, and maintain Licensed Patents including the costs of interferences, oppositions, re-examinations, and patent litigation. For clarity, patent litigation may result in a positive cash position from damages and therefore is subject to distribution rights of the Parties of Article 7.

1.10.“Sublicense” means the grant by Company to a Third Party of any license, option, first right to negotiate, or other right granted under the Licensed Patents, in whole or in part. For the avoidance of doubt, any arm’s length Third Party distributor (“Distributor”) to which Company or any of its Sublicensees sells a Licensed Product for resale of Licensed Product by the Distributor, and where Distributor has no other rights other than to resell Licensed Product, and for which resale Company and Sublicensees receive no further consideration (including but not limited to royalties and/or commissions) beyond the price for the initial sale to the Distributor shall not be a considered a Sublicense.

1.11.“Sublicensee” means a Third Party holding a Sublicense under the Licensed Patents.

1.12.“Sublicensing Consideration” means all consideration, including but not limited to upfront fees, milestone payments, maintenance fees, non cash consideration, and premiums over Fair Market Value of stock, but excluding royalties, payable by each Sublicensee and attributable to the grant of a Sublicense. For avoidance of doubt, the following are not deemed to be Sublicensing Consideration: (A) consideration paid to Company by Sublicensees for the performance of bona fide product development work, research work, clinical studies and regulatory approvals performed by Company, pursuant to and as supported by an express agreement including a performance plan and commensurate budget; (B) payments made as consideration for the issuance of equity or debt securities of Company at fair market value; and (C) contractually required reimbursement of payment amounts otherwise due under this Agreement from Company to University for Patent Expenses pursuant to Section A4 (Patent Expense Payment); and (D) to the extent a milestone under Section A3.5 (Financial Milestones) of this Agreement is met by the Sublicensee, any pass-through payment to Company that ultimately comes to University for such milestone payment.

1.13.“Territory” means worldwide.

1.14.“Third Party” means an individual or entity other than University and Company.

1.15.“Valid Claim” means (i) a claim in an issued and unexpired patent included in the Licensed Patents that: (a) has not been held unenforceable, unpatentable or invalid by a decision of a court or other governmental agency of competent jurisdiction, and not subject to appeal, (b) has not been admitted to be invalid or unenforceable through reissue or disclaimer or otherwise, (c) has not been lost through an interference, reexamination, or reissue proceeding; or (ii) a claim of a pending patent application included in the Licensed Patents that has not been abandoned or finally rejected without the possibility of appeal or refiling and that has been pending for less than five (5) years from its priority date.

2. Term.

The term of this Agreement will commence on the Effective Date and, unless terminated earlier as provided in Article 9 “Termination”, will expire on the date on which no Valid Claim in a Licensed Patent is pending or subsisting in any country in the Territory.

3. Grant of License.

3.1.Company’s Rights.

3.1.1. License Grant. Subject to the terms and conditions of this Agreement, University hereby grants to Company, and Company hereby accepts, an exclusive license to make, have made on Company’s behalf, use, offer to sell or sell, offer to lease or lease, import, or otherwise offer to dispose or dispose of Licensed Products in the Territory in the Field of Use. The license granted in this Agreement is limited to the inventions that are expressly claimed in the Licensed Patents. No provision of this Agreement grants Company, by implication, estoppel or otherwise, any rights other than the rights expressly granted it in this Agreement to the Licensed Patents, or to any other University-owned technology, patent applications, or patents.

3.1.2. Sublicenses. Company has the right, exercisable from time to time during the term of this Agreement, to Sublicense its rights under this Agreement; Company may grant Sublicensees the right to grant sublicenses but not the right to enforce Licensed Patents. Company shall remain responsible for its obligations under this Agreement, and shall ensure that the Sublicense agreement: i) contains terms and conditions requesting Sublicensee to comply with the applicable terms and conditions under this Agreement (including a release substantially similar to that provided by Company in Section 10.1 “Company’s Release”; a warranty substantially similar to that provided by Company in Section 11.1 “Authority”; University disclaimers and exclusions of warranties under Subsections 11.2 “Disclaimers”; and limitations of remedies and damages substantially similar to those provided by Company in Sections 12.1 “Remedy Limitation” and 12.2 “Damage Cap”); and (ii) specifically incorporates provisions of this Agreement regarding obligations pertaining to indemnification, use of names and insurance. Company shall deliver to University a true, correct, and complete copy of any Sublicense agreement or other agreement under which Company purports or intends to grant Sublicense rights at least 20 business days prior to the execution of the agreement, along with a request for review within 20 days pursuant to this Subsection 3.1.2 “Sublicenses”. University will review the unexecuted Sublicense and will, within 20 business days of receipt of the proposed

Sublicense, either provide express written approval for the Sublicense as presented or decline consent for the transaction. Such approval will not be unreasonably withheld. If approval is granted, Company will provide University copies of the Sublicense agreement within 30 days of its execution. Company shall not enter into such agreement if the terms of the agreement are inconsistent in any respect with the material terms of this Agreement. Any Sublicense made in violation of this Subsection will be void and will constitute an event of default under Subsection 9.1.1 "Breach by Company".

3.2. The United States Government's Rights. The inventions covered in the Licensed Patents arose, in whole or in part, from federally supported research and the federal government of the United States of America has certain rights in and to the Licensed Patents as those rights are described in Chapter 18, Title 35 of the United States Code and accompanying regulations, including Part 401, Chapter 37 of the Code of Federal Regulation. The Parties' rights and obligations under this Agreement to any government-funded inventions, including the grant of license set forth in Subsection 3.1.1, are subject to the applicable terms of the aforementioned United States laws.

3.3. University's Reservation of Rights. University reserves all rights not expressly granted to Company under this Agreement. University retains for itself an irrevocable, nonexclusive license to make, have made, and use products, processes, and other subject matter covered by the Licensed Patents in the Field of Use for academic research, medical, instructional, or any other academic purpose. Expressly included within this University reservation of rights is the right (i) to use the Licensed Patents in sponsored research or collaborative research with any Third Party but only to the extent no such Third Party is granted any rights to the Licensed Patents or to commercialize Licensed Products, (ii) to grant material transfer agreements to materials whose composition of matter is covered by the Licensed Patents where the use of such materials is restricted to academic research, medical, instructional, or any other academic purpose, and (iii) to publish any information included in the Licensed Patents or any other information that may result from University's research.

3.4. Reservation of Rights for Humanitarian Purposes. Consistent with 35 U.S.C. §200 et seq., University retains the right to require Company to grant Sublicenses to responsible applicants in the Field of Use under the Licensed Patents on terms that are reasonable under the circumstances; or, if Company fails to grant a license, to grant the license itself. The exercise of these rights by University will only be in exceptional circumstances and only if University determines (i) the action is necessary to meet health or safety needs that are not reasonably satisfied by Company; or (ii) the action is necessary to meet requirements for public use specified by federal regulations, and such requirements are not reasonably satisfied by Company. University shall not require the granting of a sublicense, and shall not grant the license itself, unless the responsible applicant has first negotiated in good faith with Company.

4. Applications and Patents.

4.1. Pre-Agreement Patent Filings and Licensed Product Sales. Company has reviewed the Licensed Patents and represents that it is not aware of any basis to challenge or dispute the inventorship, validity, or enforceability of any of the claims made in the Licensed Patents in existence as of the Effective Date. Company further represents that, as of the

Effective Date, it has not and does not manufacture, have manufactured, use, offer to sell or sell, offer to lease or lease, import, or otherwise offer to dispose or dispose of (a) any product or good that infringes (including under the doctrine of equivalents) a claim in any Licensed Patent, or (b) any product or good that is made using a process or machine that infringes (including under the doctrine of equivalents) a claim in a Licensed Patent.

4.2.Patent Application Filings during the Term of this Agreement.

4.2.1. University Prosecutes Patents. University retains the sole and exclusive right to file or otherwise prosecute Licensed Patents, in consultation with the Company pursuant to Section 4.2.2. As set out in Section A4 "Patent Cost Reimbursement" of Exhibit A "Exclusive Patent License Schedule", Company shall pay, or reimburse University for paying, all Patent Expenses incurred prior to, on, or after the Effective Date.

4.2.2. Patent Prosecution Decisions. University, in consultation with Company, shall determine in which countries University will file, or cause to be filed, Licensed Patents. University shall request patent counsel to inform Company of the status of the prosecution of the Licensed Patents, including delivering to Company written and electronic communications from all patent offices and foreign counsel, and University shall consult with the Company on the prosecution of the Licensed Patents. Once Company begins reimbursing University for Patent Expenses pursuant to Section A4 "Patent Cost Reimbursement" of Exhibit A "Exclusive Patent License Schedule", Company's suggestions and requests regarding patent prosecution will be reasonably considered and included unless detrimental to University's intellectual property rights. In no event shall Company file a patent application where all of the inventors are under University policy obligated to assign their rights in such patent application to University. In no event shall Company file a patent application where one or more, but not all, of the inventors are under University policy obligated to assign their rights in such patent application to University without University's prior consent which shall not be unreasonably withheld or delayed.

4.2.3. University's Independent Patent Filings. At its sole expense, University may file, prosecute or maintain Licensed Patents in any country in which Company has not requested University to file, prosecute or maintain such Licensed Patents in accordance with this Article 4 "Applications and Patents" and those applications and resultant patents will not be subject to this Agreement.

4.2.4. No Limitation on University's Right to Prosecute Patents. No provision of this Agreement limits, conditions, or otherwise affects University's right to prosecute Licensed Patents in any country, except as expressly provided herein.

4.3.Maintenance of Licensed Patents. Subject to Company's compliance with Section A4 "Patent Cost Reimbursement" of attached Exhibit A "Exclusive Patent License Schedule", University shall take all commercially reasonable steps to cause each Licensed Patent to remain or be valid and subsisting.

4.4.Ownership of the Licensed Patents. No provision of this Agreement grants Company any rights, titles, or interests (except for the grant of license in Subsection 3.1.1

"License Grant" of this Agreement) in the Licensed Patents, notwithstanding Company's payment of all or any portion of the patent prosecution, maintenance, and related costs.

5. Commercialization.

5.1. Commercialization and Performance Milestones. Company shall use its commercially reasonable efforts, consistent with sound and reasonable business practices and judgment, to commercialize the inventions covered by the Licensed Patents and to make and sell Licensed Products as soon as practicable and to maximize sales thereof. Unless an extension is provided due to the occurrence of an Event of Force Majeure during the term of this Agreement, Company shall perform, or shall cause to happen or be performed, the performance milestones described in Section A2 "Performance Milestones" of attached Exhibit A "Exclusive Patent License Schedule".

5.2. Covenants Regarding the Manufacture of Licensed Products. Company hereby covenants and agrees that the manufacture, use, sale, or transfer of Licensed Products will comply with all applicable federal and state laws, including all federal export laws and regulations. Company hereby further covenants and agrees that, to the extent required by 35 United States Code Section 204, it shall, and it shall cause each Sublicensee, to substantially manufacture in the United States of America all products embodying or produced through the use of an invention that is subject to the rights of the federal government of the United States of America.

5.3. Commercialization Reports. Within 30 days of the anniversary of the Effective Date of each year during the term of this Agreement, Company shall deliver to University written reports of Company's and Sublicensees' efforts and plans to commercialize the inventions covered by the Licensed Patents and to make, have made on its behalf, use, offer to sell or sell, offer to lease or lease, import, or otherwise offer to dispose or dispose of Licensed Products. Company shall not be obligated to prepare such commercialization reports in years Company or Sublicensee delivers to University a written sales report under Section 6.4 "Sales Reports" and will resume if sales of Licensed Products ceases. In relation to each of the performance milestones described in Section A2 "Performance Milestones" of attached Exhibit A "Exclusive Patent License Schedule", each commercialization report will include sufficient information to demonstrate compliance of those performance milestones and will set out timeframes and plans for those which have not yet been met.

5.4. Use of University's Name and Trademarks or the Names of University Faculty, Staff, or Students. No provision of this Agreement grants Company or Sublicensee any right or license to use the name or trademarks of University or the names or identities of any member of the faculty, staff, or student body of University. Company shall not use, and shall not permit a Sublicensee to use, any such trademarks, names, or identities without University's and, as the case may be, such member's prior written approval.

6. Payments, Reimbursements, Reports, and Records.

6.1. Payments. Company shall deliver to University the payments specified in Sections A3 "Payments" and A4 "Patent Cost Reimbursement" of attached Exhibit A "Exclusive

Patent License Schedule". Company shall make such payments by check, wire transfer, or any other mutually agreed-upon and generally accepted method of payment. All checks to University will be made payable to "University of Washington" and will be mailed to the address specified in Article 21 "Notices" of this Agreement and will include the University agreement number 37475A. Upon request, University shall deliver to Company written wire transfer instructions.

6.2.Currency and Checks. All computations and payments made under this Agreement will be in United States dollars. The exchange rate for the currency into dollars as reported in the *Wall Street Journal* as the New York foreign exchange mid-range rate on the last business day of the month in which the transaction was entered into will be used for determining the dollar value of transactions conducted in non-United States dollar currencies.

6.3.Late Payments. University may charge Company a late fee for all amounts owed to University that are overdue by 30 days or more. The late fee will be computed as the United States prime rate plus [**], as set forth by *The Wall Street Journal* (Western edition) of the outstanding, unpaid balance. The payment of a late fee will not foreclose or limit University from exercising any other rights it may have as a consequence of the lateness of any payment.

6.4.Sales Reports. Within 30 days after the last day of each calendar quarter commencing the calendar quarter after Company effects its first commercial sale of a Licensed Product and during the term of this Agreement, Company shall deliver to University a written sales report (a copy of the form of which is attached as Exhibit B "Royalty Report Form") recounting the number and Net Sales (expressed in U. S. dollars) of all sales, leases, or other dispositions of Licensed Products, whether made by Company or a Sublicensee, during such calendar quarter. Included in each sales report will be the name of each Distributor, and the number and type of Licensed Product sold, leased, or otherwise provided to such Distributor. Company shall deliver such written report to University even if Company is not required hereunder to pay to University a payment for sales, leases, or other dispositions of Licensed Products during the calendar quarter.

6.5.Records Retention and Audit Rights.

6.5.1. Records Retained. Throughout the term of this Agreement and for five (5) years thereafter, Company, at its expense, shall keep and maintain and shall cause each Sublicensee to keep and maintain complete and accurate records of all sales, leases, and other dispositions of Licensed Products during the term of this Agreement and all other records related to this Agreement.

6.5.2. Auditing Rights. Company shall permit, at the request of University, one or more accountants selected exclusively by the University ("Accountants") to have access to Company's records and books of account pertaining to this Agreement. Accountants' access will be during ordinary working hours to audit Company's records for any payment period ending prior to such request, the correctness of any report or payment made under this Agreement, or to obtain information as to the payments due for any period in the case of failure of Company to report or make payment pursuant to the terms of this Agreement or to verify Company's compliance with its payment obligations hereunder. Company shall cause each Sublicensee that

manufactures, sells, leases, or otherwise disposes of Licensed Products on behalf of Company to grant University the right to inspect and audit Sublicensee's records.

6.5.3. Scope of Disclosure. Accountants shall not disclose to University any information relating to the business of Company except that which is necessary to inform University of: the accuracy or inaccuracy of Company's reports and payments; compliance or noncompliance by Company with the terms and conditions of this Agreement; and the extent of any inaccuracy or noncompliance.

6.5.4. Accountant Copies. If Accountants believe there is an inaccuracy in any of Company's payments or noncompliance by Company with any terms and conditions, Accountants shall have the right to make and retain copies (including photocopies) of any pertinent portions of the records and books of account.

6.5.5. Costs of Audit. If Company's royalties calculated for any calendar year quarterly period are under-reported by more than 5%, the costs of any audit and review initiated by University will be borne by Company; otherwise, University shall bear the costs of any audit initiated by University.

7. Infringement.

7.1.Third-Party Infringement of a Licensed Patent.

7.1.1. Notice of Third Party's Infringement. If a Party learns of substantial, credible evidence that a Third Party is infringing a Licensed Patent in the Field of Use in the Territory, that Party will promptly deliver written notice of the possible infringement to the other Party, describing in detail all relevant information to which that Party has access or control suggesting infringement of the Licensed Patent.

7.1.2. Company's First Right to Settle. During the term of this Agreement, Company has the first right to respond to, defend, and prosecute in its own name and at its own expense actions or suits relating to Licensed Patents. To enjoy said first right, Company must initiate bona fide action to respond to any alleged infringement within 90 days of learning of said infringement. If required by law, University agrees to be joined as a party plaintiff; provided that Company must notify University at least ten (10) days before filing suit and provided that Company shall reimburse University for all reasonable legal fees and costs incident thereto. Company shall not settle any suits or actions in any manner relating to the Licensed Patents without obtaining the prior written consent of University.

7.1.2.1Distribution of Proceeds from Settlement. Out of any proceeds from any settlement for infringement of Licensed Patents, Company is allowed to first recover its reasonable attorney's fees and other out-of-pocket expenses directly related to any action, suit, or settlement for infringement of Licensed Patents. Any remaining proceeds will be distributed as follows: Company shall retain [**] and shall distribute [**] to University. Any payment by an alleged infringer that constitutes consideration for Net Sales of infringing product, however, will be handled according to the payment provisions of Article 6 "Payments, Reimbursements, Reports, and Records" and Section A3.3 "Running Royalty Payments" of Exhibit A "Exclusive Patent License Schedule". Any payment by an alleged infringer that constitutes consideration

for the grant of a Sublicense will be handled according to Section A3.7 "Sublicensing Consideration" of Exhibit A "Exclusive Patent License Schedule".

7.1.2.2Limitation on Infringement Actions. Excluded from the rights granted herein is the right to bring an infringement action against any inventor or their present or future not-for-profit employers, for infringement of the License Patents in carrying out not-for-profit research.

7.1.3. University Right to Institute Action. If Company fails, within 90 days of learning of an alleged infringement, to secure cessation of the infringement, institute suit against the infringer, or to provide to University satisfactory evidence that Company is engaged in bona fide negotiations for the acceptance by infringer of a Sublicense in and to relevant patents in Licensed Patents for the Field of Use, then University may, upon written notice to Company, assume full right and responsibility to secure cessation of the infringement, institute suit against the infringer, or secure acceptance of a Sublicense by Company from the alleged infringer in and to relevant patents in Licensed Patents. Such license shall not be subject to Company's approval. If University, in accordance with the terms and conditions of this Agreement, chooses to institute suit against an alleged infringer, University may bring such suit in its own name (or, if required by law, in its and Company's name) and at its own expense, and Company shall, but at University's expense for Company's direct associated expenses, fully and promptly cooperate and assist University in connection with any such suit. All license fees, royalties, damages, awards, or settlement proceeds arising from such a University-initiated action will be solely for the account of University.

7.1.4. No Obligation to Institute Action. Neither Company nor University is obligated under this Agreement to institute or prosecute a suit against any alleged infringer of Licensed Patents.

8. Patent Validity.

8.1.Notice and Investigation of Third Party Challenges. If any Third Party challenges the validity or enforceability of any of the Licensed Patents, the Party having such information shall immediately notify the other Party.

8.2.Tender to University of Third Party Actions. In the event of Third Party legal action challenging the validity or enforceability of any of the Licensed Patents, University, at its sole discretion, shall have the right to assume and control the sole defense of the claim at University's expense. If University opts not to assume and control the sole defense of the claim within 30 days after becoming aware of challenge, Company shall have the right to assume the defense of the claim at its own expense. Company shall not settle any suits or actions in any manner relating to the Licensed Patents without obtaining the prior written consent of University.

8.3.Enforceability of Licensed Patents. Notwithstanding challenge by any Third Party, any Licensed Patent will be enforceable under this Agreement until such Licensed Patent is determined to be invalid.

9. Termination.

9.1. By University.

9.1.1. Breach by Company. If Company breaches or fails to perform one or more of its material duties under this Agreement, University may deliver to Company a written notice of default. University may terminate this Agreement by delivering to Company a written notice of termination if the default has not cured in full within 60 days of the delivery to Company of the notice of default.

9.1.2. Events of Default. University may terminate this Agreement by delivering to Company a written notice of termination at least ten (10) days prior to the date of termination if Company (i) becomes insolvent; (ii) voluntarily files or has filed against it a petition under applicable bankruptcy or insolvency laws that Company fails to have released within 30 days after filing; (iii) proposes any dissolution, composition, or financial reorganization with creditors or if a receiver, trustee, custodian, or similar agent is appointed; (iv) makes a general assignment for the benefit of creditors; or (v) if Company challenges the validity of the Licensed Patents.

9.2. By Company. Company may terminate this Agreement at any time by delivering to University a written notice of termination at least 60 days prior to the effective date of termination.

9.3. Effect of Termination.

9.3.1. License Terminated. After termination of this Agreement, Company shall not make, have made, use, offer to sell or sell, offer to lease or lease, import, or otherwise offer to dispose or dispose of Licensed Products.

9.3.2. Final Report to University. Within 60 days after the end of the calendar quarter following the expiration or termination of this Agreement, Company shall submit a final report to University. Any payments, including those incurred but not yet paid (such as the pro-rata minimum annual royalty, and those related to patent expense incurred as of the date of termination but not yet paid), due to University shall become immediately due and payable upon termination or expiration.

9.3.3. Termination of Sublicenses. Upon termination of this Agreement for any reason prior to expiration, each Sublicense will terminate and Company will include a statement to that effect in each Sublicense. Company shall be liable for any costs, expenses, or damages payable to any Sublicensee arising out of the termination of a Sublicense. At any time within 30 days following termination of this Agreement, a Sublicensee may notify University that it wishes to enter into a direct license with University in order to retain its rights to the Licensed Patents granted to it under its Sublicense (such 30-day period following termination, the "Initial Notice Period"). Following receipt of such notice, University and Sublicensee shall enter into a license agreement the terms of which shall be substantially similar to the terms of this Agreement; and the scope of such direct license, the licensed territory or the duration of the license grant shall be comparable to the corresponding terms granted by the Company to such Sublicensee; provided that such Sublicensee will be granted at least the same scope of rights as it obtained from Company under its Sublicense. For the sake of clarity, the financial terms, including without limitation, the running royalty rate and milestone payments, shall be identical to the

corresponding financial terms set forth in this Agreement. Notwithstanding the foregoing, each Sublicensee's right to enter into such direct license shall be conditioned upon:

9.3.3.1 Written Notification to University. Such Sublicensee informing University in writing, pursuant to Article 21 "Notices", that it wishes to enter into such direct license with University, within the Initial Notice Period;

9.3.3.2 Sublicensee Good Standing. Such Sublicensee being in good standing with Company under its Sublicense, and such Sublicense not being the subject of a dispute between Sublicensee and Company, or between Company and University under this Agreement;

9.3.3.3 Valid Sublicense. Such Sublicense having been validly entered into by Company and Sublicensee pursuant to the terms of Section 3.1.2 "Sublicenses";

9.3.3.4 Sublicensee Certification that Conditions Satisfied. Such Sublicensee using reasonable efforts to certify or otherwise demonstrate that the conditions set forth in subsections 9.3.3.1 "Written Notification to University", 9.3.3.2 "Sublicensee Good Standing", and 9.3.3.3 "Valid Sublicense" have been met within 30 days of expiration of the Initial Notice Period (or within such longer period of time as University agrees is reasonable under the circumstances, based on the nature and extent of any documentation reasonably requested by University); and

9.3.3.5 Time Limitations. Such negotiations for a direct license not exceeding 90 days from the end of the 30-day (or longer, if applicable) period described in subsection 9.3.3.4 "Sublicensee Certification that Conditions Satisfied" (subject to extension of said 90-day period by mutual written agreement of University and Sublicensee).

University may, at its sole discretion, waive any of these requirements. If all of the conditions set forth in this Section 9.3.3 "Sublicenses" are met, then Sublicensee will be granted such direct license by University. If any condition set forth in this Section 9.3.3 "Sublicenses" is not met, then after expiration of any time period granted to Sublicensee with respect to meeting such condition (for example and to the extent applicable, the Initial Notice Period and/or the periods described in subsections 9.3.3.4 "Sublicensee Certification that Conditions Satisfied" and 9.3.3.5 "Time Limitations"), Sublicensee shall not make, have made, use, offer to sell or sell, offer to lease or lease, import, or otherwise offer to dispose or dispose of Licensed Products and University shall be free to license or not license Licensed Patents to such Sublicensee according to its sole discretion.

10. Release, Indemnification, and Insurance.

10.1. Company's Release. For itself and its employees, Company hereby releases University and its regents, employees, and agents forever from any suits, actions, claims, liabilities, demands, damages, losses, or expenses (including reasonable attorneys' and investigative expenses) relating to or arising out of (i) the manufacture, use, lease, sale, or other disposition of a Licensed Product; or (ii) the assigning or sublicensing of Company's rights under this Agreement.

10.2.Company's Indemnification. Throughout the term of this Agreement and thereafter, Company shall indemnify, defend, and hold University and its regents, employees, and agents harmless from all suits, actions, claims, liabilities, demands, damages, losses, or expenses (including reasonable attorneys' and investigative expenses), relating to or arising out of the manufacture, use, lease, sale, or other disposition of a Licensed Product, including, without limitation, personal injury, property damage, breach of contract and warranty and products-liability claims relating to a Licensed Product and claims brought by a Sublicensee.

10.3.Company's Insurance.

10.3.1.General Insurance Requirement. Throughout the term of this Agreement, or during such period as the Parties shall agree in writing, Company shall maintain, and shall cause each Sublicensee to maintain, in full force and effect commercial general liability (CGL) insurance, with single claim limits consistent with industry standards. Such insurance policy will include coverage for claims that may be asserted by University against Company under section 10.2 "Company's Indemnification". Such insurance policy must name the Board of Regents of the University of Washington as an additional insured and will require the insurer to deliver written notice to University at the address set forth in Article 21 "Notices" of this Agreement, at least 45 days prior to the termination of the policy. Company shall deliver to University a copy of the certificate of insurance for such policy.

10.3.2.Clinical Trial Liability Insurance. Within thirty (30) days prior to the initiation of human clinical trials with respect to Licensed Product(s), Company shall provide to University certificates evidencing the existence and amount of clinical trials liability insurance. Company shall issue irrevocable instructions to its insurance agent and to the issuing insurance company to notify University of any discontinuance or lapse of such insurance not less than 45 days prior to the time that any such discontinuance is due to become effective. Company shall provide University a copy of such instructions upon their transmittal to the insurance agent and issuing insurance company. Company shall further provide University, at least annually, proof of continued coverage.

11. Warranties.

11.1.Authority. Each Party represents and warrants to the other Party that it has full corporate power and authority to execute, deliver, and perform this Agreement, and that no other corporate proceedings by such Party are necessary to authorize the Party's execution or delivery of this Agreement.

11.2.Disclaimers.

11.2.1.General Disclaimers. **EXCEPT FOR THE EXPRESS WARRANTY SET FORTH IN SECTION 11.1 "Authority" OF THIS AGREEMENT, UNIVERSITY DISCLAIMS AND EXCLUDES ALL WARRANTIES, EXPRESS AND IMPLIED, CONCERNING EACH LICENSED PATENT AND EACH LICENSED PRODUCT, INCLUDING, WITHOUT LIMITATION, WARRANTIES OF NON-INFRINGEMENT AND THE IMPLIED WARRANTIES OF MERCHANTABILITY AND FITNESS FOR A PARTICULAR PURPOSE.**

11.2.2.Patent Disclaimers. University expressly disclaims any warranties concerning and makes no representations:

11.2.2.1Patent Issuance. That the Licensed Patent(s) will be approved or will issue;

11.2.2.2Licensed Patent Validity/Scope. Concerning the validity or scope of any Licensed Patent; or

11.2.2.3Non-Infringement. That the manufacture, use, sale, lease or other disposition of a Licensed Product will not infringe a Third Party's patent or violate a Third Party's intellectual property rights.

12. Damages.

12.1.Remedy Limitation. **EVEN IF ADVISED OF THE POSSIBILITY OF SUCH DAMAGES, IN NO EVENT SHALL UNIVERSITY BE LIABLE FOR (A) PERSONAL INJURY OR PROPERTY DAMAGES ARISING IN CONNECTION WITH THE ACTIVITIES CONTEMPLATED IN THIS AGREEMENT OR (B) LOST PROFITS, LOST BUSINESS OPPORTUNITY, INVENTORY LOSS, WORK STOPPAGE, LOST DATA OR ANY OTHER RELIANCE OR EXPECTANCY, INDIRECT, SPECIAL, INCIDENTAL OR CONSEQUENTIAL DAMAGES, OF ANY KIND.**

12.2.Damage Cap. **IN NO EVENT WILL UNIVERSITY'S TOTAL LIABILITY FOR THE BREACH OR NONPERFORMANCE OF THIS AGREEMENT EXCEED THE AMOUNT OF PAYMENTS PAID TO UNIVERSITY UNDER SECTION A3 "PAYMENTS" of Exhibit A "EXCLUSIVE PATENT LICENSE SCHEDULE" OF THIS AGREEMENT. THIS LIMITATION WILL APPLY TO CONTRACT, TORT, AND ANY OTHER CLAIM OF WHATEVER NATURE.**

13. Amendment and Waiver.

This Agreement may be amended from time to time only by a written instrument signed by the Parties. No term or provision of this Agreement will be waived and no breach excused unless such waiver or consent will be in writing and signed by the Party claimed to have waived or consented. No waiver of a breach will be deemed to be a waiver of a different or subsequent breach.

14. Assignment.

The rights and licenses granted by University in this Agreement are personal to Company and Company shall not assign its interest or delegate its duties under this Agreement without the written consent of University; any such assignment or delegation made without written consent of University will not release Company from its obligations under this Agreement. The preceding sentence notwithstanding, Company, without the prior approval of University, may assign all, but no less than all, its rights and delegate all, but no less than all, its duties under this Agreement to a Third Party provided that:

(i) the assignment is made to such Third Party as a part of and in connection with (a) the sale by Company of all but no less than all of its assets to the Third Party, (b) if Company has more than one bona fide drug development programs, the sale by Company of all but no less than all of the assets of its business most closely associated with this Agreement, to the Third Party, (c) the sale, transfer, or exchange by the shareholders, partners, or equity owners of Company of a majority interest in Company to the Third Party, or (d) the merger of Company into the Third Party (each of the events described in part (a), (b), (c), or (d) of this paragraph, an "Assignment").

(ii) Company obtains from such Third Party written agreement to honor all obligations under this Agreement accrued by Company before Assignment and all obligations under this Agreement to accrue by such Third Party assignee after Assignment, including any and all financial obligations, and

(iii) no later than ten (10) days after the close of the transaction pursuant to which such Assignment is made, Company shall provide written notice to University of the Assignment, as well as a substitution of parties document, in which such Third Party assignee assumes responsibility for all of Company's outstanding and future obligations relating to this Agreement. Any assignment made in violation of this Article will be void and will, without further act, cause the immediate termination of this Agreement, effective retroactively to the date of the Assignment.

This Agreement will inure to the benefit of Company and University and their respective permitted assignees and trustees.

15. Confidentiality.

15.1. Form of transfer. Confidential Information may be conveyed in tangible or intangible form. Disclosing Party must clearly mark its Confidential Information "confidential." If disclosing Party communicates Confidential Information in non-written form, it shall reduce such communications to writing, clearly mark it "confidential", and provide a copy to receiving Party within 30 days of original communication at the address in Article 21 "Notices".

15.2. No Unauthorized Disclosure of Confidential Information. Beginning on the Effective Date and continuing throughout the term of this Agreement and thereafter for a period of [**], receiving Party shall not disclose or otherwise make known or available to any Third Party any disclosing Party Confidential Information, without the express prior written consent of disclosing Party. Notwithstanding the foregoing, receiving Party shall be permitted to disclose disclosing Party Confidential Information to (i) actual or potential investors, lenders, consultants, collaborators, Sublicensees, or development partners, which disclosure will be made under conditions of confidentiality and limited use and (ii) its attorney or agent as reasonably required. In no event shall receiving Party incorporate or otherwise use disclosing Party's Confidential Information in connection with any patent application filed by or on behalf of receiving Party. Receiving Party shall restrict the use of disclosing Party's Confidential Information exclusively to the terms of this Agreement. Receiving Party shall use reasonable procedures to safeguard

disclosing Party's Confidential Information. In the case where Company is the receiving Party, Company's confidentiality obligations will also apply equally to Sublicensees.

15.3. Access to University Information. University is an agency of the state of Washington and is subject to the Washington Public Records Act, RCW 42.56 et seq., ("Act"), and no obligation assumed by University under this Agreement shall be deemed to be inconsistent with University's obligations as defined under the Act and as interpreted by University in its sole discretion. If University receives a request for public records under the Act for documents containing Company Confidential Information, and if University concludes that the documents are not otherwise exempt from public disclosure, University will provide Company notice of the request before releasing such documents. Such notice will be provided in a timely manner to afford Company sufficient time to review such documents and/or seek a protective order, at Company's expense utilizing the procedures described in RCW 42.56.540. University shall have no obligation to protect Company Confidential Information from disclosure in response to a request for public records.

15.4. Disclosure as Required by Law. Either Party shall have the right to disclose the other Party's Confidential Information as required by law or valid court order, provided that such Party shall inform the Party who owns such Confidential Information prior to such disclosure and shall limit the scope and recipient of disclosure to the extent required by such law or court order.

16. Consent and Approvals.

Except as otherwise expressly provided, all consents or approvals required under the terms of this Agreement must be in writing and will not be unreasonably withheld or delayed.

17. Construction.

The headings preceding and labeling the sections of this Agreement are for the purpose of identification only and will not in any event be employed or used for the purpose of construction or interpretation of any portion of this Agreement. As used herein and where necessary, the singular includes the plural and vice versa, and masculine, feminine, and neuter expressions are interchangeable.

18. Enforceability.

If a court of competent jurisdiction adjudges a provision of this Agreement unenforceable, invalid, or void, such determination will not impair the enforceability of any of the remaining provisions hereof and the provisions will remain in full force and effect.

19. No Third-Party Beneficiaries.

No provision of this Agreement, express or implied, confers upon any person other than the Parties to this Agreement any rights, remedies, obligations, or liabilities hereunder. No Sublicensee shall have a right to enforce or seek damages under this Agreement.

20. Language.

Unless otherwise expressly provided in this Agreement, all notices, reports, and other documents and instruments that a Party hereto elects or is required by the terms of this Agreement to deliver to the other Party hereto will be in English.

21. Notices.

All notices, requests, and other communications that a Party is required or elects to deliver will be in writing and will be delivered personally, or by facsimile or electronic mail (provided such delivery is confirmed), or by a recognized overnight courier service or by United States mail, first-class, certified or registered, postage prepaid, return receipt requested, to the other Party at its address set forth below or to another address as a Party may designate by notice given pursuant to this article:

If to University: UW CoMotion
ATTN: Director, Innovation Development
4311 11th Avenue NE, Suite 500
Seattle, WA 98105-4608
Facsimile No.:

If to Company: Attn: Charles Legg, Head of Program Management
Solid GT
One Broadway
Cambridge, MA 02142
E-mail:

22. Patent Marking.

Company shall mark all material forms of Licensed Product(s) or packaging pertaining thereto made and sold by Company in the United States with patent marking conforming to 35 U.S.C. §287(a), as amended from time to time. Such marking shall further identify the pendency of any U.S. patent application and/or any issued U.S. or foreign patent forming any part of the Licensed Patents. All Licensed Product(s) shipped to or sold in other countries will be marked in such a manner as to provide notice to potential infringers pursuant to the patent law and practice of the country of manufacture or sale.

23. Publicity.

University shall have the right to report in its customary publications and presentations that University and Company have entered into a license agreement for the technology covered by the Licensed Patents and University may use Company logos in such publications and presentations provided that University does not modify Company's logos and does not through such use imply any endorsement by Company of University.

The Parties will cooperate with one another to review and respond to any press release or similar communication proposed by the other Party regarding the non-confidential subject matter of this Agreement. The specific content and timing of such press releases or similar communication is subject to mutual agreement by the Parties, which will not be unreasonably

withheld. Further, University and Company shall issue a joint press release regarding this Agreement, subject to both Party's review and approval of the specific content thereof, and such press release shall include specific mention of the contributions of University personnel and University in developing the technology in a prominent portion of the press release. Company shall provide University with appropriate quotes for such press release. University may post the press release in digital and print publications as well as on University's own website.

24. Relationship of Parties.

In entering into, and performing their duties under, this Agreement, the Parties are acting as independent contractors and independent employers. No provision of this Agreement shall create or be construed as creating a partnership, joint venture, or agency relationship between the Parties. No Party shall have the authority to act for or bind the other Party in any respect.

25. Relationship with Principal Investigator.

Company acknowledges that Principal Investigator is employed by University and has certain pre-existing obligations to University, including obligations with respect to disclosure and ownership of intellectual property and obligations arising from sponsored research agreements between University and Third Parties. Accordingly, Company agrees that to the extent that any consulting agreement is inconsistent with any of Principal Investigator's obligations to University, including the reporting of all inventions developed while employed by University (regardless of where arising) and including contractual obligations arising under any sponsored research agreements between University and Third Parties, then Principal Investigator's obligations to University shall prevail and to such extent any inconsistent provisions of this consulting agreement shall be deemed inapplicable and unenforceable.

26. Security Interest.

In no event shall Company grant, or permit any person to assert or perfect, a security interest in Licensed Patents or in Company's rights under this Agreement.

27. Survival.

Immediately upon the termination or expiration of this Agreement all Company's rights under this Agreement will terminate; provided, however, Company's obligations that have accrued prior to the effective date of termination or expiration of this Agreement (e.*g.*, the obligation to report and make payments on sales, leases, or dispositions of Licensed Products and to reimburse University for costs) and the obligations specified in Sections 6.1 "Payments" and 6.4 "Sales Reports" will survive. The obligations and rights set forth in Sections 6.5 "Records Retention and Audit Rights" and 9.3 "Effect of Termination" and Articles 10 "Release, Indemnification, and Insurance", 11 "Warranties", 12 "Damages", 15 "Confidentiality", 29 "Applicable Law" and 30 "Forum Selection" will survive the termination or expiration of this Agreement.

28. Collection Costs and Attorneys' Fees.

If a Party fails to perform an obligation or otherwise breaches one or more of the terms of this Agreement, the other Party may recover from the non-performing breaching Party all its costs (including actual attorneys' and investigative fees) to enforce the terms of this Agreement.

29. Applicable Law.

The internal laws of the state of Washington will govern the validity, construction, and enforceability of this Agreement, without giving effect to the conflict of laws principles thereof.

30. Forum Selection.

A suit, claim, or other action to enforce the terms of this Agreement will be brought exclusively in the state and federal courts of King County, Washington. Company hereby submits to the jurisdiction of that court and waives any objections it may have to that court asserting jurisdiction over Company or its assets and property.

31. Entire Agreement.

Company has evaluated the Licensed Patents under a Confidentiality Agreement ("CDA") with University (UW # 35111A) with an effective date of June 16th, 2014 and under an Exclusive Option ("Option") with University (UW # 36201A) with an effective date of February 5th, 2015. This Agreement (including all attachments, exhibits, and amendments) is the final and complete understanding between the Parties concerning licensing the Licensed Patents. This Agreement supersedes any and all prior or contemporaneous negotiations, representations, and agreements, whether written or oral, concerning the Licensed Patents. However, the obligations of nonuse and nondisclosure for Confidential Information disclosed pursuant to the CDA shall survive until June 16, 2019. Confidential Information disclosed pursuant to this Agreement shall be governed by the terms of this Agreement. This Agreement may not be modified in any manner, except by written agreement signed by an authorized representative of both Parties.

IN WITNESS WHEREOF, the Parties have caused this Agreement to be duly executed by their respective authorized representatives.

University of Washington		***Solid GT***	
By:	/s/ Fiona Wills	By:	/s/ Ilan Ganot
Name:	Fiona Wills	Name:	Ilan Ganot
Title:	Director, Innovation Development	Title:	CEO
Date:	10/16/2015	Date:	10/16/2015

Exhibit A

Exclusive Patent License Schedule

A1. Licensed Patents:

UW Reference #	Application Serial #	Filing Date	Type	Status
[**]	[**]	[**]	[**]	[**]

A2. Performance Milestones (Section 5.1 "Commercialization and Performance Milestones"):

Company shall meet the following performance milestones:

A2.1 Milestone 1 – [**]

A2.2 Milestone 2- [**]

A2.3 Milestone 3 – [**]

A2.4 Milestone 4 – [**]

A3. Payments (Section 6.1):

A3.1 Up-front Payment. Company shall pay to University within 14 days of the Effective Date [**] as an up-front payment. This up-front payment shall be non-refundable and not creditable against future royalty obligations.

A3.2 Annual Maintenance Fee. Company shall pay to University an annual maintenance fee of [**] due on the 2nd anniversary of the Effective Date and every year thereafter for the term of the Agreement. Annual Maintenance Fees shall terminate immediately when Company begins to pay Minimum Annual Royalties (A3.4).

A3.3 Running Royalty Payments. Company shall pay to University within 30 days after the last day of each calendar quarter during the term of this Agreement an amount equal to [**] of Net Sales during such quarter as a running royalty payment.

A3.4 Minimum Annual Royalties. Company as well as each of its Sublicensees shall pay minimum annual royalties of [**] for the term of this Agreement to be creditable against running royalty payments for the preceding calendar year on a non-cumulative basis and to be due in full and payable on January 31st of each year beginning on January 31st of the second year following the first commercial sale and continuing during the term of this Agreement.

A3.4.1 If this Agreement is terminated prior to the payment of a minimum annual royalty in any given year the amount due for that minimum annual royalty payment will be prorated on the basis of the number of full quarters that have elapsed prior to termination since the last payment of a minimum annual royalty.

A3.5 Financial Milestones. Company shall pay to University the following non-cumulative and non-refundable milestone achievement payments within 30 days of achieving the corresponding milestone, whether achieved by Company or a Sublicensee. For clarity, payments will be due only once in respect of the first achievement of the milestones below for a Licensed Product, regardless of the number of Licensed Products to achieve the milestone.

A3.5.1 [**]

A3.5.2 [**]

A3.5.3 [**]

A3.5.4 [**]

A3.6 Third Party Royalties. If Company is required to pay royalties to a Third Party based on Company's manufacture, use, or sale of Licensed Product subject to one or more patents of such Third Party then the royalty Company pays to University may be reduced by [**] of the royalty actually paid to the Third Party provided that use of any Third Party patent is required for such manufacture, use, or sale of Licensed Product, and provided that the royalty to the University shall not fall below [**].

A3.7 Sublicensing Consideration. Within 30 days of the end of each calendar quarter during the term of this Agreement, Company shall pay to University [**] of any Sublicensing Consideration received by Company during such calendar quarter unless reduced by achievement of milestones by Company or its Sublicensee prior to execution of the particular Sublicense in accordance with the schedule below. A reduction of the percentage of Sublicensing Consideration payable to University under this Agreement wexhbill be negotiated in good faith between the Parties where, in addition to the Sublicense of any rights granted to Company hereunder, Company also grants Sublicensee a license under a Third Party's intellectual property rights, which license is necessary for Sublicensee to manufacture, have manufactured, use, offer to sell or sell, offer to lease or lease, import, or otherwise offer to dispose or dispose of Licensed Product(s) without infringing such Third Party's intellectual property rights, and only to the extent that the total aggregate consideration for such combined license is treated as Sublicensing Consideration.

A3.7.1 After achievement of Milestone 1: [**]

A3.7.2 After achievement of Milestone 2: [**]

A3.7.3 After achievement of Milestone 3: [**]

A3.8 Assignment Fee. Within 30 days of any assignment of rights granted to Company under this Agreement, Company shall pay to University [**] of any Assignment Consideration received by Company.

A4. Patent Cost Reimbursement: Company shall reimburse University for all Patent Expenses incurred prior to the Effective Date within 60 days of the Effective Date. Company shall pay, or reimburse University for paying, all Patent Expenses incurred on or after the Effective Date within 30 days of its receipt of University's invoice for such Patent Expenses. University reserves the right to request advance payments for certain Patent Expenses, at University's discretion.

Exhibit 10.8

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

LICENSE AGREEMENT

THIS LICENSE AGREEMENT ("AGREEMENT") is made and entered into this 15th day of October 2015 ("EFFECTIVE DATE"), by and between THE CURATORS OF THE UNIVERSITY OF MISSOURI, a public corporation of the State of Missouri ("UNIVERSITY") and Solid GT, LLC having offices at One Broadway Street, Cambridge, MA 02142 ("LICENSEE"). UNIVERSITY and LICENSEE may sometimes be referred to herein as a "PARTY" or "PARTIES" as the case may be.

WHEREAS, UNIVERSITY has an ownership interest in certain PATENT RIGHTS as defined herein related to [**]; and

WHEREAS, the PATENT RIGHTS were developed under a research program sponsored by NIH. Therefore, this AGREEMENT is subject to the terms and conditions of the Bayh-Dole Act, Public Law 96-517 and 98-620 as amended; and

WHEREAS, LICENSEE is desirous of obtaining a license to practice the PATENT RIGHTS under the terms and conditions of this AGREEMENT; and

WHEREAS, UNIVERSITY is desirous of granting such a license to LICENSEE in accordance with the terms and conditions of this AGREEMENT.

NOW, THEREFORE, in consideration of the foregoing premises and the covenants, representations and warranties contained herein, the PARTIES agree as follows:

Article I.DEFINITIONS

Section 1.01"IMPROVEMENTS" shall mean any modification, enhancement, or improvement to an invention described in the PATENT RIGHTS which is owned by, licensed to, or otherwise controlled by LICENSEE that (1) would be infringed, either directly or indirectly, by the practice of an invention claimed in the PATENT RIGHTS; or (2) if not for the license granted under this AGREEMENT, would infringe, either directly or indirectly, one or more claims of the PATENT RIGHTS.

Section 1.02"LICENSED FIELD" means the following business areas: Treatment of Duchene Muscular Dystrophy and other disease indications resulting from a lack of functional dystrophin.

Section 1.03"LICENSED PRODUCT" means any product, apparatus, kit, composition, or component thereof (a) whose use, sale, offer for sale, or importation of which is covered, in whole or in part, by any VALID CLAIM contained in the PATENT RIGHTS or (b) which is made by any method, procedure, process, or step which is covered, in whole or in part, by any VALID CLAIM contained in the PATENT RIGHTS.

Section 1.04"LICENSED TERRITORY" means worldwide.

Section 1.05"NET SALES" means the amount billed or invoiced for the SALE of LICENSED PRODUCTS, less:

(a) Customary trade, quantity or cash discounts;

(b) Amounts repaid or credited by reason of rejection or return;

(c) Charges for transportation or delivery to be paid by or on behalf of LICENSEE's customer, to the extent such charges are separately stated on purchase orders, invoices or other documents of SALE; and

(d) Sales, tariff duties and/or use taxes directly imposed and with reference to particular SALES. In calculating NET SALES, no deductions shall be made for commissions paid to individuals whether they are with independent sales agencies or regularly employed by LICENSEE and on its payroll, or for cost of collections. In the event LICENSEE SELLS a LICENSED PRODUCT to a third party in a bona fide arm's length transaction, for consideration, in whole or in part, other than cash, then the NET SALES price for such LICENSED PRODUCT shall be deemed to be the standard invoice price then being invoiced by LICENSEE in an arm's length transaction with similar entities and in the absence of such standard invoice price, then the reasonable fair market value of the LICENSED PRODUCT. For the purposes of calculating NET SALES, LICENSEE's SALES to a SUBLICENSEE under this AGREEMENT for end use (but not resale) by the SUBLICENSEE shall be treated as SALES by LICENSEE at the greater of the (i) billed/invoiced price of LICENSEE the SUBLICENSEE or (ii) the billed/invoiced price that LICENSEE would have charged a third party in a bona fide arm's length transaction. For the purposes of calculating NET SALES, LICENSEE's SALES to a SUBLICENSEE under this AGREEMENT for resale to end users by the SUBLICENSEE shall be treated as SALES at the billed/invoiced price to the end users of SUBLICENSEE.

Section 1.06"NON-COMMERCIAL RESEARCH PURPOSES" means the use or practice of the PATENT RIGHTS for research, teaching, educational, or academic purposes which are undertaken at UNIVERSITY or at a non-profit, academic, educational, or governmental institution. Without limiting the foregoing, NON-COMMERCIAL RESEARCH PURPOSES includes the use or practice of the PATENT RIGHTS for research (including sponsored research) that leads, or may lead, to patentable or unpatentable inventions that may be licensed or otherwise transferred, either directly or indirectly, to third parties.

Section 1.07"PATENT RIGHTS" means UNIVERSITY'S rights in any of the following: (i) [**] and (ii) [**]; (collectively (i), (ii), are the "PATENT APPLICATIONS"); and (iii) any provisional, non-provisional, divisional, continuation (but not continuations-in-part), extension, renewal, re-examination, reissue, substitute, supplementary protection certificate, utility model, or similar legal protection claiming priority to or from the PATENT APPLICATIONS; and (iv) any corresponding foreign applications or patents thereof. All of the foregoing will be

automatically incorporated in and added to this AGREEMENT and shall periodically be added to Appendix A attached to this AGREEMENT and made part thereof.

Section 1.08"ROYALTY PERIOD(S)" means the three-month periods ending on March 31, June 30, September 30, and December 31.

Section 1.09"SALE", "SELL", or "SOLD" means the sale, use, transfer, distribution or disposition of a LICENSED PRODUCT for value to a third party.

Section 1.10"SUBLICENSEE" means any person or entity to whom LICENSEE transfers any right or interest granted to LICENSEE by UNIVERSITY under this Agreement.

Section 1.11"VALID CLAIM" means either (a) a claim of an issued patent that has not been held unenforceable or invalid by an agency or a court of competent jurisdiction in any unappealable or unappealed decision or (b) a claim of a pending patent application that has not been abandoned or finally rejected without the possibility of appeal or refiling and that has been pending for less than ten (10) years from its priority date.

Article II.GRANT

Section 2.01Grant. Subject to the terms and conditions of this AGREEMENT, UNIVERSITY hereby grants to LICENSEE and LICENSEE accepts a royalty-bearing, exclusive license under the PATENT RIGHTS to make, have made, use, SELL, have SOLD, import, distribute, or otherwise transfer LICENSED PRODUCTS within the LICENSED TERRITORY for use within LICENSED FIELD for a term as set forth in Section 10.01 unless this AGREEMENT shall be sooner terminated according to the terms hereof. For the avoidance of doubt, this grant is subject to the rights retained by UNIVERSITY in Section 2.03, UNIVERSITY'S publication rights in Section 2.06, and any rights of the GOVERNMENT as set forth in Section 2.07.

Section 2.02Sublicenses. The license granted in Section 2.01 above shall include the right to grant written sublicenses, subject to UNIVERSITY'S prior written approval which approval shall not be unreasonably withheld. SUBLICENSEE shall have no right to grant further sublicenses without written consent from University, where such consent is within University's sole discretion. In determining whether to approve a sublicense (or any amendment thereto), UNIVERSITY will consider, among other things, whether the provisions of the proposed sublicense arc consistent with and similar to those required of LICENSEE by this AGREEMENT. All sublicenses must comply with the following:

(a) LICENSEE shall deliver to UNIVERSITY a true and correct copy of each fully executed sublicense granted by LICENSEE, and any modification or termination thereof, within thirty (30) days after execution, modification, or termination.

(b) LICENSEE shall deliver to UNIVERSITY copies of all reports due to LICENSEE from SUBLICENSEE within thirty (30) days receipt of such reports by LICENSEE.

(c) LICENSEE shall, at such times as UNIVERSITY directs and at UNIVERSITY'S request, permit the inspection of SUBLICENSEE's records by UNIVERSITY's

auditors or an independent certified public accountant selected by UNIVERSITY under the terms of Section 4.05.

(d) No sublicense shall relieve LICENSEE of its representations, warranties, or obligations under this AGREEMENT. LICENSEE shall be responsible to UNIVERSITY for the performance of its SUBLICENSEES under each sublicense agreement granting rights to any PATENT RIGHTS. LICENSEE shall collect and guarantee all payments due UNIVERSITY from any SUBLICENSEE.

(e) Any sublicense granted by LICENSEE to a SUBLICENSEE shall incorporate all of the representations, warranties, terms, conditions, and obligations of this AGREEMENT, which shall be binding upon each SUBLICENSEE as if such SUBLICENSEE were a party to this AGREEMENT. LICENSEE shall require that any sublicense agreement:

(i) be consistent with the terms, conditions, covenants, warranties, representations, limitations, obligations, and duties of LICENSEE under this AGREEMENT;

(ii) prohibit the SUBLICENSEE from granting further sublicenses without written consent from UNIVERSITY, where such consent is within UNIVERSITY's sole discretion; and

(iii) contain express provisions under which the SUBLICENSEE expressly accepts duties and obligations at least equivalent to those accepted by the LICENSEE in the following sections of this AGREEMENT: Section 2.03 (reserved rights), Section 2.04 (license to University), Section 2.06 (publication), Section 2.07 (governmental rights), Section 3.07 (challenge to patent rights), Section 4.03 (reporting), Section 4.05 (records), Section 6.01 (indemnity), Section 6.02 (insurance); Section 6.03 (disclaimer of warranties), Section 6.04 (damages exclusion/ limitation of remedies), Section 6.06 (sublicenses) Section 7.03 (entity status), Section 10.05 (assignment of sublicenses), Section 11.01 (marking), Section 11.02 (compliance with laws / export controls), Section 11.03 (university name), and Section 11.11 (severability).

(f) If any sublicense agreement granting any rights to the PATENT RIGHTS does not comport with above requirements in this Section 2.02(e), then that agreement shall be invalid, unenforceable, and void.

(g) Upon any termination of this AGREEMENT, all SUBLICENSEE's rights shall also terminate except as set forth in Section 10.05 (assignment of sublicenses).

Section 2.03Reserved Rights. UNIVERSITY reserves the right to make, use or otherwise practice the PATENT RIGHTS for NON-COMMERCIAL RESEARCH PURPOSES and to grant nonexclusive licenses to non-profit, academic, educational, or governmental institutions a royalty-free right to make, use or otherwise practice the PATENT RIGHTS for NON-COMMERCIAL RESEARCH PURPOSES. UNIVERSITY also reserves the right to transfer

tangible research materials and intangible materials incorporating the PATENT RIGHTS to other non-profit, academic, educational, or governmental institutions for such NON-COMMERCIAL RESEARCH PURPOSES. LICENSEE agrees that, notwithstanding any other provision of this AGREEMENT, that LICENSEE has no right to enforce the PATENT RIGHTS against UNIVERSITY or any nonprofit, academic, educational, or governmental institution with respect to such use or practice for NON-COMMERCIAL RESEARCH PURPOSES.

Section 2.04License to University. LICENSEE hereby grants to UNIVERSITY, a nonexclusive, royalty-free, irrevocable, paid-up license, with the right to grant sublicenses to non-profit, academic, educational, or governmental institutions, to practice and use IMPROVEMENTS solely for NON-COMMERCIAL RESEARCH PURPOSES.

Section 2.05License Scope. The license granted herein shall not be construed to confer any rights upon LICENSEE by implication, estoppel or otherwise as to any technology not specifically set forth in PATENT RIGHTS. UNIVERSITY shall be free to grant commercial licenses to the PATENT RIGHTS to third parties in all fields outside the LICENSE FIELD and/or outside the LICENSED TERRITORY.

Section 2.06Publication. LICENSEE agrees that UNIVERSITY shall have a right to publish any research results or technical data related to or arising out of the PATENT RIGHTS in accordance with UNIVERSITY's general policies and that this AGREEMENT shall not restrict, in any fashion, UNIVERSITY's right to publish.

Section 2.07Governmental Rights. LICENSEE understands that the PATENT RIGHTS were developed under a funding agreement with the Government of the United States of America ("GOVERNMENT") and that the GOVERNMENT may have certain rights relative thereto. Thus, notwithstanding anything hereunder, any and all licenses and other rights granted hereunder are limited by and subject to the rights and requirements of the GOVERNMENT which may arise out of its sponsorship of the research which led to the conception or reduction to practice of the PATENT RIGHTS. The GOVERNMENT is entitled, as a right, under the provisions of 35 U.S.C. §§ 200-212 and applicable regulations of Title 37 of the Code of Federal Regulations: (i) to a nonexclusive, nontransferable, irrevocable, paid-up license to practice or have practiced for or on the behalf of the GOVERNMENT any of the PATENT RIGHTS throughout the world and (ii) to exercise march in rights on PATENT RIGHTS. This AGREEMENT shall be exclusive, to the extent allowed in accordance with Public Laws 96-517 and 98-620 in the LICENSED FIELD and is explicitly made subject to the GOVERNMENT's rights under such GOVERNMENT funding agreement and any applicable law or regulation. If there is a conflict between the GOVERNMENT funding agreement, applicable law or regulation and this AGREEMENT, the terms of the GOVERNMENT funding agreement, applicable law or regulation shall prevail. LICENSEE agrees to take any actions necessary to enable UNIVERSITY to satisfy its obligations with the GOVERNMENT relating to the PATENT RIGHTS. LICENSEE agrees, during the period of exclusivity of this license in the United States, that any LICENSED PRODUCT produced for SALE in the United States will be manufactured substantially in the United States as required by 35 U.S.C. § 204.

Article III.PAYMENTS

Section 3.01 License Payments: In consideration of rights granted by UNIVERSITY to LICENSEE under this AGREEMENT, LICENSEE will pay UNIVERSITY the following:

(a) License Execution Payment. LICENSEE shall pay to UNIVERSITY a nonrefundable license execution fee in the amount of [**], due and payable when this AGREEMENT is fully executed.

(b) Annual Maintenance Fee. LICENSEE shall pay to UNIVERSITY an annual license maintenance fee ("ANNUAL MAINTENANCE FEE"). This ANNUAL MAINTENANCE FEE shall be due on the anniversary of the EFFECTIVE DATE of each of the years specified below. However, the ANNUAL MAINTENANCE FEE will be offset by sponsored research support received by UNIVERSITY from Solid GT in the preceding applicable twelve (12) month period from the anniversary of the EFFECTIVE DATE. For example, if LICENSEE provides UNIVERSITY with $[**] of funding received between the EFFECTIVE DATE and the first anniversary of the EFFECTIVE DATE, then no ANNUAL MAINTENANCE FEE for 2016 shall be due and owing. If LICENSEE then provides UNIVERSITY with an additional funding of $[**] between the first anniversary of the EFFECTIVE DATE and the second anniversary of the EFFECTIVE DATE, then, the ANNUAL MAINTENANCE FEE for 2017 shall be $[**] ($[**]-[**]).

The ANNUAL MAINTENANCE FEE shall be:

(1) In 2016: [**];

(2) In 2017: [**];

(3) In 2018: [**]; and in each year thereafter during the term of this AGREEMENT until Minimum Annual Royalties apply.

(c) Running Royalty / Earned Royalty. LICENSEE shall pay UNIVERSITY a running royalty equal to [**] of NET SALES (hereinafter "SALES ROYALTY") for LICENSED PRODUCTS SOLD by LICENSEE or SUBLICENSEES. A SALES ROYALTY accrues when LICENSED PRODUCTS are invoiced or shipped, whichever occurs first.

If LICENSEE or SUBLICENSEE is required to pay royalties to one or more third parties in consideration of a license or similar right in order to make, use, or sell LICENSED PRODUCTS, LICENSEE shall be entitled to credit up to [**] of the amounts actually paid by LICENSEE or SUBLICENSEE to such third parties against the royalties due to UNIVERSITY under this AGREEMENT in the same ROYALTY PERIOD; provided, however, that in no event will the royalties due to UNIVERSITY, when aggregated with any other offsets and credits allowed under this AGREEMENT, be less than [**] of the SALES ROYALTY on NET SALES, as defined above, in any REPORTING PERIOD. For clarity, the maximum adjusted royalty in Section 3.01(c) are [**].

LICENSEE or its SUBLICENSEE(S) (as applicable) will promptly notify UNIVERSITY should a compulsory license be granted, or be the subject of a possible grant, by LICENSEE or a SUBLICENSEE to a third party under the applicable laws, rules, regulations, guidelines, or other directives of any governmental or supranational agency in the LICENSED TERRITORY under the PATENT RIGHTS, and the total amount payable under this Section 3.01(c) with respect to the SALES ROYALTY in such country will be adjusted to match any lower amount such third party may be allowed to pay solely with respect to the NET SALES of such LICENSED PRODUCT in such country, but not any other countries.

(d) Minimum Annual Royalty Payment. LICENSEE shall pay to UNIVERSITY a non-refundable minimum annual royalty of [**] due and payable beginning on the anniversary of the EFFECTIVE DATE after first commercial sale of LICENSED PRODUCTS in the United States or a European Union country. Each minimum annual royalty payment is creditable against SALES ROYALTY due UNIVERSITY during the twelve (12) month period following each date the minimum annual royalty becomes due and is subsequently paid. For the avoidance of doubt, such minimum annual royalty shall be considered a payment in advance of royalties yet to accrue.

(e) Milestone Payments. LICENSEE shall pay UNIVERSITY a milestone payment fee in accordance with the following schedule for each LICENSED PRODUCT developed.

Event		Amount
Milestone A:	[**]	$ [**]
Milestone B:	[**]	$ [**]
Milestone C:	[**]	$ [**]
Milestone D:	[**]	$ [**]

Milestone fees are non-refundable. Royalty payments in a given license year shall not be creditable against any milestone fees.

Section 3.02Sublicense Royalties and Fees

(a) Sublicensee Earned Royalty. For the avoidance of doubt, LICENSEE shall pay to UNIVERSITY an amount for NET SALES made by SUBLICENSEES equal to what LICENSEE would have been required to pay to UNIVERSITY had LICENSEE made such NET SALES.

(b) Other Sublicensee Payments. In consideration of rights granted by UNIVERSITY to LICENSEE under this AGREEMENT, in addition to the sublicensee earned royalty of Section 3.02(a), LICENSEE further agrees to pay UNIVERSITY a specified portion of other revenue or consideration received

from any SUBLICENSEE as consideration for the sublicense of PATENT RIGHTS to SUBLICENSEES as per the following schedule.

Event	Specified Portion
[**]	[**]
[**]	[**]
[**]	[**]

Such revenue or other consideration attributable to the SUBLICENSE of PATENT RIGHTS ("SUBLICENSE REVENUE") shall include, but not be limited to, all option fees, license issue fees (up-front payments), license maintenance fees, milestone payments, payments for equity in excess of fair market value, joint marketing fees and research and development funding in excess of LICENSEE's cost of performing such research and development (other than the earned royalty specified in Section 3.02(a)). In the event that LICENSEE agrees to receive only equity at fair market value from the SUBLICENSEE for development rights and as payment for all milestone events per agreement between LICENSEE and SUBLICENSEE, UNIVERSITY is entitled to a portion of that equity equal to the specified portion percentage for sublicenses listed above or may opt to receive a cash equivalent based on the estimated fair market value at time agreement is signed. For clarity, SUBLICENSE REVENUE shall not include (1) research and development funding provided to LICENSEE by SUBLICENSEE, (2) payments made as consideration for the issuance of equity or debt securities of LICENSEE at fair market value; provided that, if a SUBLICENSEE pays more than fair market value for equity or debt securities, only the portion in excess of fair market value shall be considered revenue, and (3) payments received from SUBLICENSEE and applied to reimburse LICENSEE for any out-of-pocket expenses related to the filing, prosecution, protection, defense and maintenance of patents and patent applications.

In addition, for each LICENSED PRODUCT developed, if LICENSEE receives from a SUBLICENSEE under any sublicense a payment that constitutes SUBLICENSEE REVENUE and which payment is directly attributable to the occurrence of a milestone or event substantially equivalent to a milestone triggering a payment under Section 3.01(e), and LICENSEE has paid to UNIVERSITY the corresponding Specified Portion of the amount of SUBLICENSE REVENUE that is attributable to such payment as set forth in Section 3.02(b), then such payment to UNIVERSITY from LICENSEE shall be fully creditable against the milestone payment owing from LICENSEE to UNIVERSITY under Section 3.01(e) for that applicable milestone.

Section 3.03How Payments are Made. All payments to UNIVERSITY pursuant to this AGREEMENT shall be paid in U.S. dollars. Conversion of foreign currency to U.S. dollars shall be made at the conversion rate existing in the United States (as reported in the in the Wall Street Journal) on the last working day of each ROYALTY PERIOD. Such payments shall be without deduction of exchange, collection or other charges. Such payments shall be made payable to The Curators of the University of Missouri and shall be mailed to the Office of Intellectual Property Administration, 475 McReynolds Hall, Columbia, MO 65211.

Section 3.04Payment Deadlines. Unless stipulated otherwise, all payments due UNIVERSITY hereunder shall be made within thirty (30) days after the end of each ROYALTY PERIOD. Late payments shall be subject to an interest charge of one and one half percent (1.5%) per month. LICENSEE shall also be responsible for payment of all bank transfer charges.

Section 3.05No Taxes. Taxes and/or other governmental charges or fees shall not be levied on the payments made to UNIVERSITY under this Article III and shall not be deducted from any payments due UNIVERSITY under this Article III. LICENSEE shall be responsible for any and all taxes, fees, levies, duties, or other charges imposed by the government of any country on such payments.

Section 3.06Default Payment. In the event of default in payment of any payment owing to UNIVERSITY under the terms of this AGREEMENT, and if it becomes necessary for UNIVERSITY to engage outside legal counsel to collect such payment, LICENSEE shall pay all expenses, costs and attorneys' fees incurred by UNIVERSITY in connection therewith. Further, in the event that UNIVERSITY brings a lawsuit against a SUBLICENSEE for failure to pay any royalties or other payments due, LICENSEE shall pay all expenses, costs and attorneys' fees incurred by UNIVERSITY in connection therewith. LICENSEE shall use its best commercial efforts to enforce any SUBLICENSEE obligation or payment if the breach of that obligation or payment would be a breach of this AGREEMENT if made by LICENSEE. To the extent that LICENSEE may as to UNIVERSITY cure such breach by its own performance, *e.g.,* by making any payments due to UNIVERSITY regardless of SUBLICENSEE'S failure to pay LICENSEE, then LICENSEE shall do so at its own risk and expense.

Section 3.07Challenge to Patent Rights. In the event that LICENSEE or one or more of its SUBLICENSEES directly or indirectly: (a) issues a press release, public announcement, news release alleging invalidity or unenforceability of any claim within the PATENT RIGHTS; or (b) asserts a claim or counterclaim in the courts or before the applicable governmental agency (*e.g.,* the United States Patent Trial and Appeal Board) seeking to attack, invalidate or render unenforceable any claim within the PATENT RIGHTS; or (c) assists a third party with either or both (a) or (b) (each of (a), (b), or (c) being a "CHALLENGE EVENT"), then LICENSEE or its SUBLICENSEE as applicable shall provide at least ninety (90) days written notice to UNIVERSITY prior to initiating such a CHALLENGE EVENT, along with a copy of any prior art which forms the basis for the CHALLENGE EVENT and a claim-by-claim detailed analysis of patent invalidity and/or unenforceability. Upon the occurrence of a CHALLENGE EVENT, UNIVERSITY, shall have the right, but not the obligation, to terminate this AGREEMENT with respect to such LICENSEE and/or SUBLICENSEE by providing written notice of the same. In the event that UNIVERSITY elects not to terminate this AGREEMENT, then all payments due under Article III by LICENSEE or SUBLICENSEE(s) as applicable shall double. Moreover, should the outcome of any such action or proceeding be unsuccessful, then LICENSEE and/or SUBLICENSEE challenging such claim shall pay (1) triple all payments after the pendency of the aforementioned action and (2) UNIVERSITY'S costs, expenses, and reasonable attorneys' fees incurred in such action. An action or proceeding shall be deemed "unsuccessful" for purposes of this Section 3.07 if: (i) the proceeding or lawsuit is terminated for any reason prior to a settlement or judgment from which no appeal can be or is taken; (ii) one or more of the claims within the PATENT RIGHTS challenged by said lawsuit remain valid and enforceable after any

such settlement or judgment is in effect; or (iii) if LICENSEE would still require a license to any of the PATENT RIGHTS to sell any of its products after any such settlement or judgment is in effect. Any such judicial challenge by LICENSEE or a SUBLICENSEE shall be brought in the courts of Missouri, and LICENSEE and its SUBLICENSEE agree not to challenge personal jurisdiction in that forum. LICENSEE or such SUBLICENSEE shall not be relieved from any payments that accrue before any decision invalidating a claim within the PATENT RIGHTS or a claim not involved in such decision. LICENSEE or such SUBLICENSEE shall have no right to recoup any such payments paid before or during the period of challenge.

Article IV.REPORTING

Section 4.01Commercialization Plan. Prior to signing this AGREEMENT, LICENSEE has provided to UNIVERSITY a written plan (hereinafter "COMMERCIALIZATION PLAN") for the LICENSED PRODUCT within the respective LICENSED FIELD and within the respective country or countries of the LICENSED TERRITORY to be introduced by LICENSEE into commercial use. The COMMERCIALIZATION PLAN shall include, without limitation: (1) planned research and development activities, (2) milestones and evidence of sufficient financial resources to successfully implement the COMMERCIALIZATION PLAN and ensure that LICENSED PRODUCT will be kept reasonably available to the public, and (3) projection of sales and proposed marketing efforts. Such COMMERCIALIZATION PLAN is incorporated as Appendix B.

Section 4.02Section 4.02 First Sale. LICENSEE shall report to UNIVERSITY the date of first SALE of LICENSED PRODUCTS in each country of LICENSED TERRITORY within thirty (30) days of occurrence.

Section 4.03Reporting. Within 30 days after each ROYALTY PERIOD following the first SALE of LICENSED PRODUCT, whether SOLD by LICENSEE or its SUBLICENSEE, if any exists, LICENSEE must deliver to UNIVERSITY a true and accurate written report, even if no payments are due UNIVERSITY, giving the particulars of the business conducted by LICENSEE and its SUBLICENSEE(s) during the ROYALTY PERIOD as are pertinent to calculating payments hereunder. This report will include at least:

(a) the quantities of LICENSED PRODUCT produced or manufactured;

(b) the total NET SALES, including any deductions applicable as provided in Section 1.05;

(c) the exchange rate used;

(d) the offsets of minimum annual royalties or other offsets allowed under this AGREEMENT;

(e) the method used to calculate the royalties thereon;

(f) the total SALES ROYALTY computed and due UNIVERSITY;

(g) the royalties due UNIVERSITY on additional payments from SUBLICENSEE(s) under Section 3.02; and

(h) the names and addresses of all SUBLICENSEES of LICENSEE.

If no payment is due, LICENSEE shall so report to UNIVERSITY. An exemplary report format is set forth in Appendix C. This report shall identify the issued patents and/or patent applications under PATENT RIGHTS that cover the particular LICENSED PRODUCT being reported. LICENSEE shall direct its authorized representative to certify that reports required hereunder are correct to the best of LICENSEE's knowledge and information. Failure to provide reports as required under this Article shall be a material breach of this AGREEMENT.

LICENSEE shall provide sufficient data for UNIVERSITY to verify the royalty calculations and any reasonable additional information UNIVERSITY requires to determine LICENSEE's satisfaction of the reporting requirements hereunder or to clarify the information contained in reports provided by LICENSEE. LICENSEE shall provide such additional information to UNIVERSITY within thirty (30) days of receiving a request from UNIVERSITY. Simultaneously with the delivery of each report, LICENSEE must pay to UNIVERSITY the amount, if any, due for the period of each report.

Section 4.04Annual Commercialization Report. On or before each anniversary of the EFFECTIVE DATE, irrespective of having a first SALE or offer for SALE, LICENSEE must deliver to UNIVERSITY a written annual report as to LICENSEE's (and any SUBLICENSEE's) efforts and accomplishments during the preceding year in diligently commercializing LICENSED PRODUCT in the LICENSED FIELD, including but not limited to,

(a) research and development expenditures and progress,

(b) regulatory filings and approvals,

(c) manufacturing,

(d) sublicensing activities,

(e) marketing and sales,

(f) jobs created,

(g) capital raised and source of funding,

(h) LICENSEE's (and, if applicable, SUBLICENSEE's) commercialization plans for the upcoming year.

LICENSEE shall also promptly provide any reasonable additional information UNIVERSITY requested to evaluate LICENSEE'S performance under this AGREEMENT.

Section 4.05Records. LICENSEE shall keep full, true and accurate books of account containing all particulars that may be necessary for the purpose of showing the amounts payable to UNIVERSITY. The books of account shall be kept at LICENSEE's principal place of business or the principal place of business of the appropriate division of LICENSEE to which this AGREEMENT relates. The books, ledgers, records, and the supporting data shall be open at all reasonable times for five (5) years following the end of the calendar year to which they pertain, for the inspection by UNIVERSITY or its representatives for the purpose of verifying LICENSEE's royalty statements or compliance in other respects with this AGREEMENT. If the amounts due to UNIVERSITY are determined to have been underpaid, LICENSEE will pay the amount of such underpayment and interest on the amount of such underpayment with interest accumulating at the rate as set forth in Section 3.04 accruing from the date such payment was originally due to UNIVERSITY. Should such inspection lead to the discovery of a greater than [**] discrepancy or [**] or more in reporting to UNIVERSITY's detriment, LICENSEE agrees to pay the full cost of such inspection and audit.

Section 4.06Sunshine Law. LICENSEE acknowledges that UNIVERSITY is subject to the Missouri Sunshine Act, 610 RSMo, and that all plans and reports marked "Confidential" shall be treated by UNIVERSITY as confidential to the extent permitted by law.

Article V.DUE DILIGENCE

Section 5.01LICENSEE shall use reasonable efforts to effect introduction of the LICENSED PRODUCT into the commercial market as soon as practicable, consistent with sound and reasonable business practices and judgment; thereafter, until the expiration or termination of this AGREEMENT, LICENSEE shall keep LICENSED PRODUCT reasonably available to the public.

Section 5.02UNIVERSITY shall have the right, at UNIVERSITY's sole discretion, to either terminate or render this license nonexclusive in an individual LICENSED FIELD and/or individual country or countries within the LICENSED TERRITORY if LICENSEE or its SUBLICENSEE (if applicable):

(a) Has not within [**] of the EFFECTIVE DATE [**], or

(b) Has not within [**] of the EFFECTIVE DATE [**], or

(c) Has not within [**] of the EFFECTIVE DATE [**].

If LICENSEE believes that it will not achieve one of the foregoing milestones, it may notify UNIVERSITY in writing in advance of the relevant deadline (a "DELAYED MILESTONE NOTICE"), which notice shall include (a) a reasonable explanation of the reasons for such failure and (b) a reasonable, detailed, written plan for promptly achieving a reasonable extended and/or amended milestone. If LICENSEE provides UNIVERSITY with a DELAYED MILESTONE NOTICE that is acceptable to UNIVERSITY in its sole discretion as documented in writing, then this Section 5.02 will be amended automatically to incorporate the extended and/or amended milestones set forth in the DELAYED MILESTONE NOTICE. If LICENSEE provides UNIVERSITY with a

DELAYED MILESTONE NOTICE that is not acceptable to UNIVERSITY in its sole discretion, then UNIVERSITY may either (1) proceed with such termination or modification of this AGREEMENT or (2) negotiate revised milestones with LICENSEE.

Article VI.INDEMNITY, INSURANCE, WARRANTIES, DAMAGES

Section 6.01Indemnity. LICENSEE shall, and will require SUBLICENSEES to, at all times during the term of this AGREEMENT and thereafter, indemnify, defend and hold UNIVERSITY, its current or former Curators, officers, employees and affiliates, harmless from any claim, proceeding, suit, demand, expense, loss, penalty, judgment, or liability of any kind whatsoever, including costs, expenses and reasonable attorneys' fees, resulting from, related to, arising out of, or in connection with (1) the design, development, production, manufacture, shipping, use, importation, SALE, advertisement, labeling, promotion, or patent marking of the LICENSED PRODUCT by LICENSEE or its SUBLICENSEES, or end users, including but not limited to (i) any infringement or misappropriation of a patent, copyright, trade secret or other intellectual property or proprietary right of any third party or (ii) any product liability claims, such as those involving the death of or injury to any person or persons or damage to property; or (2) any breach of any obligation, covenant, representation, or warranty by LICENSEE or its SUBLICENSEES hereunder; or (3) the production, use or SALE of any product, process or service identified, characterized or otherwise developed with the aid of the PATENT RIGHTS by LICENSEE or its SUBLICENSEES; or (4) a breach or violation of applicable law by LICENSEE, or its SUBLICENSEES; or (5) the exercise of LICENSEE's or SUBLICENSEE's rights under this AGREEMENT. If any such claims or causes of action are made, UNIVERSITY shall be defended by counsel selected by LICENSEE, subject to UNIVERSITY's approval, which shall not be unreasonably withheld. UNIVERSITY reserves the right to be represented by its own counsel at its own expense.

Section 6.02Insurance. At such time as any LICENSED PRODUCT is being commercially distributed or SOLD (other than for the purpose of obtaining regulatory approvals) by LICENSEE, a SUBLICENSEE, or a subsidiary or agent of LICENSEE, LICENSEE shall at its sole cost and expense, procure and maintain commercial general liability insurance in amounts not less than [**] per incident and naming UNIVERSITY, its Curators, trustees, officers, agents, employees and affiliates, as additional insureds. Such commercial general liability insurance shall provide (i) product liability coverage and (ii) broad form contractual liability coverage for LICENSEE's indemnification under this AGREEMENT. Such insurance will be considered primary as to any other valid and collectible insurance, but only as to acts of the named insured. Any carrier providing coverage shall have a minimum "Best" rating of "A-XII". The minimum amounts of insurance coverage required shall not be construed to create a limit of LICENSEE's liability with respect to its indemnification under this AGREEMENT.

LICENSEE shall maintain such commercial general liability insurance beyond the expiration or termination of this AGREEMENT during (i) the period that any product, process, or service, relating to, or developed pursuant to this AGREEMENT is being commercially distributed or SOLD by LICENSEE or its SUBLICENSEE and (ii) a reasonable period after the period referred to in (i) above which in no event shall be less than [**].

LICENSEE shall provide Workers' Compensation coverage for any employee of LICENSEE that visits UNIVERSITY premises for matters relating to this AGREEMENT. In addition, Employers' Liability coverage shall be provided to such employee in an amount no less than [**] per occurrence.

LICENSEE shall provide UNIVERSITY with written evidence of the insurance requirements of this Section 6.02 within thirty (30) days after such insurance becomes necessary pursuant to this AGREEMENT. LICENSEE shall provide UNIVERSITY with written notice at least fifteen (15) days prior to the cancellation, non-renewal or material change in such insurance; if LICENSEE does not obtain replacement insurance providing comparable coverage within such fifteen (15) day period, UNIVERSITY shall have the right to terminate this AGREEMENT effective at the end of such fifteen (15) day period without notice or any additional waiting periods. It is agreed that the insurance required is required in the public interest and UNIVERSITY does not assume any liability for acts of LICENSEE, their officers, agents, and employees or of a SUBLICENSEE, their officers, agents, and employees, in connection with the granting of this AGREEMENT.

If LICENSEE elects to self-insure all or part of the limits described above, such self-insurance program must be acceptable to UNIVERSITY's Risk and Insurance Management department.

Section 6.03Disclaimer of Warranties. THE PATENT RIGHTS ARE DELIVERED "AS IS" IN EVERY RESPECT. UNIVERSITY, ITS CURRENT OR FORMER CURATORS, OFFICERS, EMPLOYEES, AND AFFILIATES MAKE NO REPRESENTATIONS AND EXTEND NO WARRANTIES OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING BUT NOT LIMITED TO WARRANTIES OF COMMERCIAL UTILITY, MERCHANTABILITY, FITNESS FOR ANY PARTICULAR PURPOSE, THE ABSENCE OF LATENT OR OTHER DEFECTS, WHETHER OR NOT DISCOVERABLE, THE SCOPE, VALIDITY OR ENFORCEABILITY OF THE PATENT RIGHTS, WHETHER ISSUED OR PENDING, OR THAT THE MANUFACTURE, USE, IMPORTATION OR SALE OF THE LICENSED PRODUCT OR THAT THE PRACTICE OF THE PATENT RIGHTS WILL NOT INFRINGE OR MISAPPROPRIATE ANY PATENT, COPYRIGHT, TRADEMARK, OR OTHER RIGHTS OF ANY THIRD PARTY.

Section 6.04Damages Exclusion / Limitation of Remedies. IN NO EVENT SHALL UNIVERSITY ITS CURRENT OR FORMER CURATORS, OFFICERS, EMPLOYEES, AND AFFILIATES BE LIABLE FOR INCIDENTAL, CONSEQUENTIAL, SPECIAL, EXEMPLARY, OR PUNITIVE DAMAGES OF ANY KIND, REGARDLESS OF THE FORM OF ACTION, WHETHER IN CONTRACT OR IN TORT, INCLUDING NEGLIGENCE OR OTHERWISE, AND INCLUDING ECONOMIC DAMAGE OR INJURY TO PROPERTY AND LOST PROFITS, ATTORNEYS' AND EXPERTS' FEES, REGARDLESS OF WHETHER UNIVERSITY MAY BE ADVISED, MAY HAVE OTHER REASON TO KNOW, OR IN FACT SHALL KNOW OF THE POSSIBILITY.

Section 6.05For the avoidance of doubt, nothing in this AGREEMENT shall be construed as:

a. a warranty or representation by UNIVERSITY as to the validity or scope of any PATENT RIGHTS;

b. a warranty or representation by UNIVERSITY that anything made, used, imported, SOLD or otherwise disposed of pursuant to any license granted under this AGREEMENT is or will be free from infringement of intellectual property rights of third parties;

c. an obligation by UNIVERSITY to bring or prosecute actions or suits against third parties for patent infringement;

d. an obligation to furnish any know-how not provided in the PATENT RIGHTS; or

e. conferring by implication, estoppel or otherwise any license or rights under any patents of UNIVERSITY other than PATENT RIGHTS, regardless of whether such patents are dominant or subordinate to the PATENT RIGHTS.

Section 6.06Sublicenses. LICENSEE shall require in any sublicense in which LICENSEE grants to a third party the right to make, have made, use, import, offer to SELL or SELL any LICENSED PRODUCT, provisions that provide UNIVERSITY, its Curators, trustees, officers, agents, employees and affiliates, comparable protections as those provided UNIVERSITY in this

Article VI. LICENSEE shall not, and shall require that its SUBLICENSEES do not, make any statements, representations or warranties whatsoever to any person or entity, or accept any liabilities or responsibilities whatsoever from any person or entity that are inconsistent with any disclaimer of warranties or damages exclusion / limitation of remedies included in this Article VI.

Article VII.DOMESTIC AND FOREIGN PATENT FILING AND MAINTENANCE

Section 7.01Ownership and Control of Patents. UNIVERSITY shall have full, complete and sole ownership of any pending applications and issued patents included in PATENT RIGHTS. UNIVERSITY shall be responsible for the preparation, filing, prosecution and maintenance of the patent applications and issued patents included in the PATENT RIGHTS. UNIVERSITY, either directly or through its attorneys at UNIVERSITY's option, shall first consult with LICENSEE or its attorneys as to the preparation, filing, prosecution, and maintenance of such patent applications and issued patents and shall furnish to LICENSEE or its attorneys copies of significant documents it sends or receives relevant to any such preparation, filing, prosecution or maintenance. LICENSEE shall cooperate with UNIVERSITY in such preparation, filing, prosecution, and maintenance. LICENSEE agrees to hold such information confidential and to use the information provided by UNIVERSITY only for the purpose of advancing the PATENT RIGHTS and shall return all such information to UNIVERSITY upon termination of LICENSEE's rights in any particular patent application or issued patent under Section 7.04 or upon termination or expiration of this AGREEMENT.

Section 7.02Patent Expenses. LICENSEE shall reimburse UNIVERSITY for all out-of-pocket expenses, costs, and attorneys' fees UNIVERSITY has incurred for the preparation, filing, prosecution and maintenance of PATENT RIGHTS (hereinafter "PATENT EXPENSES") as a separate payment apart from any royalties or other revenues owed UNIVERSITY. For PATENT EXPENSES incurred prior to the EFFECTIVE DATE, such reimbursement by LICENSEE shall be due and payable when this agreement is fully executed. For all future PATENT EXPENSES

incurred after the EFFECTIVE DATE, reimbursements by LICENSEE shall be due within thirty (30) days of receipt of UNIVERSITY's invoice by LICENSEE, and shall be non-refundable and non-creditable. Late payment of invoices of PATENT EXPENSES received by LICENSEE from UNIVERSITY shall be subject to interest charges of [**].

Section 7.03Entity Status. LICENSEE shall have a continuing obligation to keep UNIVERSITY and its patent counsel responsible for the PATENT RIGHTS informed of the entity status (large entity, small entity, and micro entity) of LICENSEE and all its SUBLICENSEES. LICENSEE agrees to give UNIVERSITY prompt notice of a change in any entity status of it or any SUBLICENSEE. A statement or future statement by LICENSEE and/or its SUBLICENSEE as to its entity status constitutes a representation that is subject to indemnity under Section 6.01.

Section 7.04Termination of Patent Rights. By written notification to UNIVERSITY at least sixty (60) days in advance of any filing or response deadline or fee due date (i.e., a date by which an action must be taken to avoid payment of a late fee), LICENSEE may elect not to have a particular patent application filed in a particular country or not to pay expenses associated with prosecuting or maintaining any particular patent application or issued patent, provided that LICENSEE pays for all PATENT EXPENSES associated with the particular patent application or issued patent incurred up to UNIVERSITY's receipt of such notification. LICENSEE's failure to provide a timely notification shall be considered by UNIVERSITY to be LICENSEE's consent that it expressly wishes to support any particular issued patent(s) or patent application(s). Upon notice that LICENSEE elects not to have a particular patent application filed or prosecuted or issued patent maintained in any particular country, or not to reimburse UNIVERSITY for all PATENT EXPENSES associated with prosecuting or maintaining any patent application or patent, UNIVERSITY may at its sole discretion elect to file, prosecute, and/or maintain such particular patent applications or issued patents at its own expense and for its own benefit, and any rights or license granted under this AGREEMENT held by LICENSEE or SUBLICENSEE(s) with respect to such patent application(s) or issued patent(s) shall be irrevocably terminated, forfeited, and relinquished. For the avoidance of doubt, LICENSEE and each SUBLICENSEE shall have no right to share in any revenue derived from such particular patent application or issued patents.

Article VIII.INFRINGEMENT OF PATENT RIGHTS

Section 8.01Notifications. LICENSEE shall promptly inform UNIVERSITY in writing of any alleged infringement of the PATENT RIGHTS by a third party and shall provide UNIVERSITY with any available evidence thereof. LICENSEE shall not notify a third party of such infringement of PATENT RIGHTS without first consulting with UNIVERSITY.

Section 8.02Enforcement. For so long as the license granted herein is exclusive, LICENSEE, at its expense, shall have the right to enforce PATENT RIGHTS against infringement by third parties. All recovery from any enforcement of the PATENT RIGHTS, including any cash or other consideration received by way of judgment, settlement or compromise (hereinafter "RECOVERY") shall be allocated in the following order: (a) to LICENSEE and UNIVERSITY for reimbursement in pro rata proportions of their costs, fees, and other related expenses to the extent that each PARTY paid for such costs, fees and expenses; and (b) any remaining amount

shall be shared between UNIVERSITY and LICENSEE in the same proportion as if such remaining RECOVERY constituted SUBLICENSE REVENUE based on the time of infringement, and if that cannot be reasonably determined to the mutual satisfaction of the PARTIES, [**] to UNIVERSITY and [**] to LICENSEE. Before LICENSEE commences a formal legal proceeding with respect to any infringement of PATENT RIGHTS, LICENSEE shall first consult with UNIVERSITY regarding the potential effects such legal proceeding may have on the public interest. UNIVERSITY shall have the right, in its sole discretion, to join such proceeding at its own expense. In the event that UNIVERSITY is involuntarily joined as a party to an infringement action brought by LICENSEE (including any counterclaim), then LICENSEE shall pay any costs, expenses, and attorneys' fees incurred by UNIVERSITY arising out of, relating to, or in connection therewith. In addition, LICENSEE agrees to consult with UNIVERSITY on any significant matters related to the litigation. LICENSEE shall be free to enter into a settlement, consent judgment, or other voluntary disposition with respect to any such action, provided that any settlement, consent judgment or other voluntary disposition thereof which (i) materially limits the scope, validity, or enforceability of patents included in the PATENT RIGHTS or (ii) admits fault or wrongdoing on the part of UNIVERSITY must be pre-approved in writing by UNIVERSITY. LICENSEE shall keep UNIVERSITY informed on all actions taken by LICENSEE in its enforcement against an infringer and shall furnish to UNIVERSITY copies of all documents related thereto. LICENSEE shall indemnify, defend, and hold harmless UNIVERSITY against any order for costs or fees that may be made against UNIVERSITY in such proceeding arising from, related to, or in connection with an act or omission made by LICENSEE.

Section 8.03Rights of University. In the event that LICENSEE elects not to exercise its right to bring an infringement action with respect to PATENT RIGHTS pursuant to the above paragraphs, then LICENSEE shall notify UNIVERSITY in writing within six (6) months of receiving notice that an infringement exists. UNIVERSITY may, at its own expense and control, following the earlier of (i) such notice from LICENSEE or (ii) the expiration of such six (6) month period without LICENSEE electing to take any action with respect to such alleged or actual infringement, take steps to defend or enforce any patent within the PATENT RIGHTS and retain all RECOVERY therefrom without a duty to account to LICENSEE. LICENSEE agrees to cooperate reasonably with UNIVERSITY in any such infringement suit or dispute.

Article IX.CONFIDENTIALITY

Section 9.01Confidential Information Defined. "CONFIDENTIAL INFORMATION" means any and all information not generally known to the public, whether or not patentable or susceptible to any other form of legal protection, that is identified or designated by UNIVERSITY as being confidential or which, in light of the circumstances under which it was disclosed, whether oral or written, is reasonably apparent to LICENSEE to be considered confidential or proprietary by UNIVERSITY, including but not limited to invention disclosures, non-public patent prosecution information, including but not limited to concepts, designs, processes, specifications, schematics, equipment, processing, techniques, technical information, drawings, diagrams, software (including source code), hardware, control systems, research, test results, manuals, trade secrets, commercialization studies, market studies, business plans received by LICENSEE from UNIVERSITY except to the extent LICENSEE can prove by written documentation that such information:

(i) was in the public domain at the time of disclosure;

(ii) later became part of the public domain through no act or omission or breach of this AGREEMENT by LICENSEE, its employees, agents, successors or assigns;

(iii) was lawfully disclosed to LICENSEE by a third party having the right to make such disclosure; or

(iv) was already known by LICENSEE at the time of disclosure; or

(v) was independently developed by LICENSEE without the aid, use or application of CONFIDENTIAL INFORMATION received from UNIVERSITY and such independent development can be properly demonstrated by LICENSEE.

Specific information shall not be deemed to be within the foregoing exceptions merely because it is embraced by more general information within the exceptions. In addition, any combination of the features shall not be deemed to be within the foregoing exception merely because individual features may be within the exceptions.

Section 9.02Restrictions on Disclosure and Use. LICENSEE agrees that (a) all CONFIDENTIAL INFORMATION shall remain the exclusive property of UNIVERSITY, (b) LICENSEE shall receive and hold the CONFIDENTIAL INFORMATION in strict confidence, (c) LICENSEE shall use the CONFIDENTIAL INFORMATION only for the purposes of this AGREEMENT, and (d) LICENSEE shall not disclose the CONFIDENTIAL INFORMATION to third parties without the prior written consent of UNIVERSITY, and (e) LICENSEE shall protect the CONFIDENTIAL INFORMATION to the same extent that it protects its own trade secrets and confidential information, but in no less than commercially reasonable care.

Section 9.03Legally required Disclosures. In the event that LICENSEE receives a request or is required by deposition, open records request, interrogatory, request for documents, subpoena, civil investigative demand, open records request, or similar process to disclose any or part or the CONFIDENTIAL INFORMATION, LICENSEE agrees to (a) immediately notify UNIVERSITY in writing of the existence, terms, and circumstances surrounding such a request or requirement and (b) assist UNIVERSITY in seeking a protective order or other appropriate remedy satisfactory to UNIVERSITY. In the event that such a protective order or other remedy is not obtained, (a) LICENSEE may disclose that portion of the CONFIDENTIAL INFORMATION which it is legally required to disclose, (b) LICENSEE and shall exercise reasonable efforts to obtain assurance that confidential treatment will be accorded the CONFIDENTIAL INFORMATION to be disclosed and (c) LICENSEE shall give written notice to UNIVERSITY of the information to be disclosed as far in advance of its disclosure as practical. LICENSEE may also disclose CONFIDENTIAL INFORMATION to governmental or other regulatory agencies in order to obtain approvals to market any LICENSED PRODUCT, but such disclosure may only be to the extent reasonable necessary to obtain approvals.

Section 9.04Disclosure to Potential Sublicensee or Assignee. Upon receiving written approval from UNIVERSITY, LICENSEE may disclose the CONFIDENTIAL INFORMATION to a

potential SUBLICENSEE or assignee of LICENSEE in each case on the condition that such potential SUBLICENSEE or assignee agrees to be bound by the confidentiality obligations contained in this AGREEMENT.

Section 9.05Survival. LICENSEE's obligations of confidentiality and non-use shall exist during the term of this AGREEMENT and for so long as such CONFIDENTIAL INFORMATION remains confidential in accordance with Section 9.01.

Article X.TERM AND TERMINATION

Section 10.01Term. This AGREEMENT shall become effective upon the EFFECTIVE DATE and, unless sooner terminated in accordance with any of the provisions herein, shall remain in full force in the LICENSED TERRITORY until the expiration of the last to expire patent or last to be abandoned patent application included in the PATENT RIGHTS.

Section 10.02Right to Terminate by Licensee. LICENSEE shall have the right to terminate this AGREEMENT at any time on six (6) months notice to UNIVERSITY if LICENSEE, prior to such termination, pays a termination fee of Fifty Thousand ($50,000) dollars.

Section 10.03Breach. In the event that either PARTY defaults or breaches any of the provisions of this AGREEMENT, the other PARTY shall have the right to terminate this AGREEMENT by giving written notice to the defaulting PARTY; provided, however, that if the defaulting PARTY cures the default within thirty (30) days after the notice shall have been given, this AGREEMENT shall continue in full force and effect. The failure on the part of either of the PARTIES hereto to exercise or enforce any right conferred upon it hereunder shall not be deemed to be a waiver of any such right nor operate to bar the exercise or enforcement thereof at any time or times thereafter. In relation to Article III (payments) and Section 7.02 (patent expenses), LICENSEE'S opportunity to cure a breach shall apply only to LICENSEE'S first two notices of a breach properly given by UNIVERSITY. Upon occurrence of a third breach, UNIVERSITY may, at its option, terminate this AGREEMENT upon thirty (30) days written notice without an opportunity to cure.

Section 10.04Rights after Termination. Upon termination for any reason, LICENSEE shall:

(a) promptly pay all amounts due UNIVERSITY through the effective date of the termination (even if they would otherwise be payable at a later date, e.g. within 30 days after invoicing), including those in Article III (payments) and Section 7.02 (patent expenses);

(b) submit all final reports under Article IV;

(c) return any CONFIDENTIAL INFORMATION provided to LICENSEE by UNIVERSITY in connection with this AGREEMENT, or, with UNIVERSITY'S prior approval, destroy such materials, and LICENSEE shall certify in writing that such materials have all been returned or destroyed;

(d) provide UNIVERSITY a copy of any regulatory data or information filed with any U.S. or foreign government agency with respect to the LICENSE PRODUCT; and

(e) shall refrain, and shall require its SUBLICENSEES to refrain unless such sublicense is assigned to UNIVERSITY under Section 10.05, from any further SALES or other commercial exploitation of the LICENSED PRODUCT except as provided in Section 10.08.

Nothing in this section shall be construed as limiting in any way UNIVERSITY'S rights or remedies that UNIVERSITY may otherwise have, either in law or in equity.

Section 10.05Assignment of Sublicenses. Upon termination of this AGREEMENT, LICENSEE's interest in sublicenses granted by it under this AGREEMENT shall at UNIVERSITY's sole option, terminate or be assigned to UNIVERSITY, including the right to receive income from SUBLICENSEES. LICENSEE shall make provision for UNIVERSITY's rights under the preceding sentence to be included in all sublicenses granted by it under this AGREEMENT.

Section 10.06Insolvency. In the event that LICENSEE (or SUBLICENSEE as applicable) dissolves, liquidates, ceases to carry on business, becomes insolvent, is unable to pay its debts as they become due, makes an assignment for the benefit of creditors, or has a petition for bankruptcy filed for or against it, this AGREEMENT (or applicable SUBLICENSE) shall automatically terminate.

Section 10.07Survival. Termination of this AGREEMENT for any reason shall not release either PARTY from any obligation theretofore accrued. All provisions of this AGREEMENT that would reasonably be expected to survive the termination or expiration of this AGREEMENT shall do so, including Article III (all—payments), Article VI (all—indemnity, insurance, warranties, damages), Article IX (all—confidentiality), Section 2.03 (reserved rights), Section 2.04 (license to University); Section 2.07 (governmental rights), Section 3.07 (challenge to patent rights), Section 4.03 (reporting), Section 4.05 (records), Section 10.04 (rights after termination), Section 10.05 (assignment of sublicenses), Section 10.08 (inventory), Section 10.09 (ongoing payments), and Article XI (general—all) survive the termination of this AGREEMENT.

Section 10.08Inventory. Upon termination of this AGREEMENT or upon termination in whole or in part through no fault of LICENSEE, LICENSEE shall provide UNIVERSITY with a written inventory of all LICENSED PRODUCTS in the possession or under the control of LICENSEE (including any in the process of manufacture). Except with respect to termination for uncured breach by LICENSEE, LICENSEE shall have the privilege of SELLING the inventory of such LICENSED PRODUCTS within a period of one hundred and eighty (180) days of such termination upon conditions most favorable to UNIVERSITY that LICENSEE can reasonably obtain and paying any applicable royalties associated with such SALES to UNIVERSITY.

Section 10.09Ongoing Payments. Any termination or cancellation under any provision of this AGREEMEMT shall not relieve LICENSEE of its obligation to pay any royalty or other fees due to UNIVERSITY at the time of such termination or cancellation.

Article XI.GENERAL

Section 11.01Marking. Prior to the issuance of patents under PATENT RIGHTS, LICENSEE agrees to mark LICENSED PRODUCTS (or their containers or labels) SOLD by LICENSEE or SUBLICENSEES under the license granted in this AGREEMENT with the words "Patent Pending" and following the issuance of one or more patents under PATENT RIGHTS, with the words "Patent No. "or in such a manner as to conform with the patent laws and practice of the country of manufacture, SALE, or importation.

Section 11.02Compliance with Laws: Export Controls. LICENSEE agrees to comply with all applicable federal, state, and local laws and regulations. In particular, LICENSEE shall comply with all applicable U.S. laws dealing with the export and/or management of commodities, technology or information, and that LICENSEE will be responsible for any violation of such by LICENSEE or its SUBLICENSEES, and that it will defend and hold UNIVERSITY harmless in the event of any legal action of any nature occasioned by such violation. LICENSEE understands that the Arms Export Control Act (AECA), including its implementing International Traffic In Arms Regulations (ITAR,) and the Export Administration Act (EAA), including its Export Administration Regulations (EAR), are some (but not all) of the laws and regulations that comprise the U.S. export laws and regulations. LICENSEE further understands that the U.S. export laws and regulations include (but are not limited to): (1) ITAR and EAR product/service/data-specific requirements; (2) ITAR and EAR ultimate destination-specific requirements; (3) ITAR and EAR end user-specific requirements; (4) ITAR and EAR end use-specific requirements; (5) Foreign Corrupt Practices Act; and (6) anti-boycott laws and regulations. LICENSEE will comply with all then-current applicable export laws and regulations of the U.S. Government (and other applicable U.S. laws and regulations) pertaining to the LICENSED PRODUCTS (including any associated products, items, articles, computer software, media, services, technical data, and other information). LICENSEE warrants that it will not, directly or indirectly, export (including any deemed export), nor re-export (including any deemed re-export) the LICENSED PRODUCT (including any associated products, items, articles, computer software, media, services, technical data, and other information) in violation of U.S. export laws and regulations or other applicable U.S. laws and regulations. LICENSEE will include an appropriate provision in its agreements with its authorized SUBLICENSEES to assure that these parties comply with all then-current applicable U.S. export laws and regulations and other applicable U.S. laws and regulations. LICENSEE'S OBLIGATIONS TO COMPLY WITH U.S. EXPORT CONTROL LAWS AND REGULATIONS ARE INDEPENDENT OF AND SURVIVE THE TERMINATION OF THIS AGREEMENT.

Section 11.03University Name. LICENSEE agrees not to identify UNIVERSITY in any promotional advertising or other promotional materials to be disseminated to the public or any portion thereof or to use the name of any UNIVERSITY faculty member, employee, or student or any trademark, service mark, trade name, or symbol of UNIVERSITY, without UNIVERSITY'S prior written consent.

Section 11.04Press. Notwithstanding Section 11.03, UNIVERSITY may disclose the existence of this AGREEMENT and non-confidential information regarding the status of LICENSEE's commercialization of LICENSED PRODUCTS in a press release, on-line, or otherwise, and on the UNIVERSITY'S website.

Section 11.05Assignment. This AGREEMENT is binding upon and shall inure to the benefit of UNIVERSITY, its successors and assigns. However, this AGREEMENT shall be personal to LICENSEE, and it is not assignable by LICENSEE to any other person or entity without the prior written consent of UNIVERSITY, such consent to be in UNIVERSITY's sole discretion, except in connection with a sale of LICENSEE or the business of LICENSEE to which this Agreement relates to a third party, whether by merger, consolidation, sale of all or substantially all of LICENSEE's assets or capital stock, which consent will not be unreasonably withheld. Any purported sale, transfer or assignment without UNIVERSITY's prior written consent shall be void ab initio, and this AGREEMENT shall immediately terminate. For purposes of this Section, "transfer" shall include any transfer by operation of law or otherwise.

Section 11.06Sponsored Research. If LICENSEE desires UNIVERSITY participation in performing research and development activities directed towards PATENT RIGHTS, negotiation for such assistance shall be separate and apart from this AGREEMENT, and shall be performed according to UNIVERSITY'S procedures related to research grant and contract activities.

Section 11.07Consulting. In the event LICENSEE wishes to engage the inventors as consultants, such an arrangement shall be separate and apart from this AGREEMENT, but shall be in keeping with UNIVERSITY'S policy on consulting and ownership of intellectual property developed by UNIVERSITY employees.

Section 11.08Notices. Any payment, notice, or other communication given under this AGREEMENT except for correspondence relating to patent preparation, filing, prosecution and/or maintenance matters under Article VII herein) shall be in writing and shall be deemed delivered when sent by certified first class mail, registered mail, or overnight courier, or by facsimile, provided that a copy of such facsimile is promptly sent by certified first class mail, registered or overnight courier, addressed to the PARTIES as follows (or at such other addresses as the PARTIES may notify each other in writing):

If to UNIVERSITY: Office of Technology Management & Industry Relations
University of Missouri, Missouri Innovation Center

If to LICENSEE:

Solid GT
One Broadway
Cambridge, MA 02142

Section 11.09No Other Relationship. In assuming and performing the respective obligations under this AGREEMENT, LICENSEE and UNIVERSITY are each acting as independent parties and neither shall be considered or represent itself as a joint venture, partner, agent or employee of the other.

Section 11.10No Waiver. None of the terms, covenants, and conditions of this AGREEMENT can be waived except by the written consent of the PARTY waiving compliance. A failure by one of the PARTIES to this AGREEMENT to assert its rights for or upon any breach or default of this AGREEMENT shall not be deemed a waiver of such rights nor shall any such waiver be implied from acceptance of any payment. No such failure or waiver in writing by any one of the PARTIES hereto with respect to any rights, shall extend to or affect any subsequent breach or impair any right consequent thereon.

Section 11.11Severability. If any sentence, paragraph, clause or combination of the same is found by a court of competent jurisdiction to be in violation of any applicable law or regulation, or is unenforceable or void for any reason whatsoever, such sentence, paragraph, clause or combinations of the same shall be severed from the AGREEMENT and the remainder of the AGREEMENT shall remain binding upon the PARTIES.

Section 11.12Headings. The headings of the paragraphs of this AGREEMENT are inserted for convenience only and shall not constitute a part hereof.

Section 11.13Choice of Law and Venue. This AGREEMENT shall be construed, interpreted, and applied in accordance with the laws of the State of Missouri. Any action to enforce the provisions of the AGREEMENT shall be brought in a court of competent jurisdiction and proper venue in the State of Missouri. LICENSEE irrevocably submits to the jurisdiction of such courts in any such action or proceeding. LICENSEE further irrevocably and unconditionally waives any objection to the laying of venue of any suit, action or proceeding in such courts and irrevocably waives and agrees not to plead or claim in any court that such suit, action or proceeding brought in any such court has been brought in an inconvenient forum.

Section 11.14Sovereign Immunity. The PARTIES agree that nothing in this AGREEMENT is intended or shall be construed as a waiver, either express or implied, of any of the immunities, lights, benefits, defenses or protections provided to UNIVERSITY under governmental or sovereign immunity laws from time to time applicable to UNIVERSITY.

Section 11.15Counterparts. This AGREEMENT may be executed in two or more counterparts, each of which shall be deemed an original and all of which together shall constitute but one and the same instrument.

Section 11.16Entire Agreement. This AGREEMENT constitutes the entire and only agreement between the PARTIES for PATENT RIGHTS and all other prior negotiations, representations, agreements, and understandings are superseded hereby. No agreements altering or supplementing the terms hereof may be made except by a written document signed by both PARTIES.

IN WITNESS WHEREOF, the PARTIES hereto have executed this AGREEMENT in duplicate originals by their duly authorized officers or representatives.

THE CURATORS OF THE UNIVERSITY OF MISSOURI		LICENSEE	
BY:	/s/ Christopher Fender	BY:	/s/ Ilan Ganot
NAME:	Christopher Fender	NAME:	ILAN GANOT
TITLE:	Director, Office of Technology Management and Industry Relations	TITLE:	CEO
DATE	October 15 2015	DATE	October 15 2015

EXECUTIVE CHAIR AGREEMENT

This Executive Chair Agreement (together with Exhibit A, the "Agreement"), effective January 1, 2022 (the "Effective Date"), is by and between Ian F. Smith, an individual having an address at 45 Commonwealth Avenue, Unit 3, Boston, MA 02116 (the "Executive Chair"), and Solid Biosciences Inc., a Delaware corporation (together with its affiliates, the "Company"), having an address at 141 Portland Street, Fifth Floor, Cambridge, MA 02139.

WHEREAS, Executive Chair is currently serving as a member and chairman of the Board of Directors of the Company (the "Board");

WHEREAS, Executive Chair has also been providing advisory services to Company pursuant to a consulting agreement between the Parties entered into on January 4, 2021 (the "Prior Agreement") and both parties acknowledge and agree that, as of the Effective Date of this Agreement, the agreed upon services under the Prior Agreement have been completed;

WHEREAS, from and after the close of business on the Effective Date, Executive Chair shall no longer serve under the Prior Agreement, but shall be retained as the Executive Chairman pursuant to the terms and conditions set forth herein; and

NOW THEREFORE, in consideration of the promises and mutual covenants herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, Executive Chair and the Company agree as follows:

1. Services. Company retains Executive Chair, and Executive Chair agrees to provide, part-time advisory services to Company, consistent with his position as Executive Chairman of the Board, and as more fully set forth on Exhibit A attached hereto (the "Services"). It is understood and agreed that the Executive Chair will not be an executive officer of the Company and that Chief Executive Officer of the Company will continue to be the Company's principal executive officer and will continue to report directly to the board of directors and to the Executive Chairman.

2. Compensation. As consideration for Services provided under this Agreement, the Company shall grant, subject to approval of Company's Board, to Executive Chair an option to purchase 194,500 shares of the Company's Common Stock ("Stock Options") at an exercise price per share equal to the closing price of the Company's Common Stock on January 3, 2021 (the "Grant Date"), and 97,250 restricted stock units (the "RSUs" and together with the RSUs, the "Awards"). The Awards will vest in equal quarterly installments with the first installment vesting three months from the Grant Date and the final installment vesting date on the date that is 12 months from the Grant Date, subject to continued services as a Director of the Company. Additionally, in the event of a change in control of the Company and on terms consistent with board of director equity award agreements, all unvested Awards will accelerate and vest in full.

3. Work-Made-for Hire.

(a) All right, title and interest in any and all writings, ideas, inventions, know-how, designs, improvements or other property created during Executive Chair's advisory relationship relating in any way to the assets, business or operations of the Company, constituting copyrights, patents, trademarks, service marks and related rights or other forms of proprietary rights or information (regardless of whether any such copyrights, patents, trademarks and service marks or other rights have or may be registered) that are created, adapted or improved by Executive Chair (whether alone or in conjunction with any other person or employee), and all material created during Executive Chair's advisory relationship that includes any of the foregoing (collectively, "Covered Material"), shall be owned by the Company and to the extent that it includes copyrightable subject matter, shall be deemed a work made for hire for the Company within the meaning of the United States Copyright Act of 1976 and for all other purposes. If any Covered Material is deemed not to be work made for hire, such Covered Material is hereby assigned by Executive Chair to the Company and Executive Chair shall not have or claim to have, under this Agreement or otherwise, any right, title or interest of any kind or nature whatsoever in such Covered Material.

(b) The Company shall have the right to apply for and obtain registrations in the United States Copyright Office and the United States Patent and Trademark Office, in its own or its designee's name, of its rights in any or all of the Covered Material. If for any reason the rights in any Covered Material are registered, or applied to be registered, in Executive Chair's name, Executive Chair shall assign in writing such application or registration to the Company and hereby authorizes and appoints the Company its agent for the purpose of recording such assignment.

(c) Upon request of Company, Executive Chair shall execute, acknowledge and deliver all applications, assignments or other instruments; make or cause to be made all rightful oaths; testify in all legal proceedings; communicate all known facts which relate to such works, copyrights, inventions, ideas, discoveries, designs and improvements; perform all lawful acts and otherwise render all such assistance as the Company may deem necessary to protect the Company's interest therein including any assistance which the Company shall deem necessary in connection with any proceeding or litigation involving the same. The Company shall reimburse Executive Chair for all reasonable out-of-pocket costs, incurred by Executive Chair in rendering any such assistance requested by the Company pursuant to this Section. In the event that Executive Chair should fail or refuse to execute such documents within a reasonable time, Executive Chair appoints Company as attorney to execute and deliver any such documents on Executive Chair's behalf.

4. Nondisclosure. Executive Chair agrees to (a) hold the Confidential Information (as defined below) in confidence; (b) not disclose any Confidential Information to any third party without the prior written consent of Company; (c) not use the Confidential Information for any purpose except solely as required to perform the Services; and (d) treat Confidential Information with no less than a reasonable degree of care. For purposes of this Agreement, the term "Confidential Information" means any and all information and derivative information, in whatever form or medium, including oral information, concerning or relating to the Company or information of any third party that the Company is under an obligation to keep confidential or that is maintained by the Company as confidential, including, without limitation, intellectual property of the Company, such as, but not limited to, Covered Material, patent applications, copyrights, copyright

applications, and trade secrets; information regarding or resulting from research and development activities performed by or on behalf of the Company and other projects (such as, but not limited to, preclinical and clinical data, design details and specifications, engineering information, and works in process); and business and financial information (such as, but not limited to, current, future, and proposed products and services, financial information and models, information relating to procurement requirements, purchasing, manufacturing, investors, customer lists, customers, suppliers, facilities, product plans, product ideas, business strategies, marketing or business plans, financial or personnel matters, investors, employees, business and contractual relationships, business forecasts, sales, strategies, operations, policies, procedures, commercialization capabilities, and information regarding third parties). Notwithstanding the foregoing, Confidential Information does not include information that Executive Chair can demonstrate: (a) is publicly known and generally available in the public domain other than in consequence of improper action by any person; or (b) was acquired by Executive Chair free and clear of any duty of confidentiality or restricted use and without improper action by the transferor of such information or any other person. Executive Chair shall keep confidential all matters entrusted to Executive Chair by or on behalf of the Company and shall not use or attempt to use any Confidential Information except as may be required in the ordinary course of performing Executive Chair's duties as an advisor to the Company, and Executive Chair shall not use any Confidential Information in any manner that may injure or cause loss or may be calculated to injure or cause loss to the Company, whether directly or indirectly. Nothing in this Agreement prohibits Executive Chair from reporting possible violations of federal or state law or regulations to any governmental agency or entity or self-regulatory institution, including but not limited to the Equal Employment Opportunity Commission, the National Labor Relations Board, the Department of Justice, the Securities and Exchange Commission, Congress, and any Inspector General, or making other disclosures that are protected under the whistleblower provisions of federal or state law or regulation. Prior authorization of the Company shall not be required to make any such reports or disclosures and Executive Chair is not required to notify the Company that Executive Chair has made such reports or disclosures.

5. Compliance with Laws. Executive Chair agrees to provide the Services to Company and its affiliates in accordance with all applicable laws and regulations and the highest professional standards. Executive Chair represents and warrants that Executive Chair has not been, and is not under consideration to be (a) debarred from providing services pursuant to Section 306 of the United States Federal Food Drug and Cosmetic Act, 21 U.S.C. § 335a; (b) excluded, debarred or suspended from, or otherwise ineligible to participate in, any federal or state health care program or federal procurement or non-procurement programs (as that term is defined in 42 U.S.C. § 1320a-7b(f)); (c) disqualified by any government or regulatory agencies from performing specific services, and is not subject to a pending disqualification proceeding; or (d) convicted of a criminal offense related to the provision of health care items or services, or under investigation or subject to any such action that is pending.

6. Compliance with Obligations to Third Parties. Executive Chair represents and warrants to Company that the terms of this Agreement and Executive Chair's performance of Services do not and will not conflict with any of Executive Chair's obligations to any third parties. Executive Chair represents that Executive Chair has not brought and will not bring with Executive Chair to Company or use in the performance of Services any equipment, funds, space, personnel, facilities, confidential information, trade secrets or other resources of any third party which are not generally available to the public, nor will Executive Chair take any other action that would result in a third-party asserting ownership of, or other rights in, any Covered Material. If Executive Chair is a faculty member at or employee of a university or hospital or another organization or company ("Institution"), Executive Chair represents and warrants that Executive Chair is not prohibited by any applicable policy of such Institution, including without limitation any policy addressing conflicts of interest or intellectual property, from performing external consulting services and assigning rights to intellectual property arising from such services to Company or a third party. To the extent Executive Chair is subject to any policy of his/her employer that requires approval of agreements governing external consulting services, Executive Chair represents that such approval has been given and covenants that such approval will be obtained prior to entering into any amendment to this Agreement requiring such approval.

7. Non-hire of Employees and Consultants. During the Term and for a one year period thereafter, Executive Chair will not (except on the Company's behalf), directly or indirectly, alone or as a consultant, partner, officer, director, employee, joint venturer, lender or stockholder of any entity, employ, hire, retain, attempt to employ, hire or retain, or knowingly permit any company or business organization by which Executive Chair is employed or which is directly or indirectly controlled by Executive Chair to employ, hire or retain, any Company employee or consultant, or any such person whose employment or consultancy with the Company has terminated within six months prior to or after Executive Chair's departure from the Company.

8. Nonsolicitation of Employees and Consultants. During the Term and for a one year period thereafter, Executive Chair will not (except on the Company's behalf), directly or indirectly, alone or as a consultant, partner, officer, director, employee, joint venturer, lender or stockholder of any entity, in any manner seek to solicit or induce any Company employee or consultant, or any such person whose employment or consultancy with the Company has terminated within six months prior to or after Executive Chair's departure from the Company, to leave his or her employment or consultancy with the Company, or assist in the recruitment or hiring of any such person.

9. Nondisparagement. Executive Chair shall not at any time, whether during or after the Term, regardless of the reason for such termination, make to any person or entity disparaging, critical or otherwise detrimental comments of a business or personal nature relating to the Company or its personnel.

10. Use of Name. Executive Chair will not use the name, logo, trade name, service mark, or trademark, or any simulation, abbreviation, or adaptation of same, or the name of Company or any of its affiliates for publicity, promotion, or other uses without Company's prior written consent.

11. Company Property. Executive Chair shall not make, use or permit to be used any Company Property otherwise than for the benefit of the Company. The term "Company Property" shall include all Confidential Information; the Company's records, files and data; all Company computers, cellular telephones, personal digital assistants, credit and/or calling cards, keys, access cards and the like; and all other documentation or materials of any nature and in any form, whether written, printed, electronic or in digital format or otherwise, relating to any matter within the scope of the business of the Company or concerning any of its dealings or affairs and any other Company property in Executive Chair's possession, custody or control. Executive Chair further agrees that upon expiration or termination of this Agreement, Executive Chair shall immediately return all Company Property to Company. Executive Chair acknowledges and agree that all Company Property shall be and remain the sole and exclusive property of the Company. Immediately upon the expiration or termination of this Agreement, Executive Chair shall deliver all Company Property in Executive Chair's possession, and all copies thereof, to the Company.

12. Term & Termination. The term of this Agreement will commence on the Effective Date and will expire on December 31, 2022, unless extended by mutual written agreement of the parties or earlier terminated in accordance with this Section 12 (the "Term"). Notwithstanding anything to the contrary herein, the Company may terminate this Agreement, for any or no reason, on written notice to Executive Chair and Executive Chair may terminate this agreement, for any reason or no reason, on fifteen (15) days prior written notice to the Company.

13. Notices. All notices required or permitted under this Agreement must be in writing and must be given by directing the notice to the address for the receiving party set forth in this Agreement or at such other address as the receiving party may specify in writing under this procedure. Notices to Company will be marked "Attention: Chief Legal Officer." All notices must be given (i) by personal delivery, with receipt acknowledged, (ii) by prepaid certified or registered mail, return receipt requested, or (iii) by prepaid recognized next business day delivery service. Notices will be effective upon receipt or at a later date stated in the notice.

14. Remedies. Executive Chair agrees that (i) Company may be irreparably injured by a breach of this Agreement by Executive Chair; (ii) money damages would not be an adequate remedy for any such breach; (iii) as a remedy for any such breach Company will be entitled to seek equitable relief, including injunctive relief and specific performance, without being required by Executive Chair to post a bond; and (iv) such remedy will not be the exclusive remedy for any breach of this Agreement.

15. Independent Contractor. Executive Chair is an independent contractor and not an employee of the Company. Executive Chair shall be responsible for all taxes arising from compensation and other amounts paid under this Agreement. Neither federal, state or local income tax, nor payroll tax of any kind, shall be withheld or paid by the Company on Executive Chair's behalf. Executive Chair will not be eligible for, and shall not participate in, any employee pension, health, welfare, or other fringe benefit plan of the Company.

16. Waiver; Amendments. Any waiver by the Company of a breach of any provision of this Agreement shall not operate or be construed as a waiver of any subsequent breach of such provision or any other provision hereof. In addition, any amendment to or modification of this Agreement or any waiver of any provision hereof must be agreed to in writing by both parties.

17.Severability. Executive Chair agrees that each provision and the subparts of each provision herein shall be treated as separate and independent clauses, and the unenforceability of any one clause shall in no way impair the enforceability of any of the other clauses of the Agreement. Moreover, if one or more of the provisions contained in this Agreement shall for any reason be held to be excessively broad as to scope, activity, subject or otherwise so as to be unenforceable at law, such provision or provisions shall be construed by the appropriate judicial body by limiting or reducing it or them, so as to be enforceable to the maximum extent compatible with the applicable law as it shall then appear. Executive Chair hereby further agrees that the language of all parts of this Agreement shall in all cases be construed as a whole according to its fair meaning and not strictly for or against either of the parties.

18.Survival. Executive Chair's obligations under Section 3 through 22 of this Agreement shall survive the expiration or termination of this Agreement and shall be binding upon Executive Chair's successors, heirs, executors, administrators and legal representatives.

19.Assignment. The Company shall have the right to assign this Agreement to its successors and assigns, and all covenants and agreements hereunder shall inure to the benefit of and be enforceable by said successors or assigns. Executive Chair may not assign this Agreement. In no event will Executive Chair assign or delegate responsibility for actual performance of the Services to any third party.

20.Governing Law. This Agreement shall be governed by and construed in accordance with the laws of the Commonwealth of Massachusetts, without giving effect to the principles of conflicts of laws of such state. The parties agree to submit to the exclusive jurisdiction of the state and federal courts located in Commonwealth of Massachusetts and waive any defense of inconvenient forum to the maintenance of any action or proceeding in such courts.

21.Entire Agreement. This Agreement sets forth the complete, sole and entire agreement between the parties with respect to the subject matter herein and supersedes any and all other agreements, negotiations, discussions, proposals, or understandings, whether oral or written, previously entered into, discussed or considered by the parties.

22.Counterparts. This Agreement may be executed in any number of counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument. A facsimile or portable document format (".pdf") copy of this Agreement, including the signature pages, will be deemed an original.

IN WITNESS HEREOF, Executive Chair has executed this Agreement as of the last date written below.

SOLID BIOSCIENCES INC.		**IAN F. SMITH**
By:	/s/ Ilan Ganot	/s/ Ian Smith
Name:	Ilan Ganot	
Title:	President & Chief Executive Officer	

EXHIBIT A

Executive Chairman Services

The Company requires, in addition to the traditional duties as Chairman of the Board, that the Executive Chair, as Executive Chairman, will be available to perform the duties customarily related to this function, including:

- Provide mentorship and guidance to the Company's Chief Executive Officer (who will remain the Company's principal executive officer) and senior leadership team on operational and strategic matters; Chief Executive Officer will continue to report to the Board, as well as to the Executive Chair
- Act as a liaison between the Company's senior management and the Board;
- Participate in regular meetings with the Chief Executive Officer and Chief Operating Officer to further advance the Company's strategic initiatives;
- Make himself available as reasonably requested by the Board or Executive Chair officers of the Company to fulfill such other duties as may be reasonably requested, consistent with his status as the Executive Chair Chairman; and
- Devote between thirty-five to fifty (35-50) hours per month, including one (1) full day per week, on a schedule and at a location(s) mutually agreed between Executive Chair and the Company's Chief Executive Officer, to include flexible availability to the Company's Chief Executive Officer and Chief Operating Officer

FIRST AMENDMENT TO

EXECUTIVE CHAIR AGREEMENT

THIS FIRST AMENDMENT TO EXECUTIVE CHAIR AGREEMENT is effective September 30, 2022 (the "First Amendment Date"), is by and between Ian F. Smith, an individual having an address at 104 Meadowbrook Road, Weston, MA, 02493 (the "Executive Chair"), and Solid Biosciences Inc., a Delaware corporation (together with its affiliates, the "Company"), having an address at 500 Rutherford Ave., Charlestown, MA 02129.

WHEREAS, Executive Chair and the Company are parties to that certain Executive Chair Agreement ("Agreement"), effective as of January 1, 2022 (the "Effective Date"), and

WHEREAS, the parties desire to amend the Agreement in manner set forth below.

NOW, THEREFORE, in consideration of the premises and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties agree as follows:

1. Compensation. Section 2 of the Agreement is amended by inserting a new Section 2A as follows: and restated in its entirety as follows:

"2A. Compensation – 2023. As consideration for Services provided under this Agreement from and after January 1, 2023, the Company shall, subject to approval of Company's Board, grant to Executive Chair equity awards (the "2023 Awards") with an aggregate total Black Scholes value of $700,000, consisting of (i) 50% stock options (the "2023 Stock Options") at an exercise price per share equal to the closing price of the Company's Common Stock on January 2, 2023 (the "2023 Grant Date") and (ii) 50% restricted stock units ("2023 RSUs"), in each case adjusted, as applicable and appropriate, to reflect fully the effect of any reclassification, stock split, reverse split, stock dividend, reorganization, recapitalization or other like change with respect to Company Common Stock occurring (or for which a record date is established) after the date of this Amendment and prior to the 2023 Grant Date. The 2023 Awards will vest in equal quarterly installments with the first installment vesting three months from the 2023 Grant Date and the final installment vesting date on the date that is 12 months from the 2023 Grant Date, subject to continued services as a Director of the Company. Additionally, in the event of (i) the early termination of this Agreement prior to the expiration of the Term and/or (ii) a change in control of the Company and on terms consistent with board of director equity award agreements, all unvested 2023 Awards will accelerate and vest in full."

2. Term. Section 12 of the Agreement is amended and restated in its entirety as follows:

"Term & Termination. The term of this Agreement will commence on the Effective Date and will expire on December 31, 2023, unless extended by mutual written agreement of the parties or earlier terminated in accordance with this Section 12 (the "Term").Notwithstanding anything to the contrary herein, the Company may terminate this Agreement, for any or no reason, on written notice to Executive Chair and Executive Chair may terminate this agreement, for any reason or no reason, on fifteen (15) days prior written notice to the Company. The Board shall periodically review the ongoing benefit of the Executive Chair position to the Company and may elect to terminate the Agreement in accordance with the preceding sentence following any such review."

3. Governing Law. This First Amendment shall be governed by and construed in accordance with the laws of the Commonwealth of Massachusetts, without giving effect to the principles of conflicts of laws of such state. The parties agree to submit to the exclusive jurisdiction of the state and federal courts located in Commonwealth of Massachusetts and waive any defense of inconvenient forum to the maintenance of any action or proceeding in such courts.

4. Entire Agreement. Except as amended hereby, the Agreement shall remain in full force and effect as originally written..

[Signature page follows]

IN WITNESS WHEREOF, the parties have executed this First Amendment to Executive Chair Agreement as of the date first above written.

SOLID BIOSCIENCES INC.

By: /s/ Ilan Ganot

Name: Ilan Ganot

Title: Chief Executive Officer

IAN F. SMITH

/s/ Ian F. Smith

SECOND AMENDMENT TO

EXECUTIVE CHAIR AGREEMENT

THIS SECOND AMENDMENT TO EXECUTIVE CHAIR AGREEMENT ("Amendment") is effective January 1, 2024 (the "Second Amendment Date"), is by and between Ian F. Smith, an individual having an address at 104 Meadowbrook Road, Weston, MA, 02493 (the "Executive Chair"), and Solid Biosciences Inc., a Delaware corporation (together with its affiliates, the "Company"), having an address at 500 Rutherford Ave., Charlestown, MA 02129.

WHEREAS, Executive Chair and the Company are parties to that certain Executive Chair Agreement ("Agreement"), effective as of January 1, 2022 (the "Effective Date"), and

WHEREAS, the parties desire to amend the Agreement in manner set forth below.

NOW, THEREFORE, in consideration of the premises and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties agree as follows:

1.Definitions. Each capitalized term used but not defined herein shall have the meaning ascribed to such term in the Agreement.

2.Compensation. Section 2 of the Agreement is amended by inserting a new Section 2B as follows:
"2A. Compensation – 2023. As consideration for Services provided under this Agreement from and after January 1, 2024, the Company shall, subject to approval of Company's Board, grant to Executive Chair equity awards (the "2024 Awards") with an aggregate total Black Scholes value of $150,000, consisting of (i) 50% stock options (the "2024 Stock Options") at an exercise price per share equal to the closing price of the Company's Common Stock on 2024 Grant Date and (ii) 50% restricted stock units ("2024 RSUs"), in each case adjusted, as applicable and appropriate, to reflect fully the effect of any reclassification, stock split, reverse split, stock dividend, reorganization, recapitalization or other like change with respect to Company Common Stock occurring (or for which a record date is established) after the date of this Amendment and prior to the Grant Date. The 2024 Awards will vest in equal quarterly installments with the first installment vesting three months from the 2024 Grant Date and the final installment vesting date on the date that is 12 months from the 2024 Grant Date, subject to continued services as a Director of the Company. Additionally, in the event of (i) the early termination of this Agreement prior to the expiration of the Term and/or (ii) a change in control of the Company and on terms consistent with Board of Director equity award agreements, all unvested 2024 Awards will accelerate and vest in full. The Company's external compensation consultants evaluated the cash rate and found the rates to be appropriate. The consultants were present for the official recommendation to the Compensation Committee."

3.Term. Section 12 of the Agreement is amended and restated Second in its entirety as follows:

"Term & Termination. The term of this Agreement will commence on the Effective Date and will expire on December 31, 2024, unless extended by mutual written agreement of the parties or earlier terminated in accordance with this Section 12 (the "Term"). Notwithstanding anything to the contrary herein, the Company may terminate this Agreement, for any or no reason, on written notice to Executive Chair, and Executive Chair may terminate this agreement, for any reason or no reason, on fifteen (15) days prior written notice to the Company. The Board shall, on or about June 1, 2024, and periodically thereafter, review the ongoing benefit of the Executive Chair position to the Company and may elect to terminate the Agreement in accordance with the preceding sentence following any such review in accordance with the preceding sentence.

3. Remaining Provisions of the Agreement. Except as provided herein, each of the other provisions of the Agreement shall remain in full force and effect.

4. References. Upon the effectiveness of this Amendment, on and after the date hereof, each reference in the Agreement to "this Agreement," "hereunder," "hereof," "herein," or words of like important shall mean and be reference to the Agreement, as amended hereby.

5. Governing Law. This Second Amendment shall be governed by and construed in accordance with the laws of the Commonwealth of Massachusetts, without giving effect to the principles of conflicts of laws of such state. The parties agree to submit to the exclusive jurisdiction of the state and federal courts located in Commonwealth of Massachusetts and waive any defense of inconvenient forum to the maintenance of any action or proceeding in such courts.

6. Counterparts. This Amendment may be executed in one or mor counterparts, including by electronic (PDF) transmission, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

7. Entire Agreement. This Agreement, as supplemented and modified by this Amendment contains the Parties' entire agreement, and there are no promises or conditions in any other agreement between the Parties, whether oral or written, concerning the subject matter hereof. As supplemented and modified by this Amendment., the agreement supersedes any prior written or oral agreements between the Parties concerning the subject matter hereof.

[Signature page follows]

IN WITNESS WHEREOF, the parties have executed this Amendment No. 2 to Executive Chair Agreement as of the date first above written.

SOLID BIOSCIENCES INC.

By: /s/ Bo Cumbo

Name: Bo Cumbo

Title: President and CEO

Date: 12/31/2023

IAN F. SMITH

/s/ Ian F. Smith

Date: 01/01/2024

Exhibit 10.27

SOLID BIOSCIENCES INC.
AMENDED AND RESTATED 2021 EMPLOYEE STOCK PURCHASE PLAN

The following constitute the provisions of the Amended and Restated 2021 Employee Stock Purchase Plan of Solid Biosciences Inc.

1. Purpose. The purpose of the Plan is to provide eligible employees of the Company and its Designated Subsidiaries with opportunities to purchase shares of Common Stock through accumulated payroll deductions. It is the intention of the Company to have the Plan qualify as an "employee stock purchase plan" under Section 423 of the Code and the regulations promulgated thereunder. The provisions of the Plan, accordingly, shall be construed consistent therewith.

2. Definitions.

(a) "Acquisition Price" shall have the meaning given such term in Section 17(b)(2) of the Plan.

(b) "Board" shall mean the Board of Directors of the Company.

(c) "Code" shall mean the Internal Revenue Code of 1986, as amended.

(d) "Committee" shall have the meaning given such term in Section 13 of the Plan.

(e) "Common Stock" shall mean the common stock, par value $0.001, of the Company.

(f) "Company" shall mean Solid Biosciences Inc.

(g) "Compensation" shall mean the amount of money reportable on the employee's Federal Income Tax Withholding Statement (or analogous non-U.S. statement), excluding overtime, shift premium, incentive or bonus awards, allowances and reimbursements for expenses such as relocation allowances for travel expenses, income or gains associated with the grant or vesting of restricted stock, income or gains on the exercise of Company stock options or stock appreciation rights, and similar items, whether or not shown or separately identified on the employee's Federal Income Tax Withholding Statement (or analogous non-U.S. statement), but including, in the case of salespersons, sales commissions to the extent determined by the Board.

(h) "Designated Subsidiaries" shall mean the Subsidiaries which have been designated by the Board from time to time in its sole discretion as eligible to participate in the Plan.

(i) "Enrollment Date" shall mean the first day of each Offering Period.

(j) "Exercise Date" shall mean the last day of each Purchase Period.

(k) "Fair Market Value" shall mean, as of any date, (a) the closing price (for the primary trading session) on any national securities exchange on which the Common Stock is listed, or (b) the average of the closing bid and asked prices in the over-the-counter-market, whichever is applicable, as published in The Wall Street Journal or another source selected by the Board. If no sales of Common Stock were made on such a day, the price of the Common Stock shall be the reported price for the preceding day on which sales were made.

(l) "Offering Period" shall mean the period of approximately twenty-four (24) months during which an option granted pursuant to the Plan may be exercised, commencing on the first Trading Day on or after June 1 and December 1 of each year and terminating on the last Trading Day in the period ending twenty-four (24) months later. The duration and timing of an Offering Period may be changed pursuant to Section 4 of this Plan.

(m) "Option Shares" shall have the meaning given such term in Section 7 of the Plan.

(n) "Participant" shall have the meaning given such term in Section 5(a) of the Plan.

(o) "Plan" shall mean this Amended and Restated 2021 Employee Stock Purchase Plan.

(p) "Purchase Period" shall mean the period commencing the day after an Exercise Date and ending on the Trading Day closest to the day that is six (6) months after the preceding Exercise Date, except that the first Purchase Period of any Offering Period shall commence on the Enrollment Date and end with the Trading Day that is six (6) months after the Enrollment Date. The duration and timing of Purchase Periods may be changed pursuant to Section 4 of the Plan.

(q) "Purchase Price" shall mean, unless the Board determines otherwise, an amount equal to 85% of the Fair Market Value of a share of Common Stock on the Enrollment Date or on the Exercise Date, whichever is lower.

(r) "Reorganization Event" shall have the meaning given such term in Section 17(b)(i) of the Plan.

(s) "Subsidiary" shall mean any present or future subsidiary corporation as defined in Section 424(f) of the Code.

(t) "Trading Day" shall mean a day on which national stock exchanges and the Nasdaq System are open for trading.

3. Eligibility.

(a) All employees of the Company and all employees of any Designated Subsidiary are eligible to participate in any one or more of the offerings to purchase Common Stock under the Plan provided that:

(i) they are customarily employed by the Company or a Designated Subsidiary for more than 20 hours a week and for more than five months in a calendar year; and

(ii) they have been employed by the Company or a Designated Subsidiary for at least thirty days prior to enrolling in the Plan; and

(iii) they are employees of the Company or a Designated Subsidiary on the given Enrollment Date.

(b) Any provisions of the Plan to the contrary notwithstanding, no employee shall be granted an option under the Plan (i) to the extent that, immediately after the grant, such employee

(or any other person whose stock would be attributed to such employee pursuant to Section 424(d) of the Code) would own capital stock of the Company or of any Subsidiary and/or hold outstanding options to purchase such stock possessing five percent (5%) or more of the total combined voting power or value of all classes of the capital stock of the Company or of any Subsidiary, or (ii) to the extent that his or her rights to purchase stock under all employee stock purchase plans of the Company and its Subsidiaries accrues at a rate which exceeds Twenty-Five Thousand Dollars ($25,000) worth of stock (determined at the Fair Market Value of the shares at the time such option is granted) for each calendar year in which such option is outstanding at any time. In the event that an employee may not be granted an option under the Plan because of the foregoing restrictions, the employee shall be granted an option to purchase the maximum number of shares that would not violate the foregoing restrictions.

(c) The Company retains the discretion to determine which eligible employees may participate in an offering pursuant to and consistent with Treasury Regulation Sections 1.423-2(e) and (f).

4. Offering Periods. The Plan shall be implemented by consecutive, overlapping Offering Periods with a new Offering Period commencing on the first Trading Day on or after June 1 and December 1 each year, or on such other date as the Board shall determine, and continuing thereafter until terminated in accordance with Section 19 hereof. The Board shall have the power to change the duration of Offering Periods and Purchase Periods (including the commencement dates thereof) with respect to future offerings without stockholder approval.

5. Participation.

(a) An eligible employee may become a participant in the Plan (a "Participant") by completing and submitting the applicable enrollment and payroll deduction authorization or other forms prescribed by the Board in accordance with enrollment procedures prescribed by the Board (which may include accessing the website designated by the Company and electronically enrolling and authorizing payroll deductions or completing other forms) prior to the applicable the applicable Enrollment Date.

(b) The payroll deduction authorization form will authorize a regular payroll deduction from the Compensation received by the employee during the Offering Period. Payroll deductions for a Participant shall commence on the first payroll following the Enrollment Date and shall end on the last payroll in the Offering Period to which such authorization is applicable, unless sooner terminated by the Participant as provided in Section 10 hereof.

6. Payroll Deductions.

(a) At the time a Participant files his or her payroll deduction authorization form, he or she shall elect to have payroll deductions made on each pay day during the Offering Period in an amount not exceeding fifteen percent (15%) of the Compensation which he or she receives on each pay day during the Offering Period. Such payroll deductions shall be in whole percentages only. The Board may, at its discretion, designate a lower maximum contribution rate. The minimum payroll deduction is such percentage of Compensation as may be established from time to time by the Board.

(b) All payroll deductions made for a Participant shall be credited to his or her account under the Plan. A Participant may not make any additional payments into such account.

(c) A Participant may discontinue his or her participation in the Plan as provided in Section 10 hereof, or may decrease the rate of his or her payroll deduction once during any Offering Period, by filing either a written or electronic new payroll deduction authorization form, as determined by the Company. However, an employee may not increase his or her payroll deduction during any Offering Period. The Board may, in its discretion, limit the number of participation rate changes during any Offering Period. If a Participant elects to discontinue payroll deductions during an Offering Period, but does not elect to withdraw his or her funds pursuant to Section 10, funds deducted prior to such election to discontinue will be applied to the purchase of Common Stock on the next occurring Exercise Date. A Participant's payroll deduction authorization form shall remain in effect for successive Offering Periods unless terminated as provided in Section 10 hereof.

(d) At the time the option (as described in Section 7) is exercised, in whole or in part, or at the time any of the Common Stock issued under the Plan is disposed of, the Participant must make adequate provision for the Company's federal, state, or other tax withholding obligations, if any, which arise upon the exercise of the option or the disposition of the Common Stock. At any time, the Company may, but shall not be obligated to, withhold from the Participant's compensation the amount necessary for the Company to meet applicable withholding obligations, including any withholding required to make available to the Company any tax deductions or benefits attributable to sale or other disposition of Common Stock by the Participant.

7. Grant of Option.

(a) On the Enrollment Date of each Offering Period, each eligible employee participating in such Offering Period shall be granted an option to purchase (at the applicable Purchase Price) up to a whole number of shares of the Common Stock the ("Option Shares") determined by dividing $50,000 by the Fair Market Value of a share of Common Stock on the Enrollment Date (subject to any adjustment pursuant to Section 17), and provided that such purchase shall be subject to the limitations set forth in Sections 3(b) and 12 hereof. The option shall be exercisable as to 25% of the Option Shares on each Exercise Date during the Offering Period. Exercise of the option shall occur as provided in Section 8 hereof, unless the Participant has withdrawn pursuant to Section 10 hereof. The option shall expire on the last day of the Offering Period following the purchase of shares pursuant to Section 8.

(b) To the extent permitted by any applicable laws, regulations, or rules of the established stock exchange, national market system, or over-the-counter market on which the Common Stock trades, if the Fair Market Value of the Common Stock on the Enrollment Date of the next Offering Period is lower than the Fair Market Value of the Common Stock on the Enrollment Date of any current Offering Period, then all Participants in such current Offering Period shall be automatically withdrawn from such Offering Period immediately after the exercise of their option on the next occurring Exercise Date and shall be automatically re-enrolled in the next Offering Period as of the first day thereof.

8. Exercise of Option. Unless a Participant withdraws from the Plan as provided in Section 10 hereof, his or her option for the purchase of shares shall be exercised automatically on

each Exercise Date during the Offering Period, and a number of full shares not exceeding the number of shares as to which such Participant's option is exercisable on such Exercise Date shall be purchased for such Participant at the applicable Purchase Price with the accumulated payroll deductions in his or her account. No fractional shares shall be purchased. Any balance remaining in a Participant's payroll deduction account at the end of a Purchase Period will be automatically refunded to the Participant, except that any balance which is less than the purchase price of one share of Common Stock will be carried forward into the Participant's payroll deduction account for the following Purchase Period or Offering Period, unless the employee elects not to participate in the next Purchase Period or Offering Period, in which case the balance in the employee's account shall be refunded. During a Participant's lifetime, a Participant's option to purchase shares hereunder is exercisable only by him or her.

9. Delivery.

(a) Certificates representing shares of Common Stock purchased under the Plan may be issued only in the name of the Participant, in the name of the Participant and another person of legal age as joint tenants with rights of survivorship, or (in the Company's sole discretion) in the name of a brokerage firm, bank, or other nominee holder designated by the Participant. The Company may, in its sole discretion and in compliance with applicable laws, authorize the use of book entry registration of shares in lieu of issuing certificates.

(b) The Participant shall have no interest or voting right in shares covered by his or her option until such option has been exercised and then only with respect to the Option Shares actually purchased for the account of the Participant.

(c) The Company may require that the shares purchased on behalf of each Participant shall be deposited directly into a brokerage account which the Company shall establish for the Participant at a Company-designated brokerage firm. The account will be known as the ESPP Broker Account. Except as otherwise provided below, the deposited shares may not be transferred (either electronically or in certificate form) from the ESPP Broker Account until the ***later*** of the following two periods: (i) the end of the two (2)-year period measured from the date the participant first commences participation in an Offering Period in which the shares were purchased and (ii) the end of the one (1)-year period measured from the Exercise Date of those shares. Such limitation shall apply both to transfers to different accounts with the same ESPP broker and to transfers to other brokerage firms. Any shares held for the required holding period may thereafter be transferred (either electronically or in certificate form) to other accounts or to other brokerage firms. The foregoing procedures shall not in any way limit when a Participant may sell his or her shares. The foregoing procedures are designed solely to assure that any sale of shares prior to the satisfaction of the required holding period is made through the ESPP Broker Account. In addition, a Participant may request a stock certificate or share transfer from his or her ESPP Broker Account prior to the satisfaction of the required holding period should the Participant wish to make a gift of any shares held in that account. However, shares may not be transferred (either electronically or in certificate form) from the ESPP Broker Account for use as collateral for a loan, unless those shares have been held for the required holding period.

10. Withdrawal; Termination of Employment.

(a) A Participant may withdraw all but not less than all the payroll deductions credited to his or her account and not yet used to exercise his or her option under the Plan at any time prior to the close of business on the fifteenth business day prior to an Exercise Date by giving written notice to the Company in the form designated by the Company. All of the Participant's payroll deductions credited to his or her account shall be paid to such Participant promptly after receipt of notice of withdrawal and such Participant's option for the Offering Period shall be automatically terminated, and no further payroll deductions for the purchase of shares shall be made for such Offering Period. If a Participant withdraws from an Offering Period, payroll deductions shall not resume at the beginning of the succeeding Offering Period unless the Participant delivers to the Company a new payroll deduction authorization form in accordance with the terms and conditions established by the Board.

(b) Upon a Participant's ceasing to be an employee, for any reason, he or she shall be deemed to have elected to withdraw from the Plan and the payroll deductions credited to such Participant's account during the Offering Period but not yet used to exercise the option shall be returned to such Participant or, in the case of his or her death, to the person or persons entitled thereto under Section 14 hereof, and such Participant's option shall be automatically terminated. If, prior to the last day of the Offering Period, the Designated Subsidiary by which the employee is employed shall cease to be a Subsidiary of the Company, or if the employee is transferred to a Subsidiary of the Company that is not a Designated Subsidiary, the employee shall be deemed to have terminated employment for purposes of this Plan.

(c) A Participant's withdrawal from an Offering Period shall not have any effect upon his or her eligibility to participate in any similar plan which may hereafter be adopted by the Company or in succeeding Offering Periods.

11. Interest. Interest will not be paid on any Participant accounts, except to the extent that the Board, in its sole discretion, elects to credit employee accounts with interest at such rate as it may from time to time determine.

12. Stock. Subject to adjustment under Section 17(a) of the Plan, a number of shares of Common Stock equal to the sum of the following have been approved for issuance under the Plan: (a) 473,525 shares of Common Stock and (b) an annual increase to be added on the first day of each fiscal year, commencing with the fiscal year ending December 31, 2024 and continuing for each fiscal year until, and including, the fiscal year ending December 31, 2033, equal to the least of (i) 293,597 shares of Common Stock; (ii) 1% of the outstanding shares of Common Stock on such date; and (iii) an amount determined by the Board. If, on a given Exercise Date, the number of shares with respect to which options are to be exercised exceeds the number of shares then available under the Plan, the Company shall make a pro rata allocation of the shares remaining available for purchase in as uniform a manner as shall be practicable and as it shall determine to be equitable.

13. Administration. The Plan shall be administered by the Board or a committee of members of the Board appointed by the Board (a "Committee"). The Board or its Committee has full and exclusive discretionary authority to construe, interpret and apply the terms of the Plan, to determine eligibility and to adjudicate all disputed claims filed under the Plan. Every finding, decision and determination made by the Board or its Committee shall, to the fullest extent

permitted by law, be final, conclusive and binding upon all parties. Any reference to the authority of the Committee to act under this Plan shall be contingent upon the Board having delegated such authority to the Committee. All references to the Board contained herein shall also refer to its Committee, as applicable.

14. Designation of Beneficiary.

(a) A Participant may file a written designation of a beneficiary who is to receive any shares and cash, if any, from the Participant's account under the Plan in the event of such Participant's death subsequent to an Exercise Date on which the option is exercised but prior to delivery to such Participant of such shares and cash. In addition, a Participant may file a written designation of a beneficiary who is to receive any cash from the Participant's account under the Plan in the event of such Participant's death prior to exercise of the option. If a Participant is married and the designated beneficiary is not the spouse, spousal consent shall be required for such designation to be effective.

(b) Such designation of beneficiary may be changed by the Participant at any time by written notice. In the event of the death of a Participant and in the absence of a beneficiary validly designated under the Plan who is living at the time of such Participant's death, the Company shall deliver such shares and/or cash to the executor or administrator of the estate of the Participant, or if no such executor or administrator has been appointed (to the knowledge of the Company), the Company, in its discretion, may deliver such shares and/or cash to the spouse or to any one or more dependents or relatives of the Participant, or if no spouse, dependent or relative is known to the Company, then to such other person as the Company may designate.

15. Transferability. Neither payroll deductions credited to a Participant's account nor any rights with regard to the exercise of an option or to receive shares under the Plan may be assigned, transferred, pledged or otherwise disposed of in any way (other than by will, the laws of descent and distribution or as provided in Section 14 hereof) by the Participant. Any such attempt at assignment, transfer, pledge or other disposition shall be without effect, except that the Company may treat such act as an election to withdraw funds from an Offering Period in accordance with Section 10 hereof.

16. Use of Funds. All payroll deductions received or held by the Company under the Plan may be used by the Company for any corporate purpose, and the Company shall not be obligated to segregate such payroll deductions.

17. Adjustments for Changes in Common Stock and Certain Other Events.

(a) Changes in Capitalization. In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of Common Stock other than an ordinary cash dividend, (i) the number and class of securities available under this Plan, (ii) the share limitation set forth in Section 7, and (iii) the Purchase Price shall be equitably adjusted to the extent determined by the Board.

(b) Reorganization Events.

(i) Definition. A "Reorganization Event" shall mean: (A) any merger or consolidation of the Company with or into another entity as a result of which all of the

Common Stock of the Company is converted into or exchanged for the right to receive cash, securities or other property or is cancelled, (B) any transfer or disposition of all of the Common Stock for cash, securities or other property pursuant to a share exchange or other transaction or (C) any liquidation or dissolution of the Company.

(ii) Consequences of a Reorganization Event on Options. In connection with a Reorganization Event, the Board may take any one or more of the following actions as to outstanding options on such terms as the Board determines: (A) provide that options shall be assumed, or substantially equivalent options shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof), (B) upon written notice to Participants, provide that all outstanding options will be terminated immediately prior to the consummation of such Reorganization Event and that all such outstanding options will become exercisable to the extent of accumulated payroll deductions as of a date specified by the Board in such notice, which date shall not be less than ten (10) days preceding the effective date of the Reorganization Event, (C) upon written notice to Participants, provide that all outstanding options will be cancelled as of a date prior to the effective date of the Reorganization Event and that all accumulated payroll deductions will be returned to participating employees on such date, (D) in the event of a Reorganization Event under the terms of which holders of Common Stock will receive upon consummation thereof a cash payment for each share surrendered in the Reorganization Event (the "Acquisition Price"), change the last day of the Offering Period to be the date of the consummation of the Reorganization Event and make or provide for a cash payment to each employee equal to (1) (i) the Acquisition Price times (ii) the number of shares of Common Stock that the Participant's accumulated payroll deductions as of immediately prior to the Reorganization Event could purchase at the Purchase Price, where the Acquisition Price is treated as the fair market value of the Common Stock on the last day of the applicable Plan Period for purposes of determining the Purchase Price under Section 2(q) hereof, and where the number of shares that could be purchased is subject to the limitations set forth in Sections 3 and 7, minus (2) the result of multiplying such number of shares by such Purchase Price, (E) provide that, in connection with a liquidation or dissolution of the Company, options shall convert into the right to receive liquidation proceeds (net of the Purchase Price thereof) and (vi) any combination of the foregoing.

(iii) For purposes of clause (b)(ii)(A) above, an option shall be considered assumed if, following consummation of the Reorganization Event, the replacement option confers the right to purchase, for each share of Common Stock subject to the option immediately prior to the consummation of the Reorganization Event, the consideration (whether cash, securities or other property) received as a result of the Reorganization Event by holders of Common Stock for each share of Common Stock held immediately prior to the consummation of the Reorganization Event (and if holders were offered a choice of consideration, the type of consideration chosen by the holders of a majority of the outstanding shares of Common Stock); provided, however, that if the consideration received as a result of the Reorganization Event is not solely common stock of the acquiring or succeeding corporation (or an affiliate thereof), the Company may, with the consent of the acquiring or succeeding corporation, provide for the consideration to be received upon the exercise of options to consist solely of such number of shares of

common stock of the acquiring or succeeding corporation (or an affiliate thereof) that the Board determines to be equivalent in value (as of the date of such determination or another date specified by the Board) to the per share consideration received by holders of outstanding shares of Common Stock as a result of the Reorganization Event.

18. Amendment or Termination.

(a) The Board may at any time, and from time to time, amend or suspend this Plan or any portion thereof, except that (i) if the approval of any such amendment by the shareholders of the Company is required by Section 423 of the Code, such amendment shall not be effected without such approval, and (ii) in no event may any amendment be made which would cause the Plan to fail to comply with Section 423 of the Code. This Plan may be terminated at any time by the Board. Upon termination of the Plan all amounts in the accounts of Participants shall be promptly refunded.

(b) Without stockholder consent and without regard to whether any Participant rights may be considered to have been "adversely affected," the Board shall be entitled to change the Offering Periods and Purchase Periods, limit the frequency and/or number of changes in the amount withheld during an Offering Period, establish the exchange ratio applicable to amounts withheld in a currency other than U.S. dollars, permit payroll withholding in excess of the amount designated by a Participant in order to adjust for delays or mistakes in the Company's processing of properly completed withholding elections, establish reasonable waiting and adjustment periods and/or accounting and crediting procedures to ensure that amounts applied toward the purchase of Common Stock for each Participant properly correspond with amounts withheld from the Participant's Compensation and establish such other limitations or procedures as the Board determines in its sole discretion advisable which are consistent with the Plan, subject, in each case, to Section 18(a) above.

19. Notices. All notices or other communications by a Participant to the Company under or in connection with the Plan shall be deemed to have been duly given when received in the form specified by the Company at the location, or by the person, designated by the Company for the receipt thereof.

20. Conditions Upon Issuance of Shares. Shares shall not be issued with respect to an option unless the exercise of such option and the issuance and delivery of such shares pursuant thereto shall comply with all applicable provisions of law, domestic or foreign, including, without limitation, the Securities Act of 1933, as amended, the Securities Exchange Act of 1934, as amended (the "Exchange Act"), the rules and regulations promulgated thereunder, and the requirements of any stock exchange upon which the shares may then be listed, and shall be further subject to the approval of counsel for the Company with respect to such compliance. As a condition to the exercise of an option, the Company may require the person exercising such option to represent and warrant at the time of any such exercise that the shares are being purchased only for investment and without any present intention to sell or distribute such shares if, in the opinion of counsel for the Company, such a representation is required by any of the aforementioned applicable provisions of law.

21. Effective Date. The amendment and restatement of the Company's 2021 Employee Stock Purchase Plan, as amended, shall become effective on November 12, 2023.

22. Governmental Regulations. The Company's obligation to sell and deliver Common Stock under this Plan is subject to listing on an established stock exchange or quotation on a national market system or an over the counter market (to the extent the Common Stock is then so listed or quoted) and the approval of all governmental authorities required in connection with the authorization, issuance, or sale of such stock.

23. Governing Law. The Plan shall be governed by Delaware law except to the extent that such law is preempted by federal law.

24. Source of Shares. Shares may be issued upon exercise of an option from authorized but unissued Common Stock, from shares held in the treasury of the Company, or from any other proper source.

25. Notification Upon Sale of Shares. Each employee agrees, by participating in the Plan, to promptly give notice to the Company of any disposition of shares purchased under the Plan where such disposition occurs within two years after the Enrollment Date with respect to the option pursuant to which such shares were purchased or within one year of the date of exercise of such option pursuant to which such shares were purchased.

26. Grants to Employees in Foreign Jurisdictions. The Company may, to comply with the laws of a foreign jurisdiction, grant options to employees of the Company or a Designated Subsidiary who are citizens or residents of such foreign jurisdiction (without regard to whether they are also citizens of the United States or resident aliens (within the meaning of Section 7701(b)(1)(A) of the Code)) with terms that are less favorable (but not more favorable) than the terms of options granted under the Plan to employees of the Company or a Designated Subsidiary who are resident in the United States. Notwithstanding the preceding provisions of this Plan, employees of the Company or a Designated Subsidiary who are citizens or residents of a foreign jurisdiction (without regard to whether they are also citizens of the United States or resident aliens (within the meaning of Section 7701(b)(1)(A) of the Code)) may be excluded from eligibility under the Plan if (a) the grant of an option under the Plan to a citizen or resident of the foreign jurisdiction is prohibited under the laws of such jurisdiction or (b) compliance with the laws of the foreign jurisdiction would cause the Plan to violate the requirements of Section 423 of the Code. The Company may add one or more appendices to this Plan describing the operation of the Plan in those foreign jurisdictions in which employees are excluded from participation or granted less favorable options.

27. Authorization of Sub-Plans. The Board may from time to time establish one or more sub-plans under the Plan with respect to one or more Designated Subsidiaries, provided that such sub-plan complies with Section 423 of the Code.

28. No Right to Employment. Participation in the Plan by a Participant will not be construed as giving a Participant the right to be retained as an employee of the Company or a Designated Subsidiary, as applicable. Further, the Company or a Designated Subsidiary may

dismiss a Participant from employment at any time, free from any liability or any claim under the Plan.

Approved by the stockholders on
June 16, 2021 and June 6, 2023

Adopted by the Board of Directors on
November 12, 2023

Exhibit 10.30

SOLID BIOSCIENCES INC.
Shares of Common Stock
(par value $0.001 per share)

AMENDED AND RESTATED SALES AGREEMENT

March 13, 2024

JEFFERIES LLC
520 Madison Avenue
New York, New York 10022

Ladies and Gentlemen:

Solid Biosciences Inc., a Delaware corporation (the "**Company**"), proposes, subject to the terms and conditions stated herein, to issue and sell from time to time through Jefferies LLC, as sales agent and/or principal (the "**Agent**"), shares of the Company's common stock, par value $0.001 per share (the "**Common Shares**"), on the terms set forth in this agreement (this "**Agreement**").

Section 1. DEFINITIONS

(a) Certain Definitions. For purposes of this Agreement, capitalized terms used herein and not otherwise defined shall have the following respective meanings:

"**Actions**" has the meaning set forth in Section 2(t).

"**Affiliate**" of a Person means another Person that directly or indirectly, through one or more intermediaries, controls, is controlled by, or is under common control with, such first-mentioned Person. The term "control" (including the terms "controlling," "controlled by" and "under common control with") means the possession, direct or indirect, of the power to direct or cause the direction of the management and policies of a Person, whether through the ownership of voting securities, by contract or otherwise.

"**Agency Period**" means the period commencing on the date of this Agreement and expiring on the earliest to occur of (i) the date on which the Agent shall have placed the Maximum Program Amount pursuant to this Agreement and (ii) the date this Agreement is terminated pursuant to Section 7.

"**Agent**" has the meaning set forth in the introductory paragraph of this Agreement.

"**Agreement**" has the meaning set forth in the introductory paragraph of this Agreement.

"**Anti-Money Laundering Laws**" has the meaning set forth in Section 2(jj).

"**Code**" has the meaning set forth in Section 2(k).

"**Commission**" means the U.S. Securities and Exchange Commission.

"**Common Shares**" has the meaning set forth in the introductory paragraph of this Agreement.

"**Company**" has the meaning set forth in the introductory paragraph of this Agreement.

"**Company Stock Plans**" has the meaning set forth in Section 2(k).

"**EDGAR**" has the meaning set forth in Section 2(b).

"**Environmental Laws**" has the meaning set forth in Section 2(cc).

"**ERISA**" has the meaning set forth in Section 2(dd).

"**Exchange Act**" means the Securities Exchange Act of 1934, as amended, and the rules and regulations of the Commission thereunder.

"**FINRA**" has the meaning set forth in Section 2(s).

"**Floor Price**" means the minimum price per share set by the Company in the Issuance Notice below which the Agent shall not sell Shares during the applicable Selling Period, which may be adjusted by the Company at any time during the Selling Period by delivering written notice of such change to the Agent and which in no event shall be less than $1.00 without the prior written consent of the Agent, which may be withheld in the Agent's sole discretion.

"**Free Writing Prospectus**" has the meaning set forth in Section 4(e).

"**GAAP**" has the meaning set forth in Section 2(g).

"**Intellectual Property**" has the meaning set forth in Section 2(w).

"**Interim Prospectus Supplement**" has the meaning set forth in Section 4(a).

"**Investment Company Act**" has the meaning set forth in Section 2(y).

"**Issuance Amount**" means the aggregate Sales Price of the Shares to be sold by the Agent pursuant to any Issuance Notice.

"**Issuance Notice**" means a written notice delivered to the Agent by the Company in accordance with this Agreement in the form attached hereto as Exhibit A that is executed by its Chief Executive Officer, President or Chief Financial Officer.

"**Issuance Notice Date**" means any Trading Day during the Agency Period that an Issuance Notice is delivered pursuant to Section 3(b)(i).

"**Issuance Price**" means the Sales Price less the Selling Commission.

"**IT Systems and Data**" has the meaning set forth in Section 2(ii).

"**Material Adverse Effect**" has the meaning set forth in Section 2(i).

"**Maximum Program Amount**" means Common Shares with an aggregate Sales Price of the lesser of (i) the number or dollar amount of Common Shares registered under the effective Registration Statement (defined below) pursuant to which the offering is being made, (ii) the number of authorized but unissued Common Shares (less Common Shares issuable upon exercise, conversion or exchange of any outstanding securities of the Company or otherwise reserved from the Company's authorized capital stock), (iii) the number or dollar amount of Common Shares permitted to be sold under Form S-3 (including General Instruction I.B.6 thereof, if applicable), or (iv) the number or dollar amount of Common Shares for which the Company has filed a Prospectus (defined below).

"**Person**" means an individual or a corporation, partnership, limited liability company, trust, incorporated or unincorporated association, joint venture, joint stock company, governmental authority or other entity of any kind.

"**Plan**" has the meaning set forth in Section 2(dd).

"**Principal Market**" means the Nasdaq Global Select Market or such other national securities exchange on which the Common Shares, including any Shares, are then listed.

"**Prospectus**" has the meaning set forth in Section 2(a).

"**Registration Statement**" has the meaning set forth in Section 2(a).

"**Regulation M**" has the meaning set forth in Section 2(oo).

"**Regulatory Authorities**" has the meaning set forth in Section 2(tt).

"**Representation Date**" has the meaning set forth in the introductory paragraph of Section 2.

"**Rule 102**" has the meaning set forth in Section 4(v).

"**Rule 462(b) Registration Statement**" has the meaning set forth in Section 2(b).

"**Sales Price**" means the actual sale execution price of each Share placed by the Agent pursuant to this Agreement.

"**Securities Act**" means the Securities Act of 1933, as amended, and the rules and regulations of the Commission thereunder.

"**Selling Commission**" means, except as otherwise agreed, three percent (3.00%) of the gross proceeds of the Shares sold pursuant to this Agreement.

"**Selling Period**" means the period set forth in the Issuance Notice.

"**Settlement Date**" means (i) prior to May 28, 2024, the second business day and (ii) on or after May 28, 2024, the first business day following each Trading Day during the Selling Period

on which Shares are sold pursuant to this Agreement, when the Company shall deliver to the Agent the amount of Shares sold on such Trading Day and the Agent shall deliver to the Company the Issuance Price received on such sales.

"**Shares**" shall mean the Company's Common Shares issued or issuable pursuant to this Agreement.

"**Specified Courts**" has the meaning set forth in Section 8(g).

"**Stock Options**" has the meaning set forth in Section 2(k).

"**Time of Sale**" has the meaning set forth in Section 3(b)(v).

"**Time of Sale Information**" has the meaning set forth in Section 2(b).

"**Trading Day**" means any day on which the Principal Market is open for trading.

"**Triggering Event Date**" has the meaning set forth in Section 4(o).

Section 2. REPRESENTATIONS AND WARRANTIES OF THE COMPANY

The Company represents and warrants to, and agrees with, the Agent that, unless such representation, warranty or agreement specifies otherwise, as of (1) the date of this Agreement, (2) each Issuance Notice Date, (3) each Settlement Date, (4) each Triggering Event Date with respect to which the Company is obligated to deliver a certificate pursuant to Section 4(o) and (5) as of each Time of Sale (each of the times referenced above is referred to herein as a "**Representation Date**"), except as may be disclosed in the Prospectus (including any documents incorporated by reference therein and any amendments or supplements thereto) on or before a Representation Date:

(a) Registration Statement. The Company has prepared and filed, or will file, with the Commission a shelf registration statement on Form S-3 that contains a prospectus specifically relating to the Shares. The Company may file one or more additional registration statements from time to time that will contain a base prospectus and related prospectus or prospectus supplement, if applicable, with respect to the Shares. Except where the context otherwise requires, such registration statement registers the issuance and sale by the Company of the Shares under the Securities Act. Such registration statement(s), including any information deemed to be a part thereof pursuant to Rule 430B under the Securities Act, including all financial statements, exhibits and schedules thereto and all documents incorporated or deemed to be incorporated therein by reference pursuant to Item 12 of Form S-3 under the Securities Act as from time to time amended or supplemented, is herein referred to as the "**Registration Statement**," and the prospectus (including the base prospectus and the sales agreement prospectus) constituting a part of such registration statement(s), together with any prospectus supplement filed with the Commission pursuant to Rule 424(b) under the Securities Act relating to a particular issuance of the Shares, including all documents incorporated or deemed to be incorporated therein by reference pursuant to Item 12 of Form S-3 under the Securities Act, in each case, as from time to time amended or supplemented, is referred to herein as the "**Prospectus**," except that if any revised prospectus is provided to the Agent by the Company for use in connection with the offering of the Shares that is not required to be filed by the Company pursuant to Rule 424(b) under the Securities Act, the

term "**Prospectus**" shall refer to such revised prospectus from and after the time it is first provided to the Agent for such use. As used in this Agreement, the terms "amendment" or "supplement" when applied to the Registration Statement or the Prospectus shall be deemed to include the filing by the Company with the Commission of any document under the Exchange Act after the date hereof that is or is deemed to be incorporated therein by reference.

All references in this Agreement to financial statements and schedules and other information which is "contained," "included" or "stated" in the Registration Statement or the Prospectus (and all other references of like import) shall be deemed to mean and include all such financial statements and schedules and other information which is or is deemed to be incorporated by reference in or otherwise deemed under the Securities Act to be a part of or included in the Registration Statement or the Prospectus, as the case may be, as of any specified date; and all references in this Agreement to amendments or supplements to the Registration Statement or the Prospectus shall be deemed to mean and include, without limitation, the filing of any document under the Exchange Act which is or is deemed to be incorporated by reference in or otherwise deemed under the Securities Act to be a part of or included in the Registration Statement or the Prospectus, as the case may be, as of any specified date.

At the time the Registration Statement was or will be declared effective and at the time the Company's most recent annual report on Form 10-K was filed with the Commission, if later, the Company met the then-applicable requirements for use of Form S-3 under the Securities Act. During the Agency Period, each time the Company files an annual report on Form 10-K the Company will meet the then-applicable requirements for use of Form S-3 under the Securities Act.

(b) Compliance with Registration Requirements. The Registration Statement and any registration statement filed pursuant to Rule 462(b) under the Securities Act ("**Rule 462(b) Registration Statement**") has been or will be declared effective by the Commission under the Securities Act prior to any Issuance Notice Date following such filing. The Company has complied to the Commission's satisfaction with all requests of the Commission for additional or supplemental information in connection therewith, if any. No stop order suspending the effectiveness of the Registration Statement or any Rule 462(b) Registration Statement is in effect and no proceedings for such purpose have been instituted or are pending or, to the best knowledge of the Company, are contemplated or threatened by the Commission.

The Prospectus when filed complied, or will comply, in all material respects with the Securities Act and, if filed by electronic transmission pursuant to the Commission's Electronic Data Gathering, Analysis, and Retrieval system ("**EDGAR**") (except as may be permitted by Regulation S-T under the Securities Act), was identical to the copy thereof delivered to the Agent for use in connection with the issuance and sale of the Shares. Each of the Registration Statement, any Rule 462(b) Registration Statement and any post-effective amendment thereto, at the time it became or becomes effective and at all subsequent times, complied and will comply in all material respects with the Securities Act and did not and will not contain any untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the statements therein not misleading. As of the date of this Agreement, the Prospectus and any Free Writing Prospectus consented to by the Agent pursuant to Section 4(e) considered together (collectively, the "**Time of Sale Information**") did not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements therein, in the light of the circumstances

under which they were made, not misleading. The Prospectus, as amended or supplemented, as of its date and at all subsequent times, did not and will not contain any untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading. The representations and warranties set forth in the three immediately preceding sentences do not apply to statements in or omissions from the Registration Statement, any Rule 462(b) Registration Statement, or any post-effective amendment thereto, or the Prospectus, or any amendments or supplements thereto, made in reliance upon and in conformity with information relating to the Agent furnished to the Company in writing by the Agent expressly for use therein, it being understood and agreed that the only such information furnished by the Agent to the Company consists of the information described in Section 6 below. There are no contracts or other documents required to be described in the Prospectus or to be filed as exhibits to the Registration Statement which have not been described or filed as required. The Registration Statement and the offer and sale of the Shares as contemplated hereby meet the requirements of Rule 415 under the Securities Act and comply in all material respects with said Rule.

(c) Ineligible Issuer Status. The Company is not an "ineligible issuer" in connection with the offering of the Shares pursuant to Rules 164, 405 and 433 under the Securities Act. Any Free Writing Prospectus that the Company is required to file pursuant to Rule 433(d) under the Securities Act has been, or will be, filed with the Commission in accordance with the requirements of the Securities Act. Each Free Writing Prospectus that the Company has filed, or is required to file, pursuant to Rule 433(d) under the Securities Act or that was prepared by or on behalf of or used or referred to by the Company complies or will comply in all material respects with the requirements of Rule 433 under the Securities Act including timely filing with the Commission or retention where required and legending, and each such Free Writing Prospectus, as of its issue date and at all subsequent times through the completion of the issuance and sale of the Shares did not, does not and will not include any information that conflicted, conflicts with or will conflict with the information contained in the Registration Statement or the Prospectus, including any document incorporated by reference therein. Except for the Free Writing Prospectuses, if any, and electronic road shows, if any, furnished to the Agent before first use, the Company has not prepared, used or referred to, and will not, without the Agent's prior consent, prepare, use or refer to, any Free Writing Prospectus.

(d) Incorporated Documents. The documents incorporated or deemed to be incorporated by reference in the Registration Statement and the Prospectus, at the time they were filed with the Commission, complied in all material respects with the requirements of the Exchange Act, as applicable, and, when read together with the other information in the Prospectus, do not contain an untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the statements therein, in light of the circumstances under which they were made, not misleading.

(e) S-3 Eligibility. The Company and the transactions contemplated by this Agreement meet the requirements for, and comply with the conditions for the use of, Form S-3 under the Act, including but not limited to Instruction I.B.1 of Form S-3. The Company is not a shell company (as defined in Rule 405 of the Act) and has not been a shell company for at least 12 calendar months.

(f) Exchange Act Compliance. The documents incorporated or deemed to be incorporated by reference in the Prospectus, at the time they were or hereafter are filed with the Commission, and any Free Writing Prospectus or amendment or supplement thereto complied and will comply in all material respects with the requirements of the Exchange Act, and, when read together with the other information in the Prospectus, at the time the Registration Statement and any amendments thereto become effective and at each Time of Sale (as defined below), as the case may be, will not contain an untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the fact required to be stated therein or necessary to make the statements therein, in the light of the circumstances under which they were made, not misleading.

(g) Financial Statements. The financial statements (including the related notes thereto) of the Company and its consolidated subsidiaries included or incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information comply in all material respects with the applicable requirements of the Securities Act and present fairly in all material respects the financial position of the Company and its consolidated subsidiaries as of the dates indicated and the results of their operations and the changes in their cash flows for the periods specified; such financial statements have been prepared in conformity with generally accepted accounting principles ("**GAAP**") in the United States applied on a consistent basis throughout the periods covered thereby, except unaudited financial statements, which are subject to normal year-end adjustments and do not contain certain footnotes as permitted by the applicable rules of the Commission, and any supporting schedules included or incorporated by reference in the Registration Statement present fairly in all material respects the information required to be stated therein; and the other financial information included or incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information has been derived from the accounting records of the Company and its consolidated subsidiaries and presents fairly in all material respects the information shown thereby.

(h) No Material Adverse Change. Since the date of the most recent financial statements of the Company included or incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information, (i) there has not been any material change in the capital stock (other than the issuance of shares of Common Stock upon exercise of stock options and warrants described as outstanding in, and the grant of options and awards under existing equity incentive plans described in, the Registration Statement, the Prospectus and the Time of Sale Information), short-term debt or long-term debt of the Company or any of its subsidiaries, or any dividend or distribution of any kind declared, set aside for payment, paid or made by the Company on any class of capital stock, or any material adverse change, or any development that would reasonably be expected to result in a material adverse change, in or affecting the business, properties, management, financial position, stockholders' equity, results of operations or prospects of the Company and its subsidiaries taken as a whole; (ii) neither the Company nor any of its subsidiaries has entered into any transaction or agreement (whether or not in the ordinary course of business) that is material to the Company and its subsidiaries taken as a whole or incurred any liability or obligation, direct or contingent, that is material to the Company and its subsidiaries taken as a whole; and (iii) neither the Company nor any of its subsidiaries has sustained any loss or interference with its business that is material to the Company and its subsidiaries taken as a whole and that is either from fire, explosion, flood or other calamity, whether or not covered by insurance, or from any labor disturbance or dispute or any action, order or decree of any court or

arbitrator or governmental or regulatory authority, except in each case as otherwise disclosed in the Registration Statement, the Prospectus and the Time of Sale Information.

(i) Organization and Good Standing. The Company and each of its subsidiaries have been duly organized and are validly existing and in good standing under the laws of their respective jurisdictions of organization, are duly qualified to do business and are in good standing in each jurisdiction in which their respective ownership or lease of property or the conduct of their respective businesses requires such qualification, and have all power and authority necessary to own or hold their respective properties and to conduct the businesses in which they are engaged, except where the failure to be so qualified or in good standing or have such power or authority would not, individually or in the aggregate, reasonably be expected to have a material adverse effect on the business, properties, management, financial position, stockholders' equity, results of operations or prospects of the Company and its subsidiaries taken as a whole or on the performance by the Company of its obligations under this Agreement (a "**Material Adverse Effect**"). Except as otherwise disclosed to the Agent, the Company does not own or control, directly or indirectly, any corporation, association or other entity other than the subsidiaries listed in Exhibit 21.1 to the most recent annual report on Form 10-K filed by the Company and incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information.

(j) Capitalization. All the outstanding shares of capital stock of the Company have been duly and validly authorized and issued and are fully paid and non-assessable and are not subject to any preemptive or similar rights that have not been validly waived; except as described in or expressly contemplated by the Registration Statement, the Prospectus and the Time of Sale Information, there are no outstanding rights (including, without limitation, preemptive rights), warrants, restricted stock units or options to acquire, or instruments convertible into or exchangeable for, any shares of capital stock or other equity interest in the Company or any of its subsidiaries, or any contract, commitment, agreement, understanding or arrangement of any kind relating to the issuance of any capital stock of the Company or any such subsidiary, any such convertible or exchangeable securities or any such rights, warrants, restricted stock units or options; the capital stock of the Company conforms in all material respects to the description thereof contained in the Registration Statement and the Prospectus; and all the outstanding shares of capital stock or other equity interests of each subsidiary owned, directly or indirectly, by the Company have been duly and validly authorized and issued, are fully paid and non-assessable (except, as otherwise described in the Registration Statement, the Prospectus and the Time of Sale Information), and are owned directly or indirectly by the Company, free and clear of any lien, charge, encumbrance, security interest, restriction on voting or transfer or any other claim of any third party.

(k) Stock Options. With respect to the stock options (the "**Stock Options**") granted pursuant to the stock-based compensation plans of the Company and its subsidiaries (the "**Company Stock Plans**"), (i) to the Company's knowledge, each Stock Option intended to qualify as an "incentive stock option" under Section 422 of the Internal Revenue Code of 1986, as amended (the "**Code**") so qualifies, (ii) each grant of a Stock Option was duly authorized no later than the date on which the grant of such Stock Option was by its terms to be effective by all necessary corporate action, including, as applicable, approval by the board of directors of the Company (or a duly constituted and authorized committee thereof) and any required stockholder approval by the necessary number of votes or written consents, and the award agreement governing

such grant (if any) was duly executed by the Company and delivered to the recipient, (iii) each such grant was made in accordance with the terms of the Company Stock Plans and all other applicable laws and regulatory rules or requirements, including the rules of the Principal Market and any other exchange on which Company securities are traded, and (iv) each such grant was properly accounted for in accordance with GAAP in the United States in the financial statements (including the related notes) of the Company.

(l) Due Authorization. The Company has the requisite power and authority to execute and deliver this Agreement and to perform its obligations hereunder; and all action required to be taken for the due and proper authorization, execution and delivery by it of this Agreement and the consummation by it of the transactions contemplated hereby has been duly and validly taken.

(m) Sales Agreement. This Agreement has been duly authorized, executed and delivered by the Company.

(n) The Shares. The Shares to be issued and sold by the Company hereunder, when issued and delivered and paid for as provided herein, will be duly authorized and validly issued, will be fully paid and nonassessable and will conform in all material respects to the descriptions thereof in the Registration Statement, the Prospectus and the Time of Sale Information; and the issuance of the Shares is not subject to any preemptive or similar rights that have not been duly waived or satisfied.

(o) Descriptions of the Sales Agreement. This Agreement conforms in all material respects to the description thereof contained in the Prospectus and the Time of Sale Information.

(p) Stock Exchange Listing. The Common Shares are registered pursuant to Section 12(b) of the Exchange Act and are listed on the Nasdaq Global Select Market, and the Company has taken no action designed to, or likely to have the effect of, terminating the registration of the Common Shares under the Exchange Act or delisting the Common Shares from the Nasdaq Global Select Market, nor has the Company received any notification that the Commission or the Nasdaq Global Select Market is contemplating terminating such registration or listing.

(q) No Violation or Default. Neither the Company nor any of its subsidiaries is (i) in violation of its charter or by-laws or similar organizational documents; (ii) in default, and no event has occurred that, with notice or lapse of time or both, would constitute such a default, in the due performance or observance of any term, covenant or condition contained in any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Company or any of its subsidiaries is a party or by which the Company or any of its subsidiaries is bound or to which any property or asset of the Company or any of its subsidiaries is subject; or (iii) in violation of any law or statute or any judgment, order, rule or regulation of any court or arbitrator or governmental or regulatory authority, except, in the case of clauses (ii) and (iii) above, for any such default or violation that would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

(r) No Conflicts. The execution, delivery and performance by the Company of this Agreement, the issuance and sale of the Shares and the consummation of the transactions contemplated by this Agreement will not (i) conflict with or result in a breach or violation of any

of the terms or provisions of, or constitute a default under, result in the termination, modification or acceleration of, or result in the creation or imposition of any lien, charge or encumbrance upon any property, right or asset of the Company or any of its subsidiaries pursuant to, any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Company or any of its subsidiaries is a party or by which the Company or any of its subsidiaries is bound or to which any property, right or asset of the Company or any of its subsidiaries is subject, (ii) result in any violation of the provisions of the charter or by-laws or similar organizational documents of the Company or any of its subsidiaries or (iii) result in the violation of any law or statute or any judgment, order, rule or regulation of any court or arbitrator or governmental or regulatory authority, except, in the case of clauses (i) and (iii) above, for any such conflict, breach, violation, default, lien, charge or encumbrance that would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

(s) No Consents Required. No consent, approval, authorization, order, registration or qualification of or with any court or arbitrator or governmental or regulatory authority is required for the execution, delivery and performance by the Company of this Agreement, the issuance and sale of the Shares and the consummation of the transactions contemplated by this Agreement and by each Prospectus, except for the registration of the Shares under the Securities Act and such consents, approvals, authorizations, orders and registrations or qualifications as may be required by the Financial Industry Regulatory Authority, Inc. ("**FINRA**"), the Nasdaq Global Select Market or under applicable state securities laws in connection with the sale and distribution of the Shares.

(t) Legal Proceedings. Except as described in the Registration Statement, the Prospectus, and the Time of Sale Information, there are no legal, governmental or regulatory investigations, actions, demands, claims, suits, arbitrations, inquiries or proceedings ("**Actions**") pending to which the Company or any of its subsidiaries is or may be a party or to which any property of the Company or any of its subsidiaries is or may be the subject that, individually or in the aggregate, if determined adversely to the Company or any of its subsidiaries, would reasonably be expected to have a Material Adverse Effect; to the knowledge of the Company, no such Actions are threatened or contemplated by any governmental or regulatory authority or threatened by others; and (i) there are no current or pending Actions that are required under the Securities Act to be described in the Registration Statement, the Prospectus, and the Time of Sale Information that are not so described in the Registration Statement, the Prospectus, and the Time of Sale Information and (ii) there are no statutes, regulations or contracts or other documents that are required under the Securities Act to be filed as exhibits to the Registration Statement or described in the Registration Statement, the Prospectus, or the Time of Sale Information that are not so filed as exhibits to the Registration Statement or described in the Registration Statement, the Prospectus, and the Time of Sale Information.

(u) Independent Accountants. PricewaterhouseCoopers LLP, who have certified certain financial statements of the Company and its subsidiaries, is an independent registered public accounting firm with respect to the Company and its subsidiaries within the applicable rules and regulations adopted by the Commission and the Public Company Accounting Oversight Board (United States) and as required by the Securities Act.

(v) Title to Real and Personal Property. The Company and its subsidiaries have good and marketable title in fee simple to, or have valid rights to lease or otherwise use, all items of real

and personal property that are material to the respective businesses of the Company and its subsidiaries taken as a whole, in each case free and clear of all liens, encumbrances, claims and defects and imperfections of title except those that (i) do not materially interfere with the use made and proposed to be made of such property by the Company and its subsidiaries or (ii) would not reasonably be expected, individually or in the aggregate, to have a Material Adverse Effect.

(w)Title to Intellectual Property. The Company and its subsidiaries own or license valid and enforceable rights to use all inventions, patent rights, trademarks, service marks, trade names, trade dress, domain names, copyrights, licenses, know-how, trade secrets and other unpatented and/or unpatentable proprietary or confidential information, systems or procedures and all similar intellectual property and proprietary rights (including all registrations and applications for registration of, and all goodwill associated with, any of the foregoing, as applicable) (collectively, "**Intellectual Property**") described in the Registration Statement, the Prospectus and the Time of Sale Information as being owned by or licensed to the Company and its subsidiaries, and all other Intellectual Property used in or reasonably necessary for the conduct of their business as currently conducted and as proposed to be conducted in the Registration Statement, the Prospectus and the Time of Sale Information. The conduct of the business of the Company and its subsidiaries does not, and the proposed conduct of such business as disclosed in the Registration Statement, the Prospectus and the Time of Sale Information will not, infringe, misappropriate or otherwise violate any Intellectual Property rights of any others. Except as described in the Registration Statement, the Prospectus and the Time of Sale Information, there is no pending or, to the Company's knowledge, threatened action, suit, proceeding or claim by any others (i) that the Company or any of its subsidiaries infringes, misappropriates or otherwise violates the Intellectual Property of others or (ii) challenging the validity, enforceability, scope or ownership of any Intellectual Property owned by or licensed to the Company or any of its subsidiaries or their rights therein. To the knowledge of the Company, no third party has infringed, misappropriated or otherwise violated any Intellectual Property owned by or exclusively licensed to the Company or any of its subsidiaries. To the Company's knowledge, none of the Intellectual Property used by the Company or any of its subsidiaries in the conduct of its business has been obtained or is being used by the Company or any of its subsidiaries in material violation of any contractual obligation binding on the Company or any of its subsidiaries. Except as set forth in the Registration Statement, the Prospectus and the Time of Sale Information, the Intellectual Property owned by the Company and its subsidiaries is all solely owned by the Company or its subsidiaries free and clear of any liens or encumbrances. To the Company's knowledge, no trademark, issued patent, pending patent application (if issued), copyright, or trade secret that is described in the Registration Statement, the Prospectus and the Time of Sale Information as being owned by or licensed to the Company or any of its subsidiaries is invalid or unenforceable. Neither the Company nor any of its subsidiaries is subject to any judgment, order, writ, injunction or decree of any court or any federal, state, local, foreign or other governmental department, commission, board, bureau, agency or instrumentality, domestic or foreign, or any arbitrator, nor has it entered into or is a party to any agreement made in settlement of any pending or threatened litigation, which materially restricts or impairs its use of any Intellectual Property. The Company and its subsidiaries have taken all commercially reasonable steps, in accordance with normal industry practice, necessary to maintain the confidentiality of all Intellectual Property the value of which to the Company or any of its subsidiaries is contingent upon maintaining the confidentiality thereof, except as would not reasonably be expected to have a Material Adverse Effect, and neither the Company nor any of its subsidiaries is aware of any material disclosure of such Intellectual Property other than to

employees, representatives, independent contractors, collaborators, licensors, licensees, agents and advisors of the Company and its subsidiaries, all of whom are bound by written obligations to maintain the confidentiality thereof. All founders, key employees and any other employees involved in the development of Intellectual Property for the Company and its subsidiaries have signed confidentiality and invention assignment agreements or similar agreements for the transfer, assignment, and/or licensing of Intellectual Property with the Company and its subsidiaries pursuant to which the Company and its subsidiaries either (i) have obtained ownership of and are the exclusive owners of or (ii) have obtained a valid and unrestricted right to exploit, sufficient for the conduct of their business, such Intellectual Property.

(x) No Undisclosed Relationships. No relationship, direct or indirect, exists between or among the Company or any of its subsidiaries, on the one hand, and the directors, officers, stockholders, customers, suppliers or other affiliates of the Company or any of its subsidiaries, on the other, that is required by the Securities Act to be described in each of the Registration Statement, the Prospectus and the Time of Sale Information and that is not so described in such documents.

(y) Investment Company Act. The Company is not, and immediately after giving effect to the offering and sale of the Shares and the application of the proceeds thereof as described in the Prospectus will not be, an "investment company" or an entity "controlled" by an "investment company" within the meaning of the Investment Company Act of 1940, as amended, and the rules and regulations of the Commission thereunder (collectively, the "**Investment Company Act**").

(z) Taxes. The Company and its subsidiaries have paid all federal, state, local and foreign taxes and filed all tax returns required to be paid or filed through the date hereof; and except as otherwise disclosed in each of the Registration Statement, the Prospectus and the Time of Sale Information, there is no tax deficiency that has been, or could reasonably be expected to be, asserted against the Company or any of its subsidiaries or any of their respective properties or assets that would reasonably be expected to have a Material Adverse Effect.

(aa) Licenses and Permits. The Company and its subsidiaries possess all licenses, sub-licenses, certificates, permits and other authorizations issued by, and have made all declarations and filings with, the appropriate federal, state, local or foreign governmental or regulatory authorities that are necessary for the ownership or lease of their respective properties or the conduct of their respective businesses as described in each of the Registration Statement, the Prospectus and the Time of Sale Information, except where the failure to possess or make the same would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect; and except as described in each of the Registration Statement, the Prospectus and the Time of Sale Information, neither the Company nor any of its subsidiaries has received notice of any revocation or material modification of any such license, sub-license, certificate, permit or authorization or has any reason to believe that any such license, sub-license, certificate, permit or authorization will not be renewed in the ordinary course.

(bb) No Labor Disputes. No labor disturbance by or dispute with employees of the Company or any of its subsidiaries exists or, to the knowledge of the Company, is contemplated or threatened, and the Company is not aware of any existing or imminent labor disturbance by, or dispute with, the employees of any of its or its subsidiaries' principal suppliers, contractors or

customers, except as would not reasonably be expected to have a Material Adverse Effect. Neither the Company nor any of its subsidiaries has received any notice of cancellation or termination with respect to any collective bargaining agreement to which it is a party.

(cc)Certain Environmental Laws. (i) The Company and its subsidiaries (x) are in compliance with all, and have not violated any, applicable federal, state, local and foreign laws (including common law), rules, regulations, requirements, decisions, judgments, decrees, orders and other legally enforceable requirements relating to pollution or the protection of human health or safety, the environment, natural resources, hazardous or toxic substances or wastes, pollutants or contaminants (collectively, "**Environmental Laws**"); (y) have received and are in compliance with all, and have not violated any, permits, licenses, certificates or other authorizations or approvals required of them under any Environmental Laws to conduct their respective businesses; and (z) have not received notice of any actual or potential liability or obligation under or relating to, or any actual or potential violation of, any Environmental Laws, including for the investigation or remediation of any disposal or release of hazardous or toxic substances or wastes, pollutants or contaminants, and have no knowledge of any event or condition that would reasonably be expected to result in any such notice, and (ii) there are no costs or liabilities associated with Environmental Laws of or relating to the Company or its subsidiaries, except in the case of each of (i) and (ii) above, for any such matter as would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect; and (iii) except as described in each of the Registration Statement, the Prospectus and the Time of Sale Information, (x) there is no proceeding that is pending, or that is, to the Company's knowledge, contemplated, against the Company or any of its subsidiaries under any Environmental Laws in which a governmental entity is also a party, other than such proceeding regarding which it is reasonably believed no monetary sanctions of $100,000 or more will be imposed, (y) the Company and its subsidiaries are not aware of any facts or issues regarding compliance with Environmental Laws, or liabilities or other obligations under Environmental Laws or concerning hazardous or toxic substances or wastes, pollutants or contaminants, that could reasonably be expected to have a material effect on the capital expenditures, earnings or competitive position of the Company and its subsidiaries, and (z) none of the Company or its subsidiaries anticipates material increase in capital expenditures relating to any Environmental Laws.

(dd)Compliance with ERISA. (i) Each employee benefit plan, within the meaning of Section 3(3) of the Employee Retirement Income Security Act of 1974, as amended ("**ERISA**"), for which the Company or any member of its "Controlled Group" (defined as any entity, whether or not incorporated, that is under common control with the Company within the meaning of Section 4001(a)(14) of ERISA or any entity that would be regarded as a single employer with the Company under Section 414(b), (c), (m) or (o) of the Code) would have any liability (each, a "**Plan**") has been maintained in compliance with its terms and the requirements of any applicable statutes, orders, rules and regulations, including but not limited to ERISA and the Code, except for noncompliance that would not reasonably be expected to result in material liability to the Company; (ii) no prohibited transaction, within the meaning of Section 406 of ERISA or Section 4975 of the Code, has occurred with respect to any Plan, excluding transactions effected pursuant to a statutory or administrative exemption that would not reasonably be expected to result in material liability to the Company; (iii) for each Plan that is subject to the funding rules of Section 412 of the Code or Section 302 of ERISA, no Plan has failed (whether or not waived), or is reasonably expected to fail, to satisfy the minimum funding standards (within the meaning of

Section 302 of ERISA or Section 412 of the Code) applicable to such Plan; (iv) to the knowledge of the Company, no Plan is, or is reasonably expected to be, in "at risk status" (within the meaning of Section 303(i) of ERISA) and no Plan that is a "multiemployer plan" within the meaning of Section 4001(a)(3) of ERISA is in "endangered status" or "critical status" (within the meaning of Sections 304 and 305 of ERISA) (v) the fair market value of the assets of each Plan exceeds the present value of all benefits accrued under such Plan (determined based on those assumptions used to fund such Plan); (vi) no "reportable event" (within the meaning of Section 4043(c) of ERISA and the regulations promulgated thereunder) has occurred or, to the knowledge of the Company, is reasonably expected to occur that either has resulted, or would reasonably be expected to result, in material liability to the Company; (vii) each Plan that is intended to be qualified under Section 401(a) of the Code is so qualified, and nothing has occurred, whether by action or by failure to act, which would reasonably be expected to cause the loss of such qualification; (viii) neither the Company nor any member of the Controlled Group has incurred, nor reasonably expects to incur, any liability under Title IV of ERISA (other than contributions to the Plan or premiums to the Pension Benefit Guarantee Corporation, in the ordinary course and without default) in respect of a Plan (including a "multiemployer plan" within the meaning of Section 4001(a)(3) of ERISA); and (ix) none of the following events has occurred or is reasonably likely to occur: (A) a material increase in the aggregate amount of contributions required to be made to all Plans by the Company or its Controlled Group affiliates in the current fiscal year of the Company and its Controlled Group affiliates compared to the amount of such contributions made in the Company's and its Controlled Group affiliates' most recently completed fiscal year; or (B) a material increase in the Company and its subsidiaries' "accumulated post-retirement benefit obligations" (within the meaning of Accounting Standards Codification Topic 715-60) compared to the amount of such obligations in the Company and its subsidiaries' most recently completed fiscal year, except in each case with respect to the events or conditions set forth in (i) through (ix) hereof, as would not, individually or in the aggregate, have a Material Adverse Effect.

(ee)Disclosure Controls. Except as disclosed in the Registration Statement, the Prospectus and the Time of Sale Information, the Company and its subsidiaries maintain an effective system of "disclosure controls and procedures" (as defined in Rule 13a-15(e) of the Exchange Act that complies with the requirements of the Exchange Act and that has been designed to ensure that information required to be disclosed by the Company in reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the Commission's rules and forms, including controls and procedures designed to ensure that such information is accumulated and communicated to the Company's management as appropriate to allow timely decisions regarding required disclosure.

(ff)Accounting Controls. The Company and its subsidiaries maintain systems of "internal control over financial reporting" (as defined in Rule 13a-15(f) of the Exchange Act) that have been designed to comply with the requirements of the Exchange Act and have been designed by, or under the supervision of, their respective principal executive and principal financial officers, or persons performing similar functions, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with GAAP. The Company and its subsidiaries maintain internal accounting controls sufficient to provide reasonable assurance that (i) transactions are executed in accordance with management's general or specific authorizations; (ii) transactions are recorded as necessary to permit preparation of financial statements in conformity with GAAP and to maintain asset

accountability; (iii) access to assets is permitted only in accordance with management's general or specific authorization; (iv) the recorded accountability for assets is compared with the existing assets at reasonable intervals and appropriate action is taken with respect to any differences and (v) interactive data in eXtensible Business Reporting Language included or incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information fairly presents the information called for in all material respects and is prepared in accordance with the Commission's rules and guidelines applicable thereto. Except as disclosed in the Registration Statement, the Prospectus and the Time of Sale Information, there are no material weaknesses in the Company's internal controls. The Company's auditors and the Audit Committee of the Board of Directors of the Company have been advised of: (i) all significant deficiencies and material weaknesses, if any, in the design or operation of internal controls over financial reporting which have adversely affected or are reasonably likely to adversely affect the Company's ability to record, process, summarize and report financial information; and (ii) to the knowledge of the Company, any fraud, whether or not material, that involves management or other employees who have a significant role in the Company's internal controls over financial reporting.

(gg)Insurance. The Company and its subsidiaries have insurance covering their respective properties, operations, personnel and businesses, including business interruption insurance, which insurance is in amounts and insures against such losses and risks which the Company reasonably believes are adequate to protect the Company and its subsidiaries and their respective businesses; and neither the Company nor any of its subsidiaries has (i) received notice from any insurer or agent of such insurer that capital improvements or other expenditures are required or necessary to be made in order to continue such insurance or (ii) any reason to believe that it will not be able to renew its existing insurance coverage as and when such coverage expires or to obtain similar coverage at reasonable cost from similar insurers as may be necessary to continue its business.

(hh)No Unlawful Payments. Neither the Company nor any of its subsidiaries, nor any director, officer or employee of the Company or any of its subsidiaries nor, to the knowledge of the Company, any agent, affiliate or other person associated with or acting on behalf of the Company or any of its subsidiaries has (i) used any corporate funds for any unlawful contribution, gift, entertainment or other unlawful expense relating to political activity; (ii) made or taken an act in furtherance of an offer, promise or authorization of any direct or indirect unlawful payment or benefit to any foreign or domestic government official or employee, including of any government-owned or controlled entity or of a public international organization, or any person acting in an official capacity for or on behalf of any of the foregoing, or any political party or party official or candidate for political office; (iii) violated or is in violation of any provision of the Foreign Corrupt Practices Act of 1977, as amended, or any applicable law or regulation implementing the OECD Convention on Combating Bribery of Foreign Public Officials in International Business Transactions, or committed an offence under the Bribery Act 2010 of the United Kingdom, or any other applicable anti-bribery or anti-corruption law; or (iv) made, offered, agreed, requested or taken an act in furtherance of any unlawful bribe or other unlawful benefit, including, without limitation, any rebate, payoff, influence payment, kickback or other unlawful or improper payment or benefit. The Company and its subsidiaries have instituted, maintain and enforce, and will continue to maintain and enforce policies and procedures designed to promote and ensure compliance with all applicable anti-bribery and anti-corruption laws.

(ii)Cybersecurity. Except as disclosed in the Registration Statement, the Prospectus and the Time of Sale Information, (i)(x) there has been no security breach or other compromise of or relating to any of the Company's information technology and computer systems, networks, hardware, software, data (including the data of its customers, employees, suppliers, vendors and any third party data maintained by or on behalf of the Company), equipment or technology (collectively, "**IT Systems and Data**") and (y) the Company has not been notified of, and has no knowledge of any event or condition that would reasonably be expected to result in, any security breach or other compromise to its IT Systems and Data; (ii) the Company is presently in compliance with all applicable laws or statutes and all judgments, orders, rules and regulations of any court or arbitrator or governmental or regulatory authority, internal policies and contractual obligations relating to the privacy and security of IT Systems and Data and to the protection of such IT Systems and Data from unauthorized use, access, misappropriation or modification, except as would not, in the case of clause (i) and (ii), individually or in the aggregate, have a Material Adverse Effect; and (iii) the Company has implemented backup and disaster recovery technology consistent with industry standards and practices.

(jj)Compliance with Anti-Money Laundering Laws. The operations of the Company and its subsidiaries are and have been conducted at all times in compliance with applicable financial recordkeeping and reporting requirements, including those of the Currency and Foreign Transactions Reporting Act of 1970, as amended, the applicable money laundering statutes of all jurisdictions where the Company or any of its subsidiaries conducts business, the rules and regulations thereunder and any related or similar rules, regulations or guidelines, issued, administered or enforced by any governmental agency (collectively, the "**Anti-Money Laundering Laws**"), and no action, suit or proceeding by or before any court or governmental agency, authority or body or any arbitrator involving the Company or any of its subsidiaries with respect to the Anti-Money Laundering Laws is pending or, to the knowledge of the Company, threatened.

(kk)No Conflicts with Sanctions Laws. Neither the Company nor any of its subsidiaries, directors, officers or employees, nor, to the knowledge of the Company, any agent, affiliate or other person associated with or acting on behalf of the Company or any of its subsidiaries is currently the subject or the target of any sanctions administered or enforced by the U.S. government (including, without limitation, the Office of Foreign Assets Control of the U.S. Department of the Treasury or the U.S. Department of State and including, without limitation, the designation as a "specially designated national" or "blocked person"), the United Nations Security Council, the European Union, His Majesty's Treasury or other relevant sanctions authority (collectively, "**Sanctions**"), nor is the Company or any of its subsidiaries located, organized or resident in a country or territory that is the subject or target of Sanctions, including, without limitation, the so-called Donetsk People's Republic, or so-called Luhansk People's Republic or any other Covered Region of Ukraine identified pursuant to Executive Order 14065, and the Crimea region, Cuba, Iran, North Korea and Syria (each, a "**Sanctioned Territory**"); and the Company will not directly or indirectly use the proceeds of the offering of the Shares hereunder, or lend, contribute or otherwise make available such proceeds to any subsidiary, joint venture partner or other person or entity (i) to fund or facilitate any activities of or business with any person that, at the time of such funding or facilitation, is the subject or target of Sanctions, (ii) to fund or facilitate any activities of or business in any Sanctioned Territory or (iii) in any other manner that will result in a violation by any person (including any person participating in the transaction,

whether as agent, advisor, investor or otherwise) of Sanctions. For the past five years, the Company and its subsidiaries have not knowingly engaged in and are not now knowingly engaged in any dealings or transactions with any person that at the time of the dealing or transaction is or was the subject or the target of Sanctions or with any Sanctioned Territory.

(ll)No Restrictions on Subsidiaries. No subsidiary of the Company is currently prohibited, directly or indirectly, under any agreement or other instrument to which it is a party or is subject, from paying any dividends to the Company, from making any other distribution on such subsidiary's capital stock or similar ownership interest, from repaying to the Company any loans or advances to such subsidiary from the Company or from transferring any of such subsidiary's properties or assets to the Company or any other subsidiary of the Company.

(mm)No Broker's Fees. Neither the Company nor any of its subsidiaries is a party to any contract, agreement or understanding with any person (other than this Agreement) that would give rise to a valid claim against any of them for a brokerage commission, finder's fee or like payment in connection with the transactions contemplated by this Agreement or any Prospectus.

(nn)No Registration Rights. Except as disclosed in the Registration Statement, the Prospectus and the Time of Sale Information, no person has the right to require the Company or any of its subsidiaries to register any securities for sale under the Securities Act by reason of the filing of the Registration Statement with the Commission or the issuance and sale of the Shares, other than rights that have been validly waived.

(oo)No Stabilization. Neither the Company nor any of its subsidiaries has taken, directly or indirectly, without given effect to activities by the Agent, any action designed to or that would reasonably be expected to cause or result in any stabilization or manipulation of the price of the Shares or any other "**reference security**" (as defined in Rule 100 of Regulation M under the Exchange Act (**"Regulation M"**)) whether to facilitate the sale or resale of the Shares or otherwise, and has taken no action which would directly or indirectly violate Regulation M. The Company acknowledges that the Agent may engage in passive market making transactions in the Shares on the Nasdaq Global Select Market in accordance with Regulation M.

(pp)Margin Rules. Neither the issuance, sale and delivery of the Shares nor the application of the proceeds thereof by the Company as described in each of the Registration Statement, the Prospectus and the Time of Sale Information will violate Regulation T, U or X of the Board of Governors of the Federal Reserve System or any other regulation of such Board of Governors.

(qq)Forward-Looking Statements. No forward-looking statement (within the meaning of Section 27A of the Securities Act and Section 21E of the Exchange Act) included or incorporated by reference in any of the Registration Statement, the Prospectus or the Time of Sale Information has been made or reaffirmed without a reasonable basis or has been disclosed other than in good faith.

(rr)Statistical and Market Data. Nothing has come to the attention of the Company that has caused the Company to believe that the statistical and market-related data included or incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale

Information is not based on or derived from sources that are reliable and accurate in all material respects.

(ss)eXtensible Business Reporting Language. The interactive data in eXtensible Business Reporting Language included or incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information fairly presents the information called for in all material respects and has been prepared in accordance with the Commission's rules and guidelines applicable thereto.

(tt)Preclinical Studies and Clinical Trials. (i) Except as described in the Registration Statement, the Prospectus and the Time of Sale Information, the preclinical studies and clinical trials conducted by or, to the knowledge of the Company, on behalf of or sponsored by the Company or its subsidiaries, or in which the Company or its subsidiaries have participated, that are described in the Registration Statement, the Prospectus and the Time of Sale Information, or the results of which are referred to in the Registration Statement, the Prospectus and the Time of Sale Information, as applicable, were, and if still pending are, being conducted in all material respects in accordance with standard medical and scientific research standards and procedures for products or product candidates comparable to those being developed by the Company and all applicable statutes and all applicable rules and regulations of the U.S. Food and Drug Administration and comparable regulatory agencies outside of the United States to which they are subject, including the European Medicines Agency (collectively, the "**Regulatory Authorities**") and Good Clinical Practice and Good Laboratory Practice requirements; (ii) the descriptions in the Registration Statement, the Prospectus and the Time of Sale Information of the results of such studies and trials are accurate and complete descriptions in all material respects and fairly present the data derived therefrom; (iii) the Company has no knowledge of any other studies or trials not described in the Registration Statement, the Prospectus and the Time of Sale Information, the results of which the Company believes are inconsistent with or reasonably call into question the results described or referred to in the Registration Statement, the Prospectus and the Time of Sale Information; (iv) the Company and its subsidiaries have operated at all times and are currently in compliance with all applicable statutes, rules and regulations of the Regulatory Authorities, except where such non-compliance would not, individually or in the aggregate, have a Material Adverse Effect; (v) the Company has provided the Agent with all substantive written notices, correspondence and summaries of all other communications from the Regulatory Authorities; and (vi) neither the Company nor any of its subsidiaries have received any written notices, correspondence or other communications from the Regulatory Authorities or any other governmental agency requiring or threatening the termination, material modification or suspension of any preclinical studies or clinical trials that are described in the Registration Statement, the Prospectus and the Time of Sale Information or the results of which are referred to in the Registration Statement, the Prospectus and the Time of Sale Information, other than ordinary course communications with respect to modifications in connection with the design and implementation of such studies or trials, and, to the Company's knowledge, there are no reasonable grounds for the same.

(uu)Regulatory Filings. The Company has not failed to file with the Regulatory Authorities any required filing, declaration, listing, registration, report or submission with respect to the Company's product candidates that are described or referred to in the Registration Statement, the Prospectus and the Time of Sale Information; all such filings, declarations, listings,

registrations, reports or submissions, as applicable, were in material compliance with applicable laws when filed; and no material deficiencies regarding compliance with applicable law have been asserted by any applicable regulatory authority with respect to any such filings, declarations, listings, registrations, reports or submissions.

(vv) Sarbanes-Oxley Act. There is and has been no material failure on the part of the Company or, to the Company's knowledge, any of the Company's directors or officers, in their capacities as such, to comply with any applicable provision of the Sarbanes-Oxley Act of 2002, as amended, and the rules and regulations promulgated in connection therewith, including Section 402 related to loans and Sections 302 and 906 related to certifications.

Any certificate signed by any officer or representative of the Company or any of its subsidiaries and delivered to the Agent or counsel for the Agent in connection with an issuance of Shares shall be deemed a representation and warranty by the Company to the Agent as to the matters covered thereby on the date of such certificate.

The Company acknowledges that the Agent and, for purposes of the opinions to be delivered pursuant to Section 4(p) hereof, counsel to the Company and counsel to the Agent, will rely upon the accuracy and truthfulness of the foregoing representations and hereby consents to such reliance.

Section 3. ISSUANCE AND SALE OF COMMON SHARES

(a) (i) Sale of Securities. On the basis of the representations, warranties and agreements herein contained, but subject to the terms and conditions herein set forth, the Company and the Agent agree that the Company may from time to time seek to sell Shares through the Agent, acting as sales agent, or directly to the Agent, acting as principal, as follows, with an aggregate Sales Price of up to the Maximum Program Amount, based on and in accordance with Issuance Notices as the Company may deliver, during the Agency Period.

(b) Mechanics of Issuances.

(i) Issuance Notice. Upon the terms and subject to the conditions set forth herein, on any Trading Day during the Agency Period on which the conditions set forth in Section 5(a) and Section 5(b) shall have been satisfied, the Company may exercise its right to request an issuance of Shares by delivering to the Agent an Issuance Notice; *provided, however*, that (A) in no event may the Company deliver an Issuance Notice to the extent that (I) the sum of (x) the aggregate Sales Price of the requested Issuance Amount, plus (y) the aggregate Sales Price of all Shares issued under all previous Issuance Notices effected pursuant to this Agreement, would exceed the Maximum Program Amount; and (B) prior to delivery of any Issuance Notice, the Selling Period for any previous Issuance Notice shall have expired or been terminated. An Issuance Notice shall be considered delivered on the Trading Day that it is received by e-mail to the persons so identified in writing by the Agent and confirmed by the Company by telephone (including a voicemail message to the persons so identified), with the understanding that, with adequate prior written notice, the Agent may modify the list of such persons from time to time.

(ii) Agent Efforts. Upon the terms and subject to the conditions set forth in this Agreement, upon the receipt of an Issuance Notice, the Agent will use its commercially reasonable

efforts consistent with its normal sales and trading practices to place the Shares with respect to which the Agent has agreed to act as sales agent, subject to, and in accordance with the information specified in, the Issuance Notice, unless the sale of the Shares described therein has been suspended, cancelled or otherwise terminated in accordance with the terms of this Agreement. For the avoidance of doubt, the parties to this Agreement may modify an Issuance Notice at any time provided they both agree in writing to any such modification.

(iii) Method of Offer and Sale. The Shares may be offered and sold (A) in privately negotiated transactions, (B) as block transactions or (C) by any other method or payment permitted by law deemed to be an "at the market" offering as defined in Rule 415 under the Securities Act, including sales made directly on the Principal Market, sales made into any other existing trading market or sales made to or through a market maker or through an electronic communications network. Nothing in this Agreement shall be deemed to require either party to agree to the method of offer and sale specified in the preceding sentence, and the method of placement of any Shares by the Agent shall be at the Agent's discretion.

(iv) Confirmation to the Company. If acting as sales agent hereunder, the Agent will provide written confirmation to the Company no later than the opening of the Trading Day next following the Trading Day on which it has placed Shares hereunder setting forth the number of shares sold on such Trading Day, the corresponding Sales Price and the Issuance Price payable to the Company in respect thereof.

(v) Settlement. Each issuance of Shares will be settled on the applicable Settlement Date for such issuance of Shares and, subject to the provisions of Section 5, on or before each Settlement Date, the Company will, or will cause its transfer agent to, electronically transfer the Shares being sold by crediting the Agent or its designee's account at The Depository Trust Company through its Deposit/Withdrawal At Custodian (DWAC) System, or by such other means of delivery as may be mutually agreed upon by the parties hereto and, upon receipt of such Shares, which in all cases shall be freely tradable, transferable, registered shares in good deliverable form, the Agent will deliver, by wire transfer of immediately available funds, the related aggregate Issuance Price in same day funds delivered to an account designated by the Company prior to the Settlement Date. The Company may sell Shares to the Agent as principal at a price agreed upon at each relevant time Shares are sold pursuant to this Agreement (each, a "**Time of Sale**").

(vi) Suspension or Termination of Sales. Consistent with standard market settlement practices, the Company or the Agent may, upon notice to the other party hereto in writing or by telephone (confirmed immediately by verifiable email), suspend any sale of Shares, and the Selling Period shall immediately terminate; *provided, however*, that (A) such suspension and termination shall not affect or impair either party's obligations with respect to any Shares placed or sold hereunder prior to the receipt of such notice; (B) if the Company suspends or terminates any sale of Shares after the Agent confirms such sale to the Company, the Company shall still be obligated to comply with Section 3(b)(v) with respect to such Shares; and (C) if the Company defaults in its obligation to deliver Shares on a Settlement Date, the Company agrees that it will hold the Agent harmless against any loss, claim, damage or expense (including, without limitation, penalties, interest and reasonable legal fees and expenses), as incurred, arising out of or in connection with such default by the Company. The parties hereto acknowledge and agree that, in performing its obligations under this Agreement, the Agent may borrow Common Shares from stock lenders in

the event that the Company has not delivered Shares to settle sales as required by subsection (v) above, and may use the Shares to settle or close out such borrowings. The Company agrees that no such notice shall be effective against the Agent unless it is made to the persons identified in writing by the Agent pursuant to Section 3(b)(i).

(vii) No Guarantee of Placement, Etc. The Company acknowledges and agrees that (A) there can be no assurance that the Agent will be successful in placing Shares, (B) the Agent will incur no liability or obligation to the Company or any other Person if it does not sell Shares and (C) the Agent shall be under no obligation to purchase Shares on a principal basis pursuant to this Agreement, except as otherwise specifically agreed by the Agent and the Company.

(viii) Material Non-Public Information. Notwithstanding any other provision of this Agreement, the Company and the Agent agree that the Company shall not deliver any Issuance Notice to the Agent, and the Agent shall not be obligated to place any Shares, during any period in which the Company is in possession of material non-public information.

(c) Fees. As compensation for services rendered, the Company shall pay to the Agent, on the applicable Settlement Date, the Selling Commission for the applicable Issuance Amount (including with respect to any suspended or terminated sale pursuant to Section 3(b)(vi)) by the Agent deducting the Selling Commission from the applicable Issuance Amount.

(d) Expenses. The Company agrees to pay all costs, fees and expenses incurred in connection with the performance of its obligations hereunder and in connection with the transactions contemplated hereby, including without limitation (i) all expenses incident to the issuance and delivery of the Shares (including all printing and engraving costs), (ii) all fees and expenses of the registrar and transfer agent of the Shares, (iii) all necessary issue, transfer and other stamp taxes in connection with the issuance and sale of the Shares, (iv) all fees and expenses of the Company's counsel, independent public or certified public accountants and other advisors, (v) all costs and expenses incurred in connection with the preparation, printing, filing, shipping and distribution of the Registration Statement (including financial statements, exhibits, schedules, consents and certificates of experts), the Prospectus, any Free Writing Prospectus (as defined below) prepared by or on behalf of, used by, or referred to by the Company, and all amendments and supplements thereto, and this Agreement, (vi) all filing fees, attorneys' fees and expenses incurred by the Company or the Agent in connection with qualifying or registering (or obtaining exemptions from the qualification or registration of) all or any part of the Shares for offer and sale under the state securities or blue sky laws or the provincial securities laws of Canada, and, if requested by the Agent, preparing and printing a "**Blue Sky Survey**" or memorandum and a "Canadian wrapper", and any supplements thereto, advising the Agent of such qualifications, registrations, determinations and exemptions, (vii) the reasonable fees and disbursements of the Agent's counsel, including the reasonable fees and expenses of counsel for the Agent in connection with FINRA review, if any, and approval of the Agent's participation in the offering and distribution of the Shares, (viii) the filing fees incident to FINRA review, if any, (ix) the costs and expenses of the Company relating to investor presentations on any "road show" undertaken in connection with the marketing of the offering of the Shares, including, without limitation, expenses associated with the preparation or dissemination of any electronic road show, expenses associated with the production of road show slides and graphics, fees and expenses of any consultants engaged in connection with the road show presentations with the prior approval of the Company, travel and

lodging expenses of the representatives, employees and officers of the Company and of the Agent and any such consultants, and the cost of any aircraft chartered in connection with the road show and (x) the fees and expenses associated with listing the Shares on the Nasdaq Global Select Market.

Section 4. ADDITIONAL COVENANTS

The Company covenants and agrees with the Agent as follows, in addition to any other covenants and agreements made elsewhere in this Agreement:

(a) Exchange Act Compliance. During the Agency Period, the Company shall (i) file, on a timely basis, with the Commission all reports and documents required to be filed under Section 13, 14 or 15 of the Exchange Act in the manner and within the time periods required by the Exchange Act and (ii) either (A) include in its quarterly reports on Form 10-Q and its annual reports on Form 10-K, a summary detailing, for the relevant reporting period, (1) the number of Shares sold through the Agent pursuant to this Agreement and (2) the net proceeds received by the Company from such sales or (B) prepare a prospectus supplement or in such other filing permitted by the Securities Act or Exchange Act (each an "**Interim Prospectus Supplement**") with such summary information and, at least once a quarter and subject to this Section 4, file such Interim Prospectus Supplement pursuant to Rule 424(b) under the Securities Act (and within the time periods required by Rule 424(b) and Rule 430B under the Securities Act)).

(b) Securities Act Compliance. After the date of this Agreement, the Company shall promptly advise the Agent in writing (i) of the receipt of any comments of, or requests for additional or supplemental information from, the Commission, (ii) of the time and date of any filing of any post-effective amendment to the Registration Statement, any Rule 462(b) Registration Statement or any amendment or supplement to the Prospectus, any Free Writing Prospectus, (iii) of the time and date that any post-effective amendment to the Registration Statement or any Rule 462(b) Registration Statement becomes effective and (iv) of the issuance by the Commission of any stop order suspending the effectiveness of the Registration Statement or any post-effective amendment thereto, any Rule 462(b) Registration Statement or any amendment or supplement to the Prospectus or of any order preventing or suspending the use of any Free Writing Prospectus or the Prospectus, or of any proceedings to remove, suspend or terminate from listing or quotation the Common Shares from any securities exchange upon which they are listed for trading or included or designated for quotation, or of the threatening or initiation of any proceedings for any of such purposes. If the Commission shall enter any such stop order at any time, the Company will use its best efforts to obtain the lifting of such order at the earliest possible moment. Additionally, the Company agrees that it shall comply with the provisions of Rule 424(b) and Rule 433, as applicable, under the Securities Act and will use its reasonable efforts to confirm that any filings made by the Company under such Rule 424(b) or Rule 433 were received in a timely manner by the Commission.

(c) Amendments and Supplements to the Prospectus and Other Securities Act Matters. If any event shall occur or condition exist as a result of which it is necessary to amend or supplement the Prospectus so that the Prospectus does not include an untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances when the Prospectus is delivered to a purchaser, not misleading, or if in the

opinion of the Agent or counsel for the Agent it is otherwise necessary to amend or supplement the Prospectus to comply with applicable law, including the Securities Act, the Company agrees (subject to Section 3(b) and 3(c)) to promptly prepare, file with the Commission and furnish at its own expense to the Agent, amendments or supplements to the Prospectus so that the statements in the Prospectus as so amended or supplemented will not include an untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances when the Prospectus is delivered to a purchaser, be misleading or so that the Prospectus, as amended or supplemented, will comply with applicable law including the Securities Act. Neither the Agent's consent to, or delivery of, any such amendment or supplement shall constitute a waiver of any of the Company's obligations under Sections 3(b) or (c).

(d) Agent's Review of Proposed Amendments and Supplements. Prior to amending or supplementing the Registration Statement (including any Rule 462(b) Registration Statement) under the Securities Act, but excluding (i) documents incorporated by reference filed under the Exchange Act that either (A) do not name Agent and do not relate to the transactions contemplated by this Agreement or (B) only include disclosure relating to periodic sales pursuant to this Agreement, and (ii) amendments or supplements that do not name Agent and do not relate to the transactions contemplated by this Agreement) or the Prospectus (excluding documents incorporated by reference filed under the Exchange Act that either (A) do not name Agent and do not relate to the transactions contemplated by this Agreement or (B) only include disclosure relating to periodic sales pursuant to this Agreement), the Company shall furnish to the Agent for review, a reasonable amount of time prior to the proposed time of filing or use thereof, a copy of each such proposed amendment or supplement, and the Company shall not file or use any such proposed amendment or supplement without the Agent's prior consent, and to file with the Commission within the applicable period specified in Rule 424(b) under the Securities Act any prospectus required to be filed pursuant to such Rule.

(e) Use of Free Writing Prospectus. Neither the Company nor the Agent has prepared, used, referred to or distributed, or will prepare, use, refer to or distribute, without the other party's prior written consent, any "written communication" that constitutes a "free writing prospectus" as such terms are defined in Rule 405 under the Securities Act with respect to the offering contemplated by this Agreement (any such free writing prospectus being referred to herein as a "**Free Writing Prospectus**").

(f) Free Writing Prospectuses. The Company shall furnish to the Agent for review, a reasonable amount of time prior to the proposed time of filing or use thereof, a copy of each proposed free writing prospectus or any amendment or supplement thereto to be prepared by or on behalf of, used by, or referred to by the Company and the Company shall not file, use or refer to any proposed free writing prospectus or any amendment or supplement thereto without the Agent's consent. The Company shall furnish to the Agent, without charge, as many copies of any free writing prospectus prepared by or on behalf of, or used by the Company, as the Agent may reasonably request. If at any time when a prospectus is required by the Securities Act (including, without limitation, pursuant to Rule 173(d)) to be delivered in connection with sales of the Shares (but in any event if at any time through and including the date of this Agreement) there occurred or occurs an event or development as a result of which any free writing prospectus prepared by or on behalf of, used by, or referred to by the Company conflicted or would conflict with the information contained in the Registration Statement or included or would include an untrue

statement of a material fact or omitted or would omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances prevailing at that subsequent time, not misleading, the Company shall promptly amend or supplement such free writing prospectus to eliminate or correct such conflict or so that the statements in such free writing prospectus as so amended or supplemented will not include an untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances prevailing at such subsequent time, not misleading, as the case may be; *provided, however*, that prior to amending or supplementing any such free writing prospectus, the Company shall furnish to the Agent for review, a reasonable amount of time prior to the proposed time of filing or use thereof, a copy of such proposed amended or supplemented free writing prospectus and the Company shall not file, use or refer to any such amended or supplemented free writing prospectus without the Agent's consent.

(g) Filing of Agent Free Writing Prospectuses. The Company shall not take any action that would result in the Agent or the Company being required to file with the Commission pursuant to Rule 433(d) under the Securities Act a free writing prospectus prepared by or on behalf of the Agent that the Agent otherwise would not have been required to file thereunder.

(h) Copies of Registration Statement and Prospectus. After the date of this Agreement through the last time that a prospectus is required by the Securities Act (including, without limitation, pursuant to Rule 173(d)) to be delivered in connection with sales of the Shares, the Company agrees to furnish the Agent with copies (which may be electronic copies) of the Registration Statement and each amendment thereto, and with copies of the Prospectus and each amendment or supplement thereto in the form in which it is filed with the Commission pursuant to the Securities Act or Rule 424(b) under the Securities Act, both in such quantities as the Agent may reasonably request from time to time; and, if the delivery of a prospectus is required under the Securities Act or under the blue sky or securities laws of any jurisdiction at any time on or prior to the applicable Settlement Date for any Selling Period in connection with the offering or sale of the Shares and if at such time any event has occurred as a result of which the Prospectus as then amended or supplemented would include an untrue statement of a material fact or omit to state any material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made when such Prospectus is delivered, not misleading, or, if for any other reason it is necessary during such same period to amend or supplement the Prospectus or to file under the Exchange Act any document incorporated by reference in the Prospectus in order to comply with the Securities Act or the Exchange Act, to notify the Agent and to request that the Agent suspend offers to sell Shares (and, if so notified, the Agent shall cease such offers as soon as practicable); and if the Company decides to amend or supplement the Registration Statement or the Prospectus as then amended or supplemented, to advise the Agent promptly by telephone (with confirmation in writing) and to prepare and cause to be filed promptly with the Commission an amendment or supplement to the Registration Statement or the Prospectus as then amended or supplemented that will correct such statement or omission or effect such compliance (it being acknowledged that the Company may delay the filing of any amendment or supplement, if, in the judgment of the Company, it is in the best interest of the Company); provided, however, that if during such same period the Agent is required to deliver a prospectus in respect of transactions in the Shares, the Company shall promptly prepare and file with the Commission such an amendment or supplement.

(i) Blue Sky Compliance. The Company shall cooperate with the Agent and counsel for the Agent to qualify or register the Shares for sale under (or obtain exemptions from the application of) the state securities or blue sky laws or Canadian provincial securities laws of those jurisdictions designated by the Agent, shall comply with such laws and shall continue such qualifications, registrations and exemptions in effect so long as required for the distribution of the Shares. The Company shall not be required to qualify as a foreign corporation or to take any action that would subject it to general service of process in any such jurisdiction where it is not presently qualified or where it would be subject to taxation as a foreign corporation. The Company will advise the Agent promptly of the suspension of the qualification or registration of (or any such exemption relating to) the Shares for offering, sale or trading in any jurisdiction or any initiation or threat of any proceeding for any such purpose, and in the event of the issuance of any order suspending such qualification, registration or exemption, the Company shall use its best efforts to obtain the withdrawal thereof at the earliest possible moment.

(j) Earnings Statement. As soon as practicable, the Company will make generally available to its security holders and to the Agent an earnings statement (which need not be audited) covering a period of at least twelve months beginning with the first fiscal quarter of the Company occurring after the date of this Agreement which shall satisfy the provisions of Section 11(a) of the Securities Act and Rule 158 under the Securities Act; provided that the Company will be deemed to have furnished such statements to its security holders and the Agent to the extent they are filed on EDGAR or any successor system.

(k) Listing; Reservation of Shares. (a) The Company will maintain the listing of the Shares on the Nasdaq Global Select Market, and (b) the Company will reserve and keep available at all times, free of preemptive rights, Shares for the purpose of enabling the Company to satisfy its obligations under this Agreement.

(l) Transfer Agent. The Company shall engage and maintain, at its expense, a registrar and transfer agent for the Shares.

(m) Due Diligence. During the term of this Agreement, the Company will reasonably cooperate with any reasonable due diligence review conducted by the Agent in connection with the transactions contemplated hereby, including, without limitation, providing information and making available documents and senior corporate officers, during normal business hours and at the Company's principal offices, as the Agent may reasonably request from time to time.

(n) Representations and Warranties. The Company acknowledges that each delivery of an Issuance Notice and each delivery of Shares on a Settlement Date shall be deemed to be (i) an affirmation to the Agent that the representations and warranties of the Company contained in or made pursuant to this Agreement are true and correct as of the date of such Issuance Notice or of such Settlement Date, as the case may be, as though made at and as of each such date, except as may be disclosed in the Prospectus (including any documents incorporated by reference therein and any supplements thereto), and (ii) an undertaking that the Company will advise the Agent if any of such representations and warranties will not be true and correct as of the Settlement Date for the Shares relating to such Issuance Notice, as though made at and as of each such date (except that such representations and warranties shall be deemed to relate to the Registration Statement and the Prospectus as amended and supplemented relating to such Shares).

(o) Deliverables at Triggering Event Dates; Certificates. The Company agrees that on or prior to the date of the first Issuance Notice and, during the term of this Agreement after the date of the first Issuance Notice, upon:

(i) the filing of the amendment or supplement of any Registration Statement or Prospectus (other than a prospectus supplement relating solely to an offering of securities other than the Shares or a prospectus filed pursuant to Section 4(a)(ii)(B)), by means of a post-effective amendment, sticker or supplement, but not by means of incorporation of documents by reference into the Registration Statement or Prospectus;

(ii) the filing with the Commission of an annual report on Form 10-K or a quarterly report on Form 10-Q (including any Form 10-K/A or Form 10-Q/A containing amended financial information or a material amendment to the previously filed annual report on Form 10-K or quarterly report on Form 10-Q), in each case, of the Company; or

(iii) the filing with the Commission of a current report on Form 8-K of the Company containing amended financial information (other than information "furnished" pursuant to Item 2.02 or 7.01 of Form 8-K or to provide disclosure pursuant to Item 8.01 of Form 8-K relating to reclassification of certain properties as discontinued operations in accordance with Statement of Financial Accounting Standards No. 144) that is material to the offering of securities of the Company in the Agent's reasonable discretion;

(any such event, a "**Triggering Event Date**"), the Company shall furnish the Agent (but in the case of clause (iii) above only if the Agent reasonably determines that the information contained in such current report on Form 8-K of the Company is material) with a certificate as of the Triggering Event Date, in the form and substance satisfactory to the Agent and its counsel, substantially similar to the form previously provided to the Agent and its counsel, modified, as necessary, to relate to the Registration Statement and the Prospectus as amended or supplemented, (A) confirming that the representations and warranties of the Company contained in this Agreement are true and correct, (B) that the Company has performed all of its obligations hereunder to be performed on or prior to the date of such certificate and as to the matters set forth in Section 5(a)(i) hereof, and (C) containing any other certification that the Agent shall reasonably request. The requirement to provide a certificate under this Section 4(o) shall be waived for any Triggering Event Date occurring at a time when no Issuance Notice is pending or a suspension pursuant to Section 3(b)(vi) is in effect, which waiver shall continue until the earlier to occur of the date the Company delivers instructions for the sale of Shares hereunder (which for such calendar quarter shall be considered a Triggering Event Date) and the next occurring Triggering Event Date. Notwithstanding the foregoing, if the Company subsequently decides to sell Shares following a Triggering Event Date when a suspension was in effect and did not provide the Agent with a certificate under this Section 4(o), then before the Company delivers the instructions for the sale of Shares or the Agent sells any Shares pursuant to such instructions, the Company shall provide the Agent with a certificate in conformity with this Section 4(o) dated as of the date that the instructions for the sale of Shares are issued.

(p) Legal Opinions. On or prior to the date of the first Issuance Notice and within five (5) Trading Days of each Triggering Event Date with respect to which the Company is obligated to deliver a certificate pursuant to Section 4(o) for which no waiver is applicable and excluding

the date of this Agreement, a negative assurances letter and the written legal opinion of Wilmer Cutler Pickering Hale and Dorr LLP, counsel to the Company, and the written legal opinions of McCarter & English, LLP and Knobbe, Martens, Olson & Bear, LLP, intellectual property counsels to the Company, or if one or more of such intellectual property counsel are no longer counsel to the Company, the opinion of such other intellectual property counsel to the Company reasonably satisfactory to the Agent, each dated the date of delivery, in form and substance reasonably satisfactory to Agent and its counsel, substantially similar to the form previously provided to the Agent and its counsel, modified, as necessary, to relate to the Registration Statement and the Prospectus as then amended or supplemented; *provided* that the Company shall be required to furnish no more than one opinion from each of Company's counsel per fiscal quarter. In lieu of such opinions for subsequent periodic filings, in the discretion of the Agent, the Company may furnish a reliance letter from such counsel to the Agent, permitting the Agent to rely on a previously delivered opinion letter, modified as appropriate for any passage of time or Triggering Event Date (except that statements in such prior opinion shall be deemed to relate to the Registration Statement and the Prospectus as amended or supplemented as of such Triggering Event Date).

(q) Comfort Letter. On or prior to the date of the first Issuance Notice and within five (5) Trading Days of each Triggering Event Date with respect to which the Company is obligated to deliver a certificate pursuant to Section 4(o) for which no waiver is applicable and excluding the date of this Agreement, the Company shall cause PricewaterhouseCoopers LLP, the independent registered public accounting firm who has audited the financial statements included or incorporated by reference in the Registration Statement, to furnish the Agent a comfort letter, dated the date of delivery, in form and substance reasonably satisfactory to the Agent and its counsel, substantially similar to the form previously provided to the Agent and its counsel; provided, however, that any such comfort letter will only be required on the Triggering Event Date specified to the extent that it contains financial statements filed with the Commission under the Exchange Act and incorporated or deemed to be incorporated by reference into a Prospectus and *provided* further that the Company shall be required to furnish no more than one comfort letter per fiscal quarter. If requested by the Agent, the Company shall also cause a comfort letter to be furnished to the Agent on the date of occurrence of any material transaction or event requiring the filing of a current report on Form 8-K containing material amended financial information of the Company, including the restatement of the Company's financial statements.

(r) Secretary's Certificate. On or prior to the date of the first Issuance Notice and within five (5) Trading Days of each Triggering Event Date with respect to which the Company is obligated to deliver a certificate pursuant to Section 4(o) for which no waiver is applicable and excluding the date of this Agreement, the Company shall furnish the Agent a certificate executed by the Secretary of the Company, signing in such capacity, dated the Applicable Time (i) certifying that attached thereto are true and complete copies of the resolutions duly adopted by the Board of Directors of the Company authorizing the execution and delivery of this Agreement and the consummation of the transactions contemplated hereby (including, without limitation, the issuance of the Shares pursuant to this Agreement), which authorization shall be in full force and effect on and as of the date of such certificate, (ii) certifying and attesting to the office, incumbency, due authority and specimen signatures of each Person who executed this Agreement for or on behalf of the Company, and (iii) containing any other certification that the Agent or its counsel shall reasonably request.

(r) Officer's Certificate. On or prior to the date of the first Issuance Notice and within five (5) Trading Days of each Triggering Event Date with respect to which the Company is obligated to deliver a certificate pursuant to Section 4(o) for which no waiver is applicable and excluding the date of this Agreement, the Company shall furnish the Agent a certificate executed by the Chief Executive Officer, President or Chief Financial Officer of the Company, signing in such capacity, dated the Applicable Time to the effect that all conditions to the delivery of the applicable Issuance Notice shall have been satisfied as at the date of such certificate (which certificate shall not be required if the foregoing representations shall be set forth in the Issuance Notice).

(s) Agent's Own Account; Clients' Account. The Company consents to the Agent trading, in compliance with applicable law, in the Common Shares for the Agent's own account and for the account of its clients at the same time as sales of the Shares occur pursuant to this Agreement.

(t) Investment Limitation. The Company shall not invest, or otherwise use the proceeds received by the Company from its sale of the Shares in such a manner as would require the Company or any of its subsidiaries to register as an investment company under the Investment Company Act.

(u) Market Activities. The Company will not take, directly or indirectly, any action designed to or that might be reasonably expected to cause or result in stabilization or manipulation of the price of the Shares or any other reference security, whether to facilitate the sale or resale of the Shares or otherwise, and the Company will, and shall cause each of its affiliates to, comply with all applicable provisions of Regulation M. If the limitations of Rule 102 of Regulation M ("**Rule 102**") do not apply with respect to the Shares or any other reference security pursuant to any exception set forth in Section (d) of Rule 102, then promptly upon notice from the Agent (or, if later, at the time stated in the notice), the Company will, and shall cause each of its affiliates to, comply with Rule 102 as though such exception were not available but the other provisions of Rule 102 (as interpreted by the Commission) did apply. If the exemptive provisions set forth in Rule 101(c)(1) of Regulation M under the 1934 Act are not satisfied with respect to the Company or the Shares, the Company shall promptly notify the Agent.

(v) Notice of Other Sale. Without the written consent of the Agent, the Company will not, directly or indirectly, offer to sell, sell, contract to sell, grant any option to sell or otherwise dispose of any Common Shares or securities convertible into or exchangeable for Common Shares (other than Shares hereunder), warrants or any rights to purchase or acquire Common Shares, during the period beginning on the third Trading Day immediately prior to the date on which any Issuance Notice is delivered to the Agent hereunder and ending on the third Trading Day immediately following the Settlement Date with respect to Shares sold pursuant to such Issuance Notice; provided, however, that such restriction will not be required in connection with (i) the Company's issuance or sale of Common Shares, options to purchase Common Shares or Common Shares issuable upon the exercise of options granted under Company Stock Plans or warrants described as outstanding in the Registration Statement, the Prospectus and the Time of Sale Information, (ii) any options and other awards granted under Company Stock Plans described in the Registration Statement, the Prospectus and the Time of Sale Information, and (iii) shares of Stock or other securities issued in connection with a transaction with an unaffiliated third party

that includes a bona fide commercial relationship (including joint ventures, marketing or distribution arrangements, collaboration agreements or intellectual property license agreements) or any acquisition of assets or acquisition of not less than a majority or controlling portion of the equity of another entity.

(w)If immediately prior to the third anniversary (the "**Renewal Deadline**") of the initial effective date of the Registration Statement, the Company has not sold the Maximum Program Amount and this Agreement has not expired or been terminated, the Company will, prior to the Renewal Deadline, file, if it has not already done so and is eligible to do so, a new automatic shelf registration statement relating to the Shares, in a form satisfactory to the Agent. The Company will, prior to the Renewal Deadline, if it has not already done so, file a new shelf registration statement relating to the Shares, in a form satisfactory to the Agent, and will use its best efforts to cause such registration statement to be declared effective within 60 days after the Renewal Deadline. The Company will take all other action necessary or appropriate to permit the issuance and sale of the Shares to continue as contemplated in the expired registration statement relating to the Shares. References herein to the Registration Statement shall include such new automatic shelf registration statement or such new shelf registration statement, as the case may be.

Section 5. CONDITIONS TO DELIVERY OF ISSUANCE NOTICES AND TO SETTLEMENT

(a) Conditions Precedent to the Right of the Company to Deliver an Issuance Notice and the Obligation of the Agent to Sell Shares During the Selling Period(s). The right of the Company to deliver an Issuance Notice hereunder is subject to the satisfaction, on the date of delivery of such Issuance Notice, and the obligation of the Agent to use its commercially reasonable efforts to place Shares during the applicable Selling Period is subject to the satisfaction, on each Trading Day during the Selling Period, of each of the following conditions:

(i) Accuracy of the Company's Representations and Warranties; Performance by the Company. The Company shall have delivered the certificate required to be delivered pursuant to Section 4(o) on or before the date on which delivery of such certificate is required pursuant to Section 4(o). The Company shall have performed, satisfied and complied with all covenants, agreements and conditions required by this Agreement to be performed, satisfied or complied with by the Company at or prior to such date, including, but not limited to, the covenants contained in Section 4(p), Section 4(q) and Section 4(r).

(ii) No Injunction. No statute, rule, regulation, executive order, decree, ruling or injunction shall have been enacted, entered, promulgated or endorsed by any court or governmental authority of competent jurisdiction or any self-regulatory organization having authority over the matters contemplated hereby that prohibits or directly and materially adversely affects any of the transactions contemplated by this Agreement, and no proceeding shall have been commenced that may have the effect of prohibiting or materially adversely affecting any of the transactions contemplated by this Agreement.

(iii)Material Adverse Changes. Except as disclosed in the Registration Statement, the Prospectus and the Time of Sale Information, (a) in the judgment of the

Agent there shall not have occurred any Material Adverse Change; and (b) there shall not have occurred any downgrading, nor shall any notice have been given of any intended or potential downgrading or of any review for a possible change that does not indicate the direction of the possible change, in the rating accorded any securities of the Company or any of its subsidiaries by any "nationally recognized statistical rating organization" as such term is defined for purposes of Section 3(a)(62) of the Exchange Act.

(iv)No Suspension of Trading in or Delisting of Common Shares; Other Events. The trading of the Common Shares (including without limitation the Shares) shall not have been suspended by the Commission, the Principal Market or FINRA and the Common Shares (including without limitation the Shares) shall have been approved for listing or quotation on and shall not have been delisted from the Principal Market. There shall not have occurred (and be continuing in the case of occurrences under clauses (i), (ii) and (iii) below) any of the following: (i) trading generally shall have been suspended or materially limited on or by any of the New York Stock Exchange or the Principal Market; (ii) trading of any securities issued or guaranteed by the Company shall have been suspended on any exchange or in any over-the-counter market; (iii) a general moratorium on commercial banking activities shall have been declared by federal or New York State authorities; or (iv) there shall have occurred any outbreak or escalation of hostilities or any change in financial markets or any calamity or crisis, either within or outside the United States, that, in the judgment of the Agent is material and adverse and makes it impracticable or inadvisable to market the Shares in the manner and on the terms contemplated by this Agreement and described in the Prospectus.

(b) Documents Required to be Delivered on each Issuance Notice Date. The Agent's obligation to use its commercially reasonable efforts to place Shares hereunder shall additionally be conditioned upon the delivery to the Agent on or before the Issuance Notice Date of:

(b) a certificate in form and substance reasonably satisfactory to the Agent, executed by the Chief Executive Officer, President or Chief Financial Officer of the Company, to the effect that all conditions to the delivery of such Issuance Notice shall have been satisfied as at the date of such certificate as required to be delivered pursuant to Section 4(s) (which certificate shall not be required if the foregoing representations shall be set forth in the Issuance Notice);

(c) a negative assurances letter and the written legal opinion of counsel to the Company required to be delivered pursuant to Section 4(p);

(d) the written legal opinions of intellectual property counsels to the Company required to be delivered pursuant to Section 4(p);

(iv) a negative assurances letter and the written legal opinion of counsel to the Agent, such opinion or opinions to be delivered on or before the date on which the delivery of the opinion by counsel to the Company is required pursuant to Section 4(p), with respect to such matters as Agent may reasonably require, and the Company shall have furnished to such counsel such documents as they request for enabling them to pass upon such matters;

(v) the comfort letter required to be delivered pursuant to Section 4(q); and

(vi) the certificate of the Secretary of the Company to be delivered pursuant to Section 4(r).

(c) No Misstatement or Material Omission. Agent shall not have advised the Company that the Registration Statement, Prospectus or the Time of Sale Information, or any amendment or supplement thereto, contains an untrue statement of fact that in the Agent's reasonable opinion is material, or omits to state a fact that in the Agent's reasonable opinion is material and is required to be stated therein or is necessary to make the statements therein not misleading.

Section 6. INDEMNIFICATION AND CONTRIBUTION

(a) Indemnification of the Agent. The Company agrees to indemnify and hold harmless the Agent, its officers and employees, and each person, if any, who controls the Agent within the meaning of the Securities Act or the Exchange Act against any loss, claim, damage, liability or expense, as incurred, to which the Agent or such officer, employee or controlling person may become subject, under the Securities Act, the Exchange Act, other federal or state statutory law or regulation, or the laws or regulations of foreign jurisdictions where Shares have been offered or sold or at common law or otherwise (including in settlement of any litigation), insofar as such loss, claim, damage, liability or expense (or actions in respect thereof as contemplated below) arises out of or is based upon (i) any untrue statement or alleged untrue statement of a material fact contained in the Registration Statement, or any amendment thereto, including any information deemed to be a part thereof pursuant to Rule 430B under the Securities Act, or the omission or alleged omission therefrom of a material fact required to be stated therein or necessary to make the statements therein not misleading; or (ii) any untrue statement or alleged untrue statement of a material fact contained in any Free Writing Prospectus that the Company has used, referred to or filed, or is required to file, pursuant to Rule 433(d) of the Securities Act or the Prospectus (or any amendment or supplement thereto), or the omission or alleged omission therefrom of a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading; and to reimburse the Agent and each such officer, employee and controlling person for any and all expenses (including the fees and disbursements of counsel chosen by the Agent) as such expenses are reasonably incurred by the Agent or such officer, employee or controlling person in connection with investigating, defending, settling, compromising or paying any such loss, claim, damage, liability, expense or action; provided, however, that the foregoing indemnity agreement shall not apply to any loss, claim, damage, liability or expense to the extent, but only to the extent, arising out of or based upon any untrue statement or alleged untrue statement or omission or alleged omission made in reliance upon and in conformity with written information furnished to the Company by the Agent expressly for use in the Registration Statement, any such Free Writing Prospectus or the Prospectus (or any amendment or supplement thereto), it being understood and agreed that the only such information furnished by the Agent to the Company consists of: (i) the Agent's name. The indemnity agreement set forth in this Section 6(a) shall be in addition to any liabilities that the Company may otherwise have.

(b) Notifications and Other Indemnification Procedures. Promptly after receipt by an indemnified party under this Section 6 of notice of the commencement of any action, such

indemnified party will, if a claim in respect thereof is to be made against an indemnifying party under this Section 6, notify the indemnifying party in writing of the commencement thereof, but the omission so to notify the indemnifying party will not relieve it from any liability which it may have to any indemnified party for contribution or otherwise than under the indemnity agreement contained in this Section 6 or to the extent it is not prejudiced as a proximate result of such failure. In case any such action is brought against any indemnified party and such indemnified party seeks or intends to seek indemnity from an indemnifying party, the indemnifying party will be entitled to participate in, and, to the extent that it shall elect, jointly with all other indemnifying parties similarly notified, by written notice delivered to the indemnified party promptly after receiving the aforesaid notice from such indemnified party, to assume the defense thereof with counsel reasonably satisfactory to such indemnified party; provided, however, if the defendants in any such action include both the indemnified party and the indemnifying party and the indemnified party shall have reasonably concluded that a conflict may arise between the positions of the indemnifying party and the indemnified party in conducting the defense of any such action or that there may be legal defenses available to it and/or other indemnified parties which are different from or additional to those available to the indemnifying party, the indemnified party or parties shall have the right to select separate counsel to assume such legal defenses and to otherwise participate in the defense of such action on behalf of such indemnified party or parties. Upon receipt of notice from the indemnifying party to such indemnified party of such indemnifying party's election so to assume the defense of such action and approval by the indemnified party of counsel, the indemnifying party will not be liable to such indemnified party under this Section 6 for any legal or other expenses subsequently incurred by such indemnified party in connection with the defense thereof unless (i) the indemnified party shall have employed separate counsel in accordance with the proviso to the preceding sentence (it being understood, however, that the indemnifying party shall not be liable for the fees and expenses of more than one separate counsel (together with local counsel), representing the indemnified parties who are parties to such action), which counsel (together with any local counsel) for the indemnified parties shall be selected by the Agent (in the case of counsel for the indemnified parties referred to in Section 6(a) above), (ii) the indemnifying party shall not have employed counsel satisfactory to the indemnified party to represent the indemnified party within a reasonable time after notice of commencement of the action or (iii) the indemnifying party has authorized in writing the employment of counsel for the indemnified party at the expense of the indemnifying party, in each of which cases the fees and expenses of counsel shall be at the expense of the indemnifying party and shall be paid as they are incurred.

(c) Settlements. The indemnifying party under this Section 6 shall not be liable for any settlement of any proceeding effected without its written consent, but if settled with such consent or if there be a final judgment for the plaintiff, the indemnifying party agrees to indemnify the indemnified party against any loss, claim, damage, liability or expense by reason of such settlement or judgment. Notwithstanding the foregoing sentence, if at any time an indemnified party shall have requested an indemnifying party to reimburse the indemnified party for fees and expenses of counsel as contemplated by Section 6(b) hereof, the indemnifying party agrees that it shall be liable for any settlement of any proceeding effected without its written consent if (i) such settlement is entered into more than 45 days after receipt by such indemnifying party of the aforesaid request and (ii) such indemnifying party shall not have reimbursed the indemnified party in accordance with such request prior to the date of such settlement. No indemnifying party shall, without the prior written consent of the indemnified party, effect any settlement, compromise or consent to the

entry of judgment in any pending or threatened action, suit or proceeding in respect of which any indemnified party is or could have been a party and indemnity was or could have been sought hereunder by such indemnified party, unless such settlement, compromise or consent includes an unconditional release of such indemnified party from all liability on claims that are the subject matter of such action, suit or proceeding.

(d) Contribution. If the indemnification provided for in this Section 6 is for any reason held to be unavailable to or otherwise insufficient to hold harmless an indemnified party in respect of any losses, claims, damages, liabilities or expenses referred to therein, then each indemnifying party shall contribute to the aggregate amount paid or payable by such indemnified party, as incurred, as a result of any losses, claims, damages, liabilities or expenses referred to therein (i) in such proportion as is appropriate to reflect the relative benefits received by the Company, on the one hand, and the Agent, on the other hand, from the offering of the Shares pursuant to this Agreement or (ii) if the allocation provided by clause (i) above is not permitted by applicable law, in such proportion as is appropriate to reflect not only the relative benefits referred to in clause (i) above but also the relative fault of the Company, on the one hand, and the Agent, on the other hand, in connection with the statements or omissions which resulted in such losses, claims, damages, liabilities or expenses, as well as any other relevant equitable considerations. The relative benefits received by the Company, on the one hand, and the Agent, on the other hand, in connection with the offering of the Shares pursuant to this Agreement shall be deemed to be in the same respective proportions as the total gross proceeds from the offering of the Shares (before deducting expenses) received by the Company bear to the total commissions received by the Agent. The relative fault of the Company, on the one hand, and the Agent, on the other hand, shall be determined by reference to, among other things, whether any such untrue or alleged untrue statement of a material fact or omission or alleged omission to state a material fact relates to information supplied by the Company, on the one hand, or the Agent, on the other hand, and the parties' relative intent, knowledge, access to information and opportunity to correct or prevent such statement or omission.

The amount paid or payable by a party as a result of the losses, claims, damages, liabilities and expenses referred to above shall be deemed to include, subject to the limitations set forth in Section 6(b), any legal or other fees or expenses reasonably incurred by such party in connection with investigating or defending any action or claim. The provisions set forth in Section 6(b) with respect to notice of commencement of any action shall apply if a claim for contribution is to be made under this Section 6(d); *provided, however,* that no additional notice shall be required with respect to any action for which notice has been given under Section 6(b) for purposes of indemnification.

The Company and the Agent agree that it would not be just and equitable if contribution pursuant to this Section 6(d) were determined by pro rata allocation or by any other method of allocation which does not take account of the equitable considerations referred to in this Section 6(d).

Notwithstanding the provisions of this Section 6(d), the Agent shall not be required to contribute any amount in excess of the agent fees received by the Agent in connection with the offering contemplated hereby. No person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) shall be entitled to contribution from any person

who was not guilty of such fraudulent misrepresentation. For purposes of this Section 6(d), each officer and employee of the Agent and each person, if any, who controls the Agent within the meaning of the Securities Act or the Exchange Act shall have the same rights to contribution as the Agent, and each director of the Company, each officer of the Company who signed the Registration Statement, and each person, if any, who controls the Company with the meaning of the Securities Act and the Exchange Act shall have the same rights to contribution as the Company.

Section 7. TERMINATION & SURVIVAL

(a) Term. Subject to the provisions of this Section 7, the term of this Agreement shall continue from the date of this Agreement until the end of the Agency Period, unless earlier terminated by the parties to this Agreement pursuant to this Section 7.

(b) Termination; Survival Following Termination. (i) Either party may terminate this Agreement prior to the end of the Agency Period, by giving written notice as required by this Agreement, upon five Trading Days' notice to the other party; provided that, (A) if the Company terminates this Agreement after the Agent confirms to the Company any sale of Shares, the Company shall remain obligated to comply with Section 3(b)(v) with respect to such Shares and (B) Section 2, Section 6, Section 7 and Section 8 shall survive termination of this Agreement. If termination shall occur prior to the Settlement Date for any sale of Shares, such sale shall nevertheless settle in accordance with the terms of this Agreement. Upon termination of this Agreement, the Company shall not have any liability to the Agent for any discount, commission or other compensation with respect to any Shares not otherwise sold by the Agent under this Agreement.

(ii) In addition to the survival provision of Section 7(b)(i), the respective indemnities, agreements, representations, warranties and other statements of the Company, of its officers and of the Agent set forth in or made pursuant to this Agreement will remain in full force and effect, regardless of any investigation made by or on behalf of the Agent or the Company or any of its or their partners, officers or directors or any controlling person, as the case may be, and, anything herein to the contrary notwithstanding, will survive delivery of and payment for the Shares sold hereunder and any termination of this Agreement.

Section 8. MISCELLANEOUS

(a) Press Releases and Disclosure. The Company may issue a press release describing the material terms of the transactions contemplated hereby as soon as practicable following the date of this Agreement, and may file with the Commission a Current Report on Form 8-K, with this Agreement attached as an exhibit thereto, describing the material terms of the transactions contemplated hereby, and the Company shall consult with the Agent prior to making such disclosures, and the parties hereto shall use all commercially reasonable efforts, acting in good faith, to agree upon a text for such disclosures that is reasonably satisfactory to all parties hereto. Notwithstanding anything to the contrary herein, the Company shall not issue any press release or make any other public disclosure or public statement (other than disclosure, including related to periodic sales pursuant to this Agreement, the Company believes is required to be included in any reports filed with the Commission pursuant to the Securities Act or Exchange Act) related to this

Agreement, or any of the transactions contemplated hereby or naming the Agent without the Agent's prior written approval.

(b) No Advisory or Fiduciary Relationship. The Company acknowledges and agrees that (i) the transactions contemplated by this Agreement, including the determination of any fees, are arm's-length commercial transactions between the Company and the Agent, (ii) when acting as a principal under this Agreement, the Agent is and has been acting solely as a principal is not the agent or fiduciary of the Company, or its stockholders, creditors, employees or any other party, (iii) the Agent has not assumed nor will assume an advisory or fiduciary responsibility in favor of the Company with respect to the transactions contemplated hereby or the process leading thereto (irrespective of whether the Agent has advised or is currently advising the Company on other matters) and the Agent does not have any obligation to the Company with respect to the transactions contemplated hereby except the obligations expressly set forth in this Agreement, (iv) the Agent and its respective affiliates may be engaged in a broad range of transactions that involve interests that differ from those of the Company, and (v) the Agent has not provided any legal, accounting, regulatory or tax advice with respect to the transactions contemplated hereby and the Company has consulted its own legal, accounting, regulatory and tax advisors to the extent it deemed appropriate.

(c) Research Analyst Independence. The Company acknowledges that the Agent's research analysts and research departments are required to and should be independent from their respective investment banking divisions and are subject to certain regulations and internal policies, and as such the Agent's research analysts may hold views and make statements or investment recommendations and/or publish research reports with respect to the Company or the offering that differ from the views of their respective investment banking divisions. The Company understands that the Agent is a full service securities firm and as such from time to time, subject to applicable securities laws, may effect transactions for its own account or the account of its customers and hold long or short positions in debt or equity securities of the companies that may be the subject of the transactions contemplated by this Agreement.

(d) Notices. All communications hereunder shall be in writing and shall be mailed, hand delivered, sent via electronic mail (if applicable) or telecopied and confirmed to the parties hereto as follows:

If to the Agent:

Jefferies LLC
520 Madison Avenue
New York, NY 10022
Attention: General Counsel

with a copy to:

Davis Polk & Wardwell LLP
450 Lexington Avenue
New York, NY 10017

Facsimile: (212) 701-5135
Attention: Deanna Kirkpatrick, Esq.

If to the Company:

Solid Biosciences Inc.
500 Rutherford Avenue, Third Floor
Charlestown, Massachusetts 02129
Attention: Chief Executive Officer

with a copy to:

Wilmer Cutler Pickering Hale and Dorr LLP
60 State Street
Boston, MA 02109
Attention: Caroline Dotolo

Any party hereto may change the address for receipt of communications by giving written notice to the others in accordance with this Section 8(d).

(e) Successors. This Agreement will inure to the benefit of and be binding upon the parties hereto, and to the benefit of the employees, officers and directors and controlling persons referred to in Section 6, and in each case their respective successors, and no other person will have any right or obligation hereunder. The term "**successors**" shall not include any purchaser of the Shares as such from the Agent merely by reason of such purchase.

(f) Partial Unenforceability. The invalidity or unenforceability of any Article, Section, paragraph or provision of this Agreement shall not affect the validity or enforceability of any other Article, Section, paragraph or provision hereof. If any Article, Section, paragraph or provision of this Agreement is for any reason determined to be invalid or unenforceable, there shall be deemed to be made such minor changes (and only such minor changes) as are necessary to make it valid and enforceable.

(g) Governing Law Provisions. This Agreement, and any claim, controversy or dispute of any kind or nature whatsoever arising out of or in any way relating to this Agreement, shall be governed by and construed in accordance with the internal laws of the State of New York applicable to agreements made and to be performed in such state. Any legal suit, action or proceeding arising out of or based upon this Agreement or the transactions contemplated hereby may be instituted in the federal courts of the United States of America located in the Borough of Manhattan in the City of New York or the courts of the State of New York in each case located in the Borough of Manhattan in the City of New York (collectively, the "**Specified Courts**"), and each party irrevocably submits to the exclusive jurisdiction (except for proceedings instituted in regard to the enforcement of a judgment of any such court, as to which such jurisdiction is non-exclusive) of such courts in any such suit, action or proceeding. Service of any process, summons, notice or document by mail to such party's address set forth above shall be effective service of process for any suit, action or other proceeding brought in any such court. The parties irrevocably and unconditionally waive any objection to the laying of venue of any suit, action or other

proceeding in the Specified Courts and irrevocably and unconditionally waive and agree not to plead or claim in any such court that any such suit, action or other proceeding brought in any such court has been brought in an inconvenient forum.

(h) General Provisions. This Agreement constitutes the entire agreement of the parties to this Agreement and supersedes all prior written or oral and all contemporaneous oral agreements, understandings and negotiations with respect to the subject matter hereof. This Agreement may be executed in two or more counterparts, each one of which shall be an original, with the same effect as if the signatures thereto and hereto were upon the same instrument, and may be delivered by facsimile transmission or by electronic delivery of a portable document format (PDF) file (including any electronic signature covered by the U.S. federal ESIGN Act of 2000, Uniform Electronic Transactions Act, the Electronic Signatures and Records Act or other applicable law, e.g., www.docusign.com). This Agreement may not be amended or modified unless in writing by all of the parties hereto, and no condition herein (express or implied) may be waived unless waived in writing by each party whom the condition is meant to benefit. The Article and Section headings herein are for the convenience of the parties only and shall not affect the construction or interpretation of this Agreement.

[Signature Page Immediately Follows]

If the foregoing is in accordance with your understanding of our agreement, kindly sign and return to the Company the enclosed copies hereof, whereupon this instrument, along with all counterparts hereof, shall become a binding agreement in accordance with its terms.

Very truly yours,

SOLID BIOSCIENCES INC.

By: /s/ Kevin Tan
Name: Kevin Tan
Title: Chief Financial Officer

The foregoing Agreement is hereby confirmed and accepted by the Agent in New York, New York as of the date first above written.

JEFFERIES LLC

By: /s/ Michael Magarro
Name: Michael Magarro
Title: Managing Director

EXHIBIT A

ISSUANCE NOTICE

[Date]

Jefferies LLC
520 Madison Avenue
New York, New York 10022

Attn: [_____]

Reference is made to the Amended and Restated Sales Agreement, between Solid Biosciences Inc. (the "**Company**") and Jefferies LLC (the "**Agent**") dated as of March 13, 2024. The Company confirms that all conditions to the delivery of this Issuance Notice are satisfied as of the date hereof.

Date of Delivery of Issuance Notice (determined pursuant to Section 3(b)(i)): _____

Issuance Amount (equal to the total Sales Price for such Shares):

$_____

Number of Days in Selling Period: _____

First Date of Selling Period: _____

Last Date of Selling Period: _____

Settlement Date(s) if other than standard [T+2][T+1] settlement:

_____

Floor Price Limitation (in no event less than $1.00 without the prior written consent of the Agent, which consent may be withheld in the Agent's sole discretion): $ ____ per share

Comments: _____

SOLID BIOSCIENCES INC.

By: _____
Name:
Title:

Exhibit 10.49

Solid Biosciences Inc.

2024 INDUCEMENT STOCK INCENTIVE PLAN

1. Purpose

The purpose of this 2024 Inducement Stock Incentive Plan (the "***Plan***") of Solid Biosciences Inc., a Delaware corporation (the "***Company***"), is to advance the interests of the Company's stockholders by enhancing the Company's ability to attract, retain and motivate persons who are expected to make important contributions to the Company with an inducement material for such persons to enter into employment with the Company and by providing such persons with equity ownership opportunities and performance-based incentives that are intended to better align the interests of such persons with those of the Company's stockholders. Except where the context otherwise requires, the term "***Company***" shall include any of the Company's present or future parent or subsidiary corporations as defined in Sections 424(e) or (f) of the Internal Revenue Code of 1986, as amended, and any regulations thereunder (the "***Code***") and any other business venture (including, without limitation, joint venture or limited liability company) in which the Company has a controlling interest, as determined by the Board of Directors of the Company (the "***Board***").

2. Eligibility

Awards under the Plan may only be granted to persons who (a) were not previously an employee or director of the Company or (b) are commencing employment with the Company following a bona fide period of non-employment, in either case as an inducement material to the individual's entering into employment with the Company and in accordance with the requirements of Nasdaq Stock Market Rule 5635(c)(4). For the avoidance of doubt, neither consultants nor advisors shall be eligible to participate in the Plan. Each person who is granted an Award under the Plan is deemed a "***Participant***." "***Award***" means Options (as defined in Section 5), SARs (as defined in Section 6), Restricted Stock (as defined in Section 7), RSUs (as defined in Section 7), and Other Stock-Based Awards (as defined in Section 8). Except as otherwise provided by the Plan, each Award may be made alone or in addition or in relation to any other Award. The terms of each Award need not be identical, and the Board need not treat Participants uniformly.

3. Administration and Delegation

(a) Administration by Board of Directors. The Plan will be administered by the Board. The Board shall have authority to grant Awards and to adopt, amend and repeal such administrative rules, guidelines and practices relating to the Plan as it shall deem advisable. The Board may construe and interpret the terms of the Plan and any Award agreements entered into under the Plan. The Board may correct any defect, supply any omission or reconcile any inconsistency in the Plan or any Award in the manner and to the extent it shall deem expedient and it shall be the sole and final judge of such expediency. All decisions by the Board shall be made in the Board's sole discretion and shall be final and binding on all persons having or claiming any interest in the Plan or in any Award. Notwithstanding the foregoing or anything in the Plan to the contrary, the grant of any Award under the Plan must be approved by the Company's independent compensation committee or a majority of the Company's independent directors (as defined in Nasdaq Stock Market Rule 5605(a)(2)) in order to comply with the exemption from the stockholder approval requirement for "inducement grants" provided under Nasdaq Stock Market Rule 5635(c)(4).

(b) Appointment of Committees. To the extent permitted by applicable law and the Nasdaq Stock Market rules, the Board may delegate any or all of its powers under the Plan to one or more committees or subcommittees of the Board (a "***Committee***"). All references in the Plan to the "***Board***" shall mean the Board or a Committee of the Board or the Delegated Persons referred to in Section 3(c) to the extent that the Board's powers or authority under the Plan have been delegated to such Committee or such Delegated Persons.

(c) Delegation to Delegated Persons. Subject to any requirements of applicable law (including as applicable Sections 152 and 157(c) of the General Corporation Law of the State of Delaware) and the Nasdaq Stock Market rules, the Board may, by resolution, delegate to one or more persons (including officers of the Company) or bodies (such persons or bodies, the "***Delegated Persons***") the power to grant Awards (subject to any limitations under the Plan) to eligible service providers of the Company and to exercise such other powers under the Plan as the

Board may determine, provided that the Board shall fix: (i) the maximum number of Awards, and the maximum number of shares issuable upon exercise thereof, that may be issued by such Delegated Persons, (ii) the time period during which such Awards, and during which the shares issuable upon exercise thereof, may be issued, and (iii) the minimum amount of consideration (if any) for which such Awards may be issued, and a minimum amount of consideration for the shares issuable upon exercise thereof; and provided further, that no Delegated Person shall be authorized to grant Awards to any "executive officer" of the Company (as defined by Rule 3b-7 under the Securities Exchange Act of 1934, as amended (the "***Exchange Act***")) or to any "officer" of the Company (as defined by Rule 16a-1(f) under the Exchange Act); and provided further, that to the extent the resolution of the Board is made dependent on facts ascertainable outside the resolution, such facts may not be dependent on a determination or action of the Delegated Person to whom authority has been delegated hereunder.

4. Stock Available for Awards

(a) Authorized Number of Shares. Subject to adjustment under Section 9, Awards may be made under the Plan for up to 1,000,000 shares of common stock, $0.001 par value per share, of the Company (the "***Common Stock***"). Shares issued under the Plan may consist in whole or in part of authorized but unissued shares or treasury shares.

(b) Share Counting. For purposes of counting the number of shares available for the grant of Awards under the Plan under this Section 4:

(1) all shares of Common Stock covered by SARs shall be counted against the number of shares available for the grant of Awards under the Plan; *provided, however*, that (i) SARs that may be settled only in cash shall not be so counted and (ii) if the Company grants an SAR in tandem with an Option for the same number of shares of Common Stock and provides that only one such Award may be exercised (a "***Tandem SAR***"), only the shares covered by the Option, and not the shares covered by the Tandem SAR, shall be so counted, and the expiration of one in connection with the other's exercise will not restore shares to the Plan;

(2) to the extent an RSU award may be settled only in cash, no shares shall be counted against the shares available for the grant of Awards under the Plan;

(3) if any Award (i) expires or is terminated, surrendered or canceled without having been fully exercised or is forfeited in whole or in part (including as the result of shares of Common Stock subject to such Award being repurchased by the Company at the original issuance price pursuant to a contractual repurchase right) or (ii) results in any Common Stock not being issued (including as a result of an SAR that was settleable either in cash or in stock actually being settled in cash), the unused Common Stock covered by such Award shall again be available for the grant of Awards; *provided, however*, that in the case of the exercise of an SAR, the number of shares counted against the shares available under the Plan shall be the full number of shares subject to the SAR multiplied by the percentage of the SAR actually exercised, regardless of the number of shares actually used to settle such SAR upon exercise and the shares covered by a Tandem SAR shall not again become available for grant upon the expiration or termination of such Tandem SAR; and

(4) shares of Common Stock delivered (by actual delivery, attestation, or net exercise) to the Company by a Participant to (i) purchase shares of Common Stock upon the exercise of an Award or (ii) satisfy tax withholding obligations with respect to Awards (including shares retained from the Award creating the tax obligation) shall be added back to the number of shares available for the future grant of Awards.

5. Stock Options

(a) General. The Board may grant options to purchase Common Stock (each, an "***Option***") and determine the number of shares of Common Stock to be covered by each Option, the exercise price of each Option and the conditions and limitations applicable to the exercise of each Option, including conditions relating to applicable federal or state securities laws, as it considers necessary or advisable. All Options under the Plan shall be Nonstatutory Stock Options. A "***Nonstatutory Stock Option***" is an Option which is not intended to be an "incentive stock option" within the meaning of Section 422 of the Code.

(b) Exercise Price. The Board shall establish the exercise price of each Option or the formula by which such exercise price will be determined. The exercise price shall be specified in the applicable option agreement. The exercise price shall be not less than 100% of the Grant Date Fair Market Value (as defined below) of the Common Stock on the date the Option is granted; *provided* that if the Board approves the grant of an Option with an exercise price to be determined on a future date, the exercise price shall be not less than 100% of the Grant Date Fair Market Value on such future date. "***Grant Date Fair Market Value***" of a share of Common Stock for purposes of the Plan will be determined as follows:

(1) if the Common Stock trades on a national securities exchange, the closing sale price (for the primary trading session) on the date of grant; or

(2) if the Common Stock does not trade on any such exchange, the average of the closing bid and asked prices on the date of grant as reported by an over-the-counter marketplace designated by the Board; or

(3) if the Common Stock is not publicly traded, the Board will determine the Grant Date Fair Market Value for purposes of the Plan using any measure of value it determines to be appropriate (including, as it considers appropriate, relying on appraisals) in a manner consistent with the valuation principles under Code Section 409A, except as the Board may expressly determine otherwise.

For any date that is not a trading day, the Grant Date Fair Market Value of a share of Common Stock for such date will be determined by using the closing sale price or average of the bid and asked prices, as appropriate, for the immediately preceding trading day and with the timing in the formulas above adjusted accordingly. The Board can substitute a particular time of day or other measure of "closing sale price" or "bid and asked prices" if appropriate because of exchange or market procedures or can, in its sole discretion, use weighted averages either on a daily basis or such longer period as complies with Code Section 409A.

The Board has sole discretion to determine the Grant Date Fair Market Value for purposes of the Plan, and all Awards are conditioned on the Participants' agreement that the Board's determination is conclusive and binding even though others might make a different determination.

(c) Duration of Options. Each Option shall be exercisable at such times and subject to such terms and conditions as the Board may specify in the applicable Option agreement; *provided, however*, that no Option will be granted with a term in excess of 10 years.

(d) Exercise of Options. Options may be exercised by delivery to the Company of a notice of exercise in a form (which may be electronic, and which may be provided to a third party equity plan administrator) approved by the Company, together with payment in full (in the manner specified in Section 5(e)) of the exercise price for the number of shares for which the Option is exercised. Shares of Common Stock subject to the Option will be delivered by the Company as soon as practicable following exercise.

(e) Payment Upon Exercise. Common Stock purchased upon the exercise of an Option granted under the Plan shall be paid for as follows:

(1) in cash or by check, payable to the order of the Company;

(2) except as may otherwise be provided in the applicable Option agreement or approved by the Board, by (i) delivery of an irrevocable and unconditional undertaking by a creditworthy broker to deliver promptly to the Company sufficient funds to pay the exercise price and any required tax withholding or (ii) delivery by the Participant to the Company of a copy of irrevocable and unconditional instructions to a creditworthy broker to deliver promptly to the Company cash or a check sufficient to pay the exercise price and any required tax withholding;

(3) to the extent provided for in the applicable Option agreement or approved by the Board, by delivery (either by actual delivery or attestation) of shares of Common Stock owned by the Participant valued at their fair market value (valued in the manner determined by (or in a manner approved by) the Board), provided (i) such method of payment is then permitted under applicable law, (ii) such Common Stock, if acquired directly from

the Company, was owned by the Participant for such minimum period of time, if any, as may be established by the Board and (iii) such Common Stock is not subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements;

(4) to the extent provided for in the applicable Nonstatutory Stock Option agreement or approved by the Board, by delivery of a notice of "net exercise" to the Company, as a result of which the Participant would receive (i) the number of shares underlying the portion of the Option being exercised, less (ii) such number of shares as is equal to (A) the aggregate exercise price for the portion of the Option being exercised divided by (B) the fair market value of the Common Stock (valued in the manner determined by (or in a manner approved by) the Board) on the date of exercise;

(5) to the extent permitted by applicable law and provided for in the applicable Option agreement or approved by the Board by payment of such other lawful consideration as the Board may determine; provided, however, that in no event may a promissory note of the Participant be used to pay the Option exercise price; or

(6) by any combination of the above permitted forms of payment, to the extent approved by the Board.

(f) Limitation on Repricing. Unless such action is approved by the Company's stockholders, the Company may not (except as provided for under Section 9): (1) amend any outstanding Option granted under the Plan to provide an exercise price per share that is lower than the then-current exercise price per share of such outstanding Option, (2) cancel any outstanding option (whether or not granted under the Plan) and grant in substitution therefor new Awards under the Plan covering the same or a different number of shares of Common Stock and having an exercise or measurement price per share lower than the then-current exercise price per share of the cancelled option, (3) cancel in exchange for a cash payment any outstanding Option with an exercise price per share above the then-current fair market value of the Common Stock (valued in the manner determined by (or in the manner approved by) the Board) or (4) take any other action under the Plan that constitutes a "repricing" within the meaning of the rules of the Nasdaq Stock Market or any other exchange or marketplace on which the Company stock is listed or traded (the "***Exchange***").

(g) No Dividend Equivalents. No Option shall provide for the payment or accrual of dividend equivalents.

6. Stock Appreciation Rights

(a) General. The Board may grant Awards consisting of stock appreciation rights ("***SARs***") entitling the holder, upon exercise, to receive an amount of Common Stock or cash or a combination thereof (such form to be determined by the Board) determined by reference to appreciation, from and after the date of grant, in the fair market value of a share of Common Stock (valued in the manner determined by (or in the manner approved by) the Board) over the measurement price established pursuant to Section 6(b). The date as of which such appreciation is determined shall be the exercise date.

(b) Measurement Price. The Board shall establish the measurement price of each SAR and specify it in the applicable SAR agreement. The measurement price shall not be less than 100% of the Grant Date Fair Market Value of the Common Stock on the date the SAR is granted; *provided* that if the Board approves the grant of an SAR effective as of a future date, the measurement price shall be not less than 100% of the Grant Date Fair Market Value on such future date.

(c) Duration of SARs. Each SAR shall be exercisable at such times and subject to such terms and conditions as the Board may specify in the applicable SAR agreement; *provided, however*, that no SAR will be granted with a term in excess of 10 years.

(d) Exercise of SARs. SARs may be exercised by delivery to the Company of a notice of exercise in a form (which may be electronic) approved by the Company, together with any other documents required by the Board.

(e) Limitation on Repricing. Unless such action is approved by the Company's stockholders, the Company may not (except as provided for under Section 9): (1) amend any outstanding SAR granted under the Plan to provide a measurement price per share that is lower than the then-current measurement price per share of such outstanding SAR, (2) cancel any outstanding SAR (whether or not granted under the Plan) and grant in substitution therefor new Awards under the Plan covering the same or a different number of shares of Common Stock and having an exercise or measurement price per share lower than the then-current measurement price per share of the cancelled SAR, (3) cancel in exchange for a cash payment any outstanding SAR with a measurement price per share above the then-current fair market value of the Common Stock (valued in the manner determined by (or in a manner approved by) the Board) or (4) take any other action under the Plan that constitutes a "repricing" within the meaning of the rules of the Exchange.

(f) No Dividend Equivalents. No SAR shall provide for the payment or accrual of dividend equivalents.

7. Restricted Stock; RSUs

(a) General. The Board may grant Awards entitling recipients to acquire shares of Common Stock ("***Restricted Stock***"), subject to the right of the Company to repurchase all or part of such shares at their issue price or other stated or formula price (or to require forfeiture of such shares if issued at no cost) from the recipient in the event that conditions specified by the Board in the applicable Award are not satisfied prior to the end of the applicable restriction period or periods established by the Board for such Award. The Board may also grant Awards entitling the recipient to receive shares of Common Stock or cash to be delivered as soon as practicable after the time such Award vests or is settled ("***RSUs***").

(b) Terms and Conditions for Restricted Stock and RSUs. The Board shall determine the terms and conditions of Restricted Stock and RSUs, including the conditions for vesting and repurchase (or forfeiture) and the issue price, if any.

(c) Additional Provisions Relating to Restricted Stock.

(1) Dividends. Any dividends (whether paid in cash, stock or property) declared and paid by the Company with respect to shares of Restricted Stock ("***Unvested Dividends***") shall be paid to the Participant only if and when such shares become free from the restrictions on transferability and forfeitability that apply to such shares. Each payment of Unvested Dividends will be made no later than the end of the calendar year in which the dividends are paid to stockholders of that class of stock or, if later, the 15th day of the third month following the lapsing of the restrictions on transferability and the forfeitability provisions applicable to the underlying shares of Restricted Stock.

(2) Stock Certificates. The Company may require that any stock certificates issued in respect of shares of Restricted Stock, as well as dividends or distributions paid on such Restricted Stock, shall be deposited in escrow by the Participant, together with a stock power endorsed in blank, with the Company (or its designee). At the expiration of the applicable restriction periods, the Company (or such designee) shall deliver the certificates no longer subject to such restrictions to the Participant or if the Participant has died, to his or her Designated Beneficiary. "***Designated Beneficiary***" means (i) the beneficiary designated, in a manner determined by the Board, by a Participant to receive amounts due or exercise rights of the Participant in the event of the Participant's death or (ii) in the absence of an effective designation by a Participant, the Participant's estate.

(d) Additional Provisions Relating to RSUs.

(1) Settlement. As soon as practicable after the vesting of and/or lapsing of any other restrictions (i.e., settlement) with respect to each RSU, the Participant shall be entitled to receive from the Company such number of shares of Common Stock or (if so provided in the applicable Award agreement) an amount of cash equal to the fair market value (valued in the manner determined by (or in a manner approved by) the Board) of such number of shares of Common Stock as are set forth in the applicable RSU agreement. The Board may provide that

settlement of RSUs shall be deferred, on a mandatory basis or at the election of the Participant in a manner that complies with Section 409A of the Code.

(2) Voting Rights. A Participant shall have no voting rights with respect to any RSUs.

(3) Dividend Equivalents. The Award agreement for RSUs may provide Participants with the right to receive an amount equal to any dividends or other distributions declared and paid on an equal number of outstanding shares of Common Stock ("***Dividend Equivalents***"). Dividend Equivalents may be settled in cash and/or shares of Common Stock, as provided in the Award agreement, and shall be subject to the same restrictions on transfer and forfeitability as the RSUs with respect to which paid.

8. Other Stock-Based Awards

(a) General. The Board may grant other Awards of shares of Common Stock, and other Awards that are valued in whole or in part by reference to, or are otherwise based on, shares of Common Stock or other property ("***Other Stock-Based Awards***"). Such Other Stock-Based Awards shall also be available as a form of payment in the settlement of other Awards granted under the Plan or as payment in lieu of compensation to which a Participant is otherwise entitled. Other Stock-Based Awards may be paid in shares of Common Stock or cash, as the Board shall determine.

(b) Terms and Conditions. Subject to the provisions of the Plan, the Board shall determine the terms and conditions of each Other Stock-Based Award, including any purchase price applicable thereto.

(c) Dividend Equivalents. The Award agreement for an Other Stock-Based Award may provide Participants with the right to receive Dividend Equivalents. Dividend Equivalents will be credited to an account for the Participant, may be settled in cash and/or shares of Common Stock as set forth in the Award agreement and shall be subject to the same restrictions on transfer and forfeitability as the Other Stock-Based Award with respect to which paid. No interest will be paid on Dividend Equivalents.

9. Adjustments for Changes in Common Stock and Certain Other Events

(a) Changes in Capitalization. In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of Common Stock other than an ordinary cash dividend, (i) the number and class of securities available under the Plan, (ii) the share counting rules set forth in Section 4, (iii) the number and class of securities and exercise price per share of each outstanding Option, (iv) the share and per-share provisions and the measurement price of each outstanding SAR, (v) the number of shares subject to and the repurchase price per share subject to each outstanding award of Restricted Stock and (vi) the share and per-share-related provisions and the purchase price, if any, of each outstanding RSU award and each outstanding Other Stock-Based Award, shall be equitably adjusted by the Company (or substituted Awards may be made, if applicable) in the manner determined by the Board. Without limiting the generality of the foregoing, in the event the Company effects a split of the Common Stock by means of a stock dividend and the exercise price of and the number of shares subject to an outstanding Option are adjusted as of the date of the distribution of the dividend (rather than as of the record date for such dividend), then an optionee who exercises an Option between the record date and the distribution date for such stock dividend shall be entitled to receive, on the distribution date, the stock dividend with respect to the shares of Common Stock acquired upon such Option exercise, notwithstanding the fact that such shares were not outstanding as of the close of business on the record date for such stock dividend.

(b) Reorganization Events.

(1) Definition. A "***Reorganization Event***" shall mean: (i) any merger or consolidation of the Company with or into another entity as a result of which all of the Common Stock of the Company is converted into or exchanged for the right to receive cash, securities or other property or is cancelled, (ii) any transfer or disposition of all of the Common Stock of the Company for cash, securities or other property pursuant to a share exchange or other transaction or (iii) any liquidation or dissolution of the Company.

(2) Consequences of a Reorganization Event on Awards Other than Restricted Stock.

(A) In connection with a Reorganization Event, the Board may take any one or more of the following actions as to all or any (or any portion of) outstanding Awards other than Restricted Stock on such terms as the Board determines (except to the extent specifically provided otherwise in an applicable Award agreement or another agreement between the Company and the Participant): (i) provide that such Awards shall be assumed, or substantially equivalent Awards shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof), (ii) upon written notice to a Participant, provide that all of the Participant's unvested Awards will be forfeited immediately prior to the consummation of such Reorganization Event and/or that all of the Participant's unexercised Awards will terminate immediately prior to the consummation of such Reorganization Event unless exercised by the Participant (to the extent then exercisable) within a specified period following the date of such notice, (iii) provide that outstanding Awards shall become exercisable, realizable or deliverable, or restrictions applicable to an Award shall lapse, in whole or in part prior to or upon such Reorganization Event, (iv) in the event of a Reorganization Event under the terms of which holders of Common Stock will receive upon consummation thereof a cash payment for each share surrendered in the Reorganization Event (the "***Acquisition Price***"), make or provide for a cash payment to Participants with respect to each Award held by a Participant equal to (A) the number of shares of Common Stock subject to the vested portion of the Award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such Reorganization Event) multiplied by (B) the excess, if any, of (I) the Acquisition Price over (II) the exercise, measurement or purchase price of such Award and any applicable tax withholdings, in exchange for the termination of such Award, (v) provide that, in connection with a liquidation or dissolution of the Company, Awards shall convert into the right to receive liquidation proceeds (if applicable, net of the exercise, measurement or purchase price thereof and any applicable tax withholdings) and (vi) any combination of the foregoing. In taking any of the actions permitted under this Section 9(b)(2), the Board shall not be obligated by the Plan to treat all Awards, all Awards held by a Participant, or all Awards of the same type, identically.

(B) Notwithstanding the terms of Section 9(b)(2)(A), in the case of outstanding RSUs that are subject to Section 409A of the Code: (i) if the applicable RSU agreement provides that the RSUs shall be settled upon a "change in control event" within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i), and the Reorganization Event constitutes such a "change in control event", then no assumption or substitution shall be permitted pursuant to Section 9(b)(2)(A)(i) and the RSUs shall instead be settled in accordance with the terms of the applicable RSU agreement; and (ii) the Board may only undertake the actions set forth in clauses (iii), (iv) or (v) of Section 9(b)(2)(A) if the Reorganization Event constitutes a "change in control event" as defined under Treasury Regulation Section 1.409A-3(i)(5)(i) and such action is permitted or required by Section 409A of the Code; if the Reorganization Event is not a "change in control event" as so defined or such action is not permitted or required by Section 409A of the Code, and the acquiring or succeeding corporation does not assume or substitute the RSUs pursuant to clause (i) of Section 9(b)(2)(A), then the unvested RSUs shall terminate immediately prior to the consummation of the Reorganization Event without any payment in exchange therefor.

(C) For purposes of Section 9(b)(2)(A)(i), an Award (other than Restricted Stock) shall be considered assumed if, following consummation of the Reorganization Event, such Award confers the right to purchase or receive pursuant to the terms of such Award, for each share of Common Stock subject to the Award immediately prior to the consummation of the Reorganization Event, the consideration (whether cash, securities or other property) received as a result of the Reorganization Event by holders of Common Stock for each share of Common Stock held immediately prior to the consummation of the Reorganization Event (and if holders were offered a choice of consideration, the type of consideration chosen by the holders of a majority of the outstanding shares of Common Stock); *provided, however*, that if the consideration received as a result of the Reorganization Event is not solely common stock of the acquiring or succeeding corporation (or an affiliate thereof), the Company may, with the consent of the acquiring or succeeding corporation, provide for the consideration to be received upon the exercise or settlement of the Award to consist solely of such number of shares of common stock of the acquiring or succeeding corporation (or an affiliate thereof) that the Board determines to be equivalent in value (as of the date of such determination or another date specified by the Board) to the per share consideration received by holders of outstanding shares of Common Stock as a result of the Reorganization Event.

(3) Consequences of a Reorganization Event on Restricted Stock. Upon the occurrence of a Reorganization Event other than a liquidation or dissolution of the Company, the repurchase and other rights of the Company with respect to outstanding Restricted Stock shall inure to the benefit of the Company's successor and

shall, unless the Board determines otherwise, apply to the cash, securities or other property which the Common Stock was converted into or exchanged for pursuant to such Reorganization Event in the same manner and to the same extent as they applied to such Restricted Stock; *provided, however*, that the Board may either provide for termination or deemed satisfaction of such repurchase or other rights under the instrument evidencing any Restricted Stock or any other agreement between a Participant and the Company, either initially or by amendment, or provide for forfeiture of such Restricted Stock if issued at no cost. Upon the occurrence of a Reorganization Event involving the liquidation or dissolution of the Company, except to the extent specifically provided to the contrary in the instrument evidencing any Restricted Stock or any other agreement between a Participant and the Company, all restrictions and conditions on all Restricted Stock then outstanding shall automatically be deemed terminated or satisfied.

10. General Provisions Applicable to Awards

(a) Transferability of Awards. Awards shall not be sold, assigned, transferred, pledged or otherwise encumbered by the Participant, either voluntarily or by operation of law, except by will or the laws of descent and distribution or pursuant to a qualified domestic relations order, and, during the life of the Participant, shall be exercisable only by the Participant; *provided, however*, that, except with respect to Awards subject to Section 409A of the Code, the Board may permit or provide in an Award for the gratuitous transfer of the Award by the Participant to or for the benefit of any immediate family member, family trust or other entity established for the benefit of the Participant and/or an immediate family member thereof if the Company would be eligible to use a Form S-8 under the Securities Act of 1933, as amended, for the registration of the sale of the Common Stock subject to such Award to such proposed transferee; *provided further*, that the Company shall not be required to recognize any such permitted transfer until such time as such permitted transferee shall, as a condition to such transfer, deliver to the Company a written instrument in form and substance satisfactory to the Company confirming that such transferee shall be bound by all of the terms and conditions of the Award. References to a Participant, to the extent relevant in the context, shall include references to authorized transferees. For the avoidance of doubt, nothing contained in this Section 10(a) shall be deemed to restrict a transfer to the Company.

(b) Documentation; Press Release. Each Award shall be evidenced in such form (written, electronic or otherwise) as the Board shall determine. Each Award may contain terms and conditions in addition to those set forth in the Plan. Promptly following the grant of an Award hereunder, the Company must disclose in a press release the material terms of the grant, the number of shares involved, and, if required by law or the rules of the Exchange, the identity of the Participant and each Participant, by accepting the Award, consents to the foregoing.

(c) Termination of Status. The Board shall determine the effect on an Award of the disability, death, termination or other cessation of employment, authorized leave of absence or other change in the employment or other status of a Participant and the extent to which, and the period during which, the Participant, or the Participant's legal representative, conservator, guardian or Designated Beneficiary, may exercise rights or receive any benefits under the Award.

(d) Withholding. The Participant must satisfy all applicable federal, state, and local or other income and employment tax withholding obligations before the Company will deliver stock certificates or otherwise recognize ownership of Common Stock under an Award. The Company may elect to satisfy the withholding obligations through additional withholding on salary or wages. If the Company elects not to or cannot withhold from other compensation, the Participant must pay the Company the full amount, if any, required for withholding or have a broker tender to the Company cash equal to the withholding obligations. Payment of withholding obligations is due before the Company will issue any shares on exercise, vesting or release from forfeiture of an Award or at the same time as payment of the exercise or purchase price, unless the Company determines otherwise. If provided for in an Award or approved by the Board, a Participant may satisfy the tax obligations in whole or in part by delivery (either by actual delivery or attestation) of shares of Common Stock, including shares retained from the Award creating the tax obligation, valued at their fair market value (valued in the manner determined by (or in a manner approved by) the Company); *provided*, *however*, except as otherwise provided by the Board, that the total tax withholding where stock is being used to satisfy such tax obligations cannot exceed the Company's minimum statutory withholding obligations (based on minimum statutory withholding rates for federal, state and local tax purposes, including payroll taxes, that are applicable to such supplemental taxable income), except that, to the extent that the Company is able to retain shares of Common Stock having a fair market value (determined by, or in a

manner approved by, the Company) that exceeds the statutory minimum applicable withholding tax without financial accounting implications or the Company is withholding in a jurisdiction that does not have a statutory minimum withholding tax, the Company may retain such number of shares of Common Stock (up to the number of shares having a fair market value equal to the maximum individual statutory rate of tax (determined by, or in a manner approved by, the Company)) as the Company shall determine in its sole discretion to satisfy the tax liability associated with any Award. Shares used to satisfy tax withholding requirements cannot be subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements.

(e) Amendment of Award. Except as otherwise provided in Sections 5(f) and 6(e) with respect to repricings, the Board may amend, modify or terminate any outstanding Award, including but not limited to, substituting therefor another Award of the same or a different type and changing the date of exercise or realization. The Participant's consent to such action shall be required unless (i) the Board determines that the action, taking into account any related action, does not materially and adversely affect the Participant's rights under the Plan or (ii) the change is permitted under Section 9.

(f) Conditions on Delivery of Stock. The Company will not be obligated to deliver any shares of Common Stock pursuant to the Plan or to remove restrictions from shares previously issued or delivered under the Plan until (i) all conditions of the Award have been met or removed to the satisfaction of the Company, (ii) in the opinion of the Company's counsel, all other legal matters in connection with the issuance and delivery of such shares have been satisfied, including any applicable securities laws and regulations and any applicable stock exchange or stock market rules and regulations and (iii) the Participant has executed and delivered to the Company such representations or agreements as the Company may consider appropriate to satisfy the requirements of any applicable laws, rules or regulations.

(g) Acceleration. The Board may at any time provide that any Award shall become immediately exercisable in whole or in part, free from some or all restrictions or conditions, or otherwise realizable in whole or in part, as the case may be.

11. Miscellaneous

(a) No Right To Employment or Other Status. No person shall have any claim or right to be granted an Award by virtue of the adoption of the Plan, and the grant of an Award shall not be construed as giving a Participant the right to continued employment or any other relationship with the Company. The Company expressly reserves the right at any time to dismiss or otherwise terminate its relationship with a Participant free from any liability or claim under the Plan, except as expressly provided in the applicable Award.

(b) No Rights As Stockholder; Clawback. Subject to the provisions of the applicable Award, no Participant or Designated Beneficiary shall have any rights as a stockholder with respect to any shares of Common Stock to be issued with respect to an Award until becoming the record holder of such shares. In accepting an Award under the Plan, a Participant agrees to be bound by any clawback policy the Company has in effect or may adopt in the future.

(c) Effective Date. The Plan shall become effective on the date on which it is adopted by the Board. It is expressly intended that approval of the Company's stockholders not be required as a condition to the effectiveness of the Plan, and the Plan's provisions shall be interpreted in a manner consistent with such intent for all purposes.

(d) Amendment of Plan. The Board may amend, suspend or terminate the Plan or any portion thereof at any time provided that no amendment that would require stockholder approval under the rules of the Exchange may be made effective unless and until the Company's stockholders approve such amendment. Unless otherwise specified in the amendment, any amendment to the Plan adopted in accordance with this Section 11(d) shall apply to, and be binding on the holders of, all Awards outstanding under the Plan at the time the amendment is adopted, provided the Board determines that such amendment, taking into account any related action, does not materially and adversely affect the rights of Participants under the Plan.

(e) Authorization of Sub-Plans (including for Grants to non-U.S. Employees). The Board may from time to time establish one or more sub-plans under the Plan for purposes of satisfying applicable securities, tax or

other laws of various jurisdictions. The Board shall establish such sub-plans by adopting supplements to the Plan containing (i) such limitations on the Board's discretion under the Plan as the Board deems necessary or desirable or (ii) such additional terms and conditions not otherwise inconsistent with the Plan as the Board shall deem necessary or desirable. All supplements adopted by the Board shall be deemed to be part of the Plan, but each supplement shall apply only to Participants within the affected jurisdiction and the Company shall not be required to provide copies of any supplement to Participants in any jurisdiction which is not the subject of such supplement.

(f) Compliance with Section 409A of the Code. If and to the extent (i) any portion of any payment, compensation or other benefit provided to a Participant pursuant to the Plan in connection with his or her employment termination constitutes "nonqualified deferred compensation" within the meaning of Section 409A of the Code and (ii) the Participant is a specified employee as defined in Section 409A(a)(2)(B)(i) of the Code, in each case as determined by the Company in accordance with its procedures, by which determinations the Participant (through accepting the Award) agrees that he or she is bound, such portion of the payment, compensation or other benefit shall not be paid before the day that is six months plus one day after the date of "separation from service" (as determined under Section 409A of the Code) (the "***New Payment Date***"), except as Section 409A of the Code may then permit. The aggregate of any payments that otherwise would have been paid to the Participant during the period between the date of separation from service and the New Payment Date shall be paid to the Participant in a lump sum on such New Payment Date, and any remaining payments will be paid on their original schedule.

The Company makes no representations or warranty and shall have no liability to the Participant or any other person if any provisions of or payments, compensation or other benefits under the Plan are determined to constitute nonqualified deferred compensation subject to Section 409A of the Code but do not to satisfy the conditions of that section.

(g) Limitations on Liability. Notwithstanding any other provisions of the Plan, no individual acting as a director, officer, employee or agent of the Company will be liable to any Participant, former Participant, spouse, beneficiary, or any other person for any claim, loss, liability, or expense incurred in connection with the Plan, nor will such individual be personally liable with respect to the Plan because of any contract or other instrument he or she executes in his or her capacity as a director, officer, employee or agent of the Company. The Company will indemnify and hold harmless each director, officer, employee or agent of the Company to whom any duty or power relating to the administration or interpretation of the Plan has been or will be delegated, against any cost or expense (including attorneys' fees) or liability (including any sum paid in settlement of a claim with the Board's approval) arising out of any act or omission to act concerning the Plan unless arising out of such person's own fraud or bad faith.

(h) Governing Law. The provisions of the Plan and all Awards made hereunder shall be governed by and interpreted in accordance with the laws of the State of Delaware, excluding choice-of-law principles of the law of such state that would require the application of the laws of a jurisdiction other than the State of Delaware.

Exhibit 10.50

SOLID BIOSCIENCES INC.
NONSTATUTORY STOCK OPTION AGREEMENT

Granted under 2024 Inducement Stock Incentive Plan

Solid Biosciences Inc. (the "Company") hereby grants the following stock option pursuant to its 2024 Inducement Stock Incentive Plan. The terms and conditions attached hereto are also a part hereof.

Notice of Grant

Name of optionee (the "Participant"):	
Grant Date:	
Number of shares of the Company's Common Stock subject to this option ("Shares"):	
Option exercise price per Share:	
Number, if any, of Shares that vest immediately on the grant date:	
Shares that are subject to vesting schedule:	
Vesting Start Date:	
Final Exercise Date:	

Vesting Schedule:

Vesting Date:	Number of Options that Vest:
All vesting is dependent on the Participant remaining an Eligible Participant, as provided herein.	

This option satisfies in full all commitments that the Company has to the Participant with respect to the issuance of stock, stock options or other equity securities.

Solid Biosciences Inc.

Signature of Participant

Street Address

City/State/Zip Code

By: _____

Name of Officer

Title:

Solid Biosciences Inc.

Nonstatutory Stock Option Agreement
Granted under 2024 Inducement Stock Incentive Plan
Incorporated Terms and Conditions

1. Grant of Option.

This agreement evidences the grant by the Company, on the grant date (the "Grant Date") set forth in the Notice of Grant that forms part of this agreement (the "Notice of Grant"), to the Participant of an option to purchase, in whole or in part, on the terms provided herein and in the Company's 2024 Inducement Stock Incentive Plan (the "Plan"), the number of Shares set forth in the Notice of Grant of common stock, $0.001 par value per share, of the Company ("Common Stock"), at the exercise price per Share set forth in the Notice of Grant. Unless earlier terminated, this option shall expire at 5:00 p.m., Eastern time, on the Final Exercise Date set forth in the Notice of Grant (the "Final Exercise Date").

The option evidenced by this agreement was granted to the Participant pursuant to the inducement grant exception under Nasdaq Stock Market Rule 5635(c)(4) as an inducement that is material to the Participant's employment with the Company.

The option evidenced by this agreement shall not be an incentive stock option as defined in Section 422 of the Internal Revenue Code of 1986, as amended, and any regulations promulgated thereunder (the "Code"). Except as otherwise indicated by the context, the term "Participant", as used in this option, shall be deemed to include any person who acquires the right to exercise this option validly under its terms.

2. Vesting Schedule.

This option will become exercisable ("vest") in accordance with the vesting schedule set forth in the Notice of Grant.

The right of exercise shall be cumulative so that to the extent the option is not exercised in any period to the maximum extent permissible it shall continue to be exercisable, in whole or in part, with respect to all Shares for which it is vested until the earlier of the Final Exercise Date or the termination of this option under Section 3 hereof or the Plan.

3. Exercise of Option.

(a) Form of Exercise. Each election to exercise this option shall be in writing, in the form of the Stock Option Exercise Notice attached as Annex A, signed by the Participant, and received by the Company at its principal office, accompanied by this agreement, or in such other form (which may be electronic) as is approved by the Company, together with payment in full in the manner provided in the Plan. The Participant may purchase less than the number of shares covered hereby, provided that no partial exercise of this option may be for any fractional share.

(b) Continuous Relationship with the Company Required. Except as otherwise provided in this Section 3, this option may not be exercised unless the Participant, at the time he or she exercises this option, is, and has been at all times since the Grant Date, an employee, director or officer of, or consultant or advisor to, the Company or any other entity the employees, officers, directors, consultants, or advisors of which are eligible to receive option grants under the Plan (an "Eligible Participant").

(c) Termination of Relationship with the Company. If the Participant ceases to be an Eligible Participant for any reason, then, except as provided in paragraphs (d) and (e) below, the right to exercise this option shall terminate three months after such cessation (but in no event after the Final Exercise Date), provided that this option shall be exercisable only to the extent that the Participant was entitled to exercise this option on the date of such cessation. Notwithstanding the foregoing, if the Participant, prior to the Final Exercise Date, violates the restrictive covenants (including, without limitation, the non-competition, non-solicitation, or confidentiality provisions) of any employment contract, any non-competition, non-solicitation, confidentiality or assignment agreement to which the Participant is a party, or any other agreement between the Participant and the Company, the right to exercise this option shall terminate immediately upon such violation.

(d) Exercise Period Upon Death or Disability. If the Participant dies or becomes disabled (within the meaning of Section 22(e)(3) of the Code) prior to the Final Exercise Date while he or she is an Eligible Participant and the Company has not terminated such relationship for "cause" as specified in paragraph (e) below, this option shall be exercisable, within the period of one year following the date of death or disability of the Participant, by the Participant (or in the case of death by an authorized transferee), provided that this option shall be exercisable only to the extent that this option was exercisable by the Participant on the date of his or her death or disability, and further provided that this option shall not be exercisable after the Final Exercise Date.

(e) Termination for Cause. If, prior to the Final Exercise Date, the Participant's employment or other relationship with the Company is terminated by the Company for Cause (as defined below), the right to exercise this option shall terminate immediately upon the effective date of such termination of employment or other relationship. If, prior to the Final Exercise Date, the Participant is given notice by the Company of the termination of his or her employment or other relationship by the Company for Cause, and the effective date of such termination is subsequent to the date of delivery of such notice, the right to exercise this option shall be suspended from the time of the delivery of such notice until the earlier of (i) such time as it is determined or otherwise agreed that the Participant's employment or other relationship shall not be terminated for Cause as provided in such notice or (ii) the effective date of such termination of employment or other relationship (in which case the right to exercise this option shall, pursuant to the preceding sentence, terminate upon the effective date of such termination of employment or other relationship). If the Participant is subject to an individual employment, consulting or other service agreement with the Company or eligible to participate in a Company severance plan or arrangement, in any case which agreement, plan or arrangement contains a definition of "cause" for termination of employment or other relationship, "Cause" shall have the meaning ascribed to such term in such agreement, plan or arrangement. Otherwise, "Cause" shall mean willful misconduct by the Participant or willful failure by the Participant to perform

his or her responsibilities to the Company (including, without limitation, breach by the Participant of any provision of any employment, consulting, advisory, nondisclosure, non-competition or other similar agreement between the Participant and the Company), as determined by the Company, which determination shall be conclusive. The Participant's employment or other relationship shall be considered to have been terminated for Cause if the Company determines, within 30 days after the Participant's resignation, that termination for Cause was warranted.

4. Withholding. No Shares will be issued pursuant to the exercise of this option unless and until the Participant pays to the Company, or makes provision satisfactory to the Company for payment of, any federal, state or local withholding taxes required by law to be withheld in respect of this option.

5. Transfer Restrictions; Clawback.

(a) This option may not be sold, assigned, transferred, pledged, encumbered or otherwise disposed of by the Participant, either voluntarily or by operation of law, except by will or the laws of descent and distribution, and, during the lifetime of the Participant, this option shall be exercisable only by the Participant.

(b) In accepting this option, the Participant agrees to be bound by any clawback policy that the Company has in place or may adopt in the future.

6. Provisions of the Plan.

This option is subject to the provisions of the Plan (including the provisions relating to amendments to the Plan), a copy of which is furnished to the Participant with this option.

ANNEX A

Solid Biosciences Inc.

Stock Option Exercise Notice

Solid Biosciences Inc.
[Address]
[Address]

Dear Sir or Madam:

I, _____ (the "Participant"), hereby irrevocably exercise the right to purchase _____shares of the Common Stock, $0.001 par value per share (the "Shares"), of Solid Biosciences Inc. (the "Company") at $_____ per share pursuant to the Company's 2024 Inducement Stock Incentive Plan and a nonstatutory stock option agreement with the Company dated _____ (the "Option Agreement"). Enclosed herewith is a payment of $_____, the aggregate purchase price for the Shares. The certificate for the Shares should be registered in my name as it appears below or, if so indicated below, jointly in my name and the name of the person designated below, with right of survivorship.

Dated:

Signature
Print Name:

Address:

Name and address of persons in whose name the Shares are to be jointly registered (if applicable):

Exhibit 10.51

SOLID BIOSCIENCES INC.
RESTRICTED STOCK UNIT AGREEMENT

Granted under 2024 Inducement Stock Incentive Plan

Solid Biosciences Inc. (the "Company") hereby grants the following restricted stock units pursuant to its 2024 Inducement Stock Incentive Plan. The terms and conditions attached hereto are also a part hereof.

Notice of Grant

Name of recipient (the "Participant"):	
Grant Date:	
Number of restricted stock units ("RSUs") granted:	
Vesting Start Date:	

Vesting Schedule:

Vesting Date:	Number of RSUs that Vest:
All vesting is dependent on the Participant remaining an Eligible Participant, as provided herein.	

This grant of RSUs satisfies in full all commitments that the Company has to the Participant with respect to the issuance of stock, stock options or other equity securities.

Solid Biosciences Inc.

Signature of Participant

By: _____

Street Address

Name of Officer
Title:

City/State/Zip Code

Solid Biosciences Inc.

Restricted Stock Unit Agreement
Granted under 2024 Inducement Stock Incentive Plan
Incorporated Terms and Conditions

1. Award of Restricted Stock Units.

In connection with the commencement of the Participant's employment with the Company, the Company has granted to the Participant, subject to the terms and conditions set forth in this Restricted Stock Unit Agreement (this "Agreement") and in the Company's 2024 Inducement Stock Incentive Plan (the "Plan"), an award with respect to the number of restricted stock units (the "RSUs") set forth in the Notice of Grant that forms part of this Agreement (the "Notice of Grant"). Each RSU represents the right to receive one share of common stock, $0.001 par value per share, of the Company (the "Common Stock") upon vesting of the RSU, subject to the terms and conditions set forth herein.

The RSUs evidenced by this Agreement were granted to the Participant pursuant to the inducement grant exception under Nasdaq Stock Market Rule 5635(c)(4), as an inducement that is material to the Participant's employment with the Company.

2. Vesting.

The RSUs shall vest in accordance with the Vesting Schedule set forth in the Notice of Grant (the "Vesting Schedule"). Any fractional shares resulting from the application of any percentages used in the Vesting Schedule shall be rounded down to the nearest whole number of RSUs. Upon the vesting of the RSU, the Company will deliver to the Participant, for each RSU that becomes vested, one share of Common Stock, subject to the payment of any taxes pursuant to Section 7. The Common Stock will be delivered to the Participant as soon as practicable following each vesting date, but in any event within 30 days of such date.

3. Forfeiture of Unvested RSUs Upon Cessation of Service.

In the event that the Participant ceases to be an employee, director or officer of, or consultant or advisor to, the Company or any other entity the employees, officers, directors, consultants, or advisors of which are eligible to receive awards under the Plan (an "Eligible Participant") for any reason or no reason, with or without cause, all of the RSUs that are unvested as of the time of such cessation shall be forfeited immediately and automatically to the Company, without the payment of any consideration to the Participant, effective as of such cessation. The Participant shall have no further rights with respect to the unvested RSUs or any Common Stock that may have been issuable with respect thereto.

4. Restrictions on Transfer.

The Participant shall not sell, assign, transfer, pledge, hypothecate, encumber or otherwise dispose of, by operation of law or otherwise (collectively "transfer") any RSUs, or any interest therein. The Company shall not be required to treat as the owner of any RSUs or issue any Common Stock to any transferee to whom such RSUs have been transferred in violation of any of the provisions of this Agreement.

5. Rights as a Stockholder.

The Participant shall have no rights as a stockholder of the Company with respect to any shares of Common Stock that may be issuable with respect to the RSUs until the issuance of the shares of Common Stock to the Participant following the vesting of the RSUs.

6. Provisions of the Plan.

This Agreement is subject to the provisions of the Plan, a copy of which is furnished to the Participant with this Agreement.

7. Tax Matters.

(a) Acknowledgments; No Section 83(b) Election. The Participant acknowledges that he or she is responsible for obtaining the advice of the Participant's own tax advisors with respect to the award of RSUs and the Participant is relying solely on such advisors and not on any statements or representations of the Company or any of its agents with respect to the tax consequences relating to the RSUs. The Participant understands that the Participant (and not the Company) shall be responsible for the Participant's tax liability that may arise in connection with the acquisition, vesting and/or disposition of the RSUs. The Participant acknowledges that no election under Section 83(b) of the Internal Revenue Code of 1986, as amended (the "Code"), is available with respect to RSUs.

(b) Withholding. The Participant acknowledges and agrees that the Company has the right to deduct from payments of any kind otherwise due to the Participant any federal, state, local or other taxes of any kind required by law to be withheld with respect to the vesting of the RSUs. To the extent the Participant has not previously executed and delivered to the Company effective durable sell-to-cover instructions that by their terms would cover any taxes required by law to be withheld with respect to the vesting of the RSUs, at such time as the Participant is not aware of any material nonpublic information about the Company or the Common Stock and is not prohibited from doing so by the Company's insider trading policy or otherwise, the Participant shall execute the instructions set forth in Schedule A attached hereto (the "Durable Automatic Sell-to-Cover Instruction") as the means of satisfying such tax obligation. If the Participant is required to but does not execute the Durable Automatic Sell-to-Cover Instruction prior to an applicable vesting date, then the Participant agrees that if under applicable law the Participant will owe taxes at such vesting date of RSUs issued hereunder on the portion of the award then vested, the Company shall be entitled to immediate payment from the Participant of the amount of any tax required to be withheld by the Company. The Company

shall not deliver any shares of Common Stock to the Participant until it is satisfied that all required withholdings have been made.

8. Miscellaneous.

(a) No Right to Continued Service. The Participant acknowledges and agrees that, notwithstanding the fact that the vesting of the RSUs is contingent upon his or her continued service to the Company, this Agreement does not constitute an express or implied promise of continued service relationship with the Participant or confer upon the Participant any rights with respect to a continued service relationship with the Company or any affiliate of the Company.

(b) Section 409A. The RSUs awarded pursuant to this Agreement are intended to be exempt from or comply with the requirements of Section 409A of the Code and the Treasury Regulations issued thereunder ("Section 409A"). The delivery of shares of Common Stock on the vesting of the RSUs may not be accelerated or deferred unless permitted or required by Section 409A.

(c) Participant's Acknowledgments. The Participant acknowledges that he or she: (i) has read this Agreement; (ii) has been represented in the preparation, negotiation and execution of this Agreement by legal counsel of the Participant's own choice or has voluntarily declined to seek such counsel; (iii) understands the terms and consequences of this Agreement; (iv) is agreeing, in accepting this award, to be bound by any clawback policy that the Company has in place or may adopt in the future; and (iv) is fully aware of the legal and binding effect of this Agreement.

(d) Governing Law. This Agreement shall be construed, interpreted and enforced in accordance with the internal laws of the State of Delaware without regard to any applicable conflicts of laws provisions.

Schedule A

DURABLE AUTOMATIC SELL-TO-COVER INSTRUCTION

This Durable Automatic Sell-to-Cover Instruction (this "Instruction"), which is being delivered to Solid Biosciences Inc. (the "Company") by the undersigned on the date set forth below (the "Adoption Date"), relates to any restricted stock units that may be granted to me from time to time by the Company under the Company's equity compensation programs, other than any restricted stock units which by the terms of the applicable award agreement require the Company to withhold shares for tax withholding obligations in connection with the vesting and settlement of such restricted stock units and therefore do not permit sell-to-cover transactions (the restricted stock units subject to this Instruction are referred to as "Covered RSUs"). This Instruction provides for "eligible sell-to-cover transactions" (as described in Rule 10b5-1(c)(1)(ii)(D)(3) under the Securities Exchange Act of 1934 (the "Exchange Act")) with respect to Covered RSUs and is intended to satisfy the affirmative defense conditions of Rule 10b5-1(c)(1) under the Exchange Act.

I acknowledge that upon vesting and settlement of any Covered RSUs in accordance with the applicable RSU's terms, whether vesting is based on the passage of time or the achievement of performance goals, I will have compensation income equal to the fair market value of the shares of the Company's common stock subject to the RSUs that are settled on such settlement date and that the Company is required to withhold income and employment taxes in respect of that compensation income.

I desire to establish a plan and process to satisfy such withholding obligation in respect of all Covered RSUs through an automatic sale of the number of the shares of the Company's common stock that would otherwise be issuable to me on each applicable settlement date in an amount sufficient to satisfy the applicable withholding obligation, with the proceeds of the sale delivered to the Company in satisfaction of the applicable withholding obligation.

I understand that the Company has arranged for the administration and execution of its equity incentive programs and the sale of securities by participants thereunder pursuant to a platform administered by a third party (the "Administrator") and the Administrator's designated brokerage partner.

Upon the settlement of any of my Covered RSUs after the 30th day following the Adoption Date (or if I am an officer of the Company on the Adoption Date, after the [120th day following the Adoption Date]) (the "Cooling-Off Period"), I hereby appoint the Administrator (or any successor administrator) to automatically sell such number of shares of the Company's common stock issuable with respect to such RSUs that vested and settled as is sufficient to generate net proceeds sufficient to satisfy the Company's minimum statutory withholding obligations with respect to the income recognized by me in connection with the vesting and settlement of such RSUs (based on minimum statutory withholding rates for all tax purposes, including payroll and social security taxes, that are applicable to such income), and the Company shall receive such net proceeds in satisfaction of such tax withholding obligation.

I hereby appoint the Chief Executive Officer, the Chief Financial Officer and the General Counsel (or, if none, the Chief Operating Officer), and any of them acting alone and with full power of substitution, to serve as my attorneys-in-fact to arrange for the sale of shares of the Company's common stock in accordance with this Instruction. I agree to execute and deliver such documents, instruments and certificates as may reasonably be required in connection with the sale of the shares of common stock pursuant to this Instruction.

I hereby certify that, as of the Adoption Date:

(i) I am not prohibited from entering into this Instruction by the Company's insider trading policy or otherwise;

(ii) I am not aware of any material nonpublic information about the Company or its common stock; and

(iii) I am adopting this Instruction in good faith and not as part of a plan or scheme to evade the prohibitions of Rule 10b-5 under the Exchange Act.

_____

Print Name: _____

Date: _____

Exhibit 10.52

SOLID BIOSCIENCES INC.
RESTRICTED STOCK UNIT AGREEMENT

Solid Biosciences Inc. (the "Company") hereby grants the following restricted stock units pursuant to its 2020 Equity Incentive Plan. The terms and conditions attached hereto are also a part hereof.

Notice of Grant

Name of recipient (the "Participant"):	
Grant Date:	
Number of restricted stock units ("RSUs") granted:	
Vesting Start Date:	

Vesting Schedule:

Vesting Date:	Number of RSUs that Vest:
All vesting is dependent on the Participant remaining an Eligible Participant, as provided herein.	

This grant of RSUs satisfies in full all commitments that the Company has to the Participant with respect to the issuance of stock, stock options or other equity securities.

SOLID BIOSCIENCES INC.

Signature of Participant

By: _____

Name of Officer

Street Address

Title:

City/State/Zip Code

Solid Biosciences Inc.

Restricted Stock Unit Agreement
Incorporated Terms and Conditions

1. Award of Restricted Stock Units.

In consideration of services rendered and to be rendered to the Company, by the Participant, the Company has granted to the Participant, subject to the terms and conditions set forth in this Restricted Stock Unit Agreement (this "Agreement") and in the Company's 2020 Equity Incentive Plan (the "Plan"), an award with respect to the number of restricted stock units (the "RSUs") set forth in the Notice of Grant that forms part of this Agreement (the "Notice of Grant"). Each RSU represents the right to receive one share of common stock, $0.001 par value per share, of the Company (the "Common Stock") upon vesting of the RSU, subject to the terms and conditions set forth herein.

2. Vesting.

The RSUs shall vest in accordance with the Vesting Schedule set forth in the Notice of Grant (the "Vesting Schedule"). Any fractional shares resulting from the application of any percentages used in the Vesting Schedule shall be rounded down to the nearest whole number of RSUs. Upon the vesting of the RSU, the Company will deliver to the Participant, for each RSU that becomes vested, one share of Common Stock, subject to the payment of any taxes pursuant to Section 7. The Common Stock will be delivered to the Participant as soon as practicable following each vesting date, but in any event within 30 days of such date.

3. Forfeiture of Unvested RSUs Upon Cessation of Service.

In the event that the Participant ceases to be an Eligible Participant (as defined below) for any reason or no reason, with or without cause, all of the RSUs that are unvested as of the time of such cessation shall be forfeited immediately and automatically to the Company, without the payment of any consideration to the Participant, effective as of such cessation. The Participant shall have no further rights with respect to the unvested RSUs or any Common Stock that may have been issuable with respect thereto. The Participant shall be an "Eligible Participant" if he or she is an employee, director or officer of, or consultant or advisor to, the Company or any other entity the employees, officers, directors, consultants or advisors of which are eligible to receive awards of RSUs under the Plan.

4. Restrictions on Transfer.

The Participant shall not sell, assign, transfer, pledge, hypothecate, encumber or otherwise dispose of, by operation of law or otherwise (collectively "transfer") any RSUs, or any interest therein. The Company shall not be required to treat as the owner of any RSUs or issue any Common Stock to any transferee to whom such RSUs have been transferred in violation of any of the provisions of this Agreement.

5. Rights as a Stockholder.

The Participant shall have no rights as a stockholder of the Company with respect to any shares of Common Stock that may be issuable with respect to the RSUs until the issuance of the shares of Common Stock to the Participant following the vesting of the RSUs.

6. Provisions of the Plan.

This Agreement is subject to the provisions of the Plan, a copy of which is furnished to the Participant with this Agreement.

7. Tax Matters.

(a) Acknowledgments; No Section 83(b) Election. The Participant acknowledges that he or she is responsible for obtaining the advice of the Participant's own tax advisors with respect to the award of RSUs and the Participant is relying solely on such advisors and not on any statements or representations of the Company or any of its agents with respect to the tax consequences relating to the RSUs. The Participant understands that the Participant (and not the Company) shall be responsible for the Participant's tax liability that may arise in connection with the acquisition, vesting and/or disposition of the RSUs. The Participant acknowledges that no election under Section 83(b) of the Internal Revenue Code of 1986, as amended, (the "Code") is available with respect to RSUs.

(b) Responsibility for Taxes; Withholding. The Participant acknowledges that, regardless of any action taken by the Company, the ultimate liability for all federal, state, local or other taxes of any kind related to the Participant's participation in the Plan and legally applicable to the Participant is and remains the Participant's responsibility and may exceed the amount, if any, actually withheld by the Company. The Participant acknowledges and agrees that the Company has the right to deduct from payments of any kind otherwise due to the Participant any federal, state, local or other taxes of any kind required by law to be withheld with respect to the vesting of the RSUs. To the extent the Participant has not previously executed and delivered to the Company effective durable sell-to-cover instructions that by their terms would cover any taxes required by law to be withheld with respect to the vesting of the RSUs, at such time as the Participant is not aware of any material nonpublic information about the Company or the Common Stock and is not prohibited from doing so by the Company's insider trading policy or otherwise, the Participant shall execute the instructions set forth in Schedule A attached hereto (the "Durable Automatic Sell-to-Cover Instruction") as the means of satisfying such tax obligation. If the Participant is required to but does not execute the Durable Automatic Sell-to-Cover Instruction prior to an applicable vesting date, then the Participant agrees that if under applicable law the Participant will owe taxes at such vesting date of RSUs issued hereunder on the portion of the award then vested, the Company shall be entitled to immediate payment from the Participant of the amount of any tax required to be withheld by the Company. The Company shall not deliver any shares of Common Stock to the Participant until it is satisfied that all required withholdings have been made.

8. Miscellaneous.

(a) Section 409A. The RSUs awarded pursuant to this Agreement are intended to be exempt from or comply with the requirements of Section 409A of the Code and the Treasury

Regulations issued thereunder ("Section 409A"). The delivery of shares of Common Stock on the vesting of the RSUs may not be accelerated or deferred unless permitted or required by Section 409A.

(b) Participant's Acknowledgements. The Participant acknowledges that he or she: (i) has read this Agreement; (ii) has been represented in the preparation, negotiation and execution of this Agreement by legal counsel of the Participant's own choice or has voluntarily declined to seek such counsel; (iii) understands the terms and consequences of this Agreement; (iv) is fully aware of the legal and binding effect of this Agreement; and (v) agrees that in accepting this award, he or she will be bound by any clawback policy that the Company may adopt in the future.

Schedule A

DURABLE AUTOMATIC SELL-TO-COVER INSTRUCTION

This Durable Automatic Sell-to-Cover Instruction (this "Instruction"), which is being delivered to Solid Biosciences Inc. (the "Company") by the undersigned on the date set forth below (the "Adoption Date"), relates to the Covered RSUs (as defined following my signature below). This Instruction provides for "eligible sell-to-cover transactions" (as described in Rule 10b5-1(c)(1)(ii)(D)(3) under the Securities Exchange Act of 1934 (the "Exchange Act")) and is intended to satisfy the affirmative defense conditions of Rule 10b5-1(c)(1) under the Exchange Act.

I acknowledge that upon vesting and settlement of any Covered RSUs in accordance with the applicable RSU's terms, whether vesting is based on the passage of time or the achievement of performance goals, I will have compensation income equal to the fair market value of the shares of the Company's common stock subject to the RSUs that are settled on such settlement date and that the Company is required to withhold income and employment taxes in respect of that compensation income.

I desire to establish a plan and process to satisfy such withholding obligation in respect of all Covered RSUs through an automatic sale of the number of the shares of the Company's common stock that would otherwise be issuable to me on each applicable settlement date, in an amount sufficient to satisfy the applicable withholding obligation, with the proceeds of the sale delivered to the Company in satisfaction of the applicable withholding obligation.

I understand that the Company has arranged for the administration and execution of its equity incentive programs and the sale of securities by participants thereunder pursuant to a platform administered by a third party (the "Administrator") and the Administrator's designated brokerage partner.

Upon the settlement of any of my Covered RSUs after the 30th day following the Adoption Date (or if I am an officer of the Company on the Adoption Date, after the [120th day following the Adoption Date]) (the "Cooling-Off Period"), I hereby appoint the Administrator (or any successor administrator) to automatically sell such number of shares of the Company's common stock issuable with respect to such RSUs that vested and settled as is sufficient to generate net proceeds sufficient to satisfy the Company's minimum statutory withholding obligations with respect to the income recognized by me in connection with the vesting and settlement of such RSUs (based on minimum statutory withholding rates for all tax purposes, including payroll and social security taxes, that are applicable to such income), and the Company shall receive such net proceeds in satisfaction of such tax withholding obligation.

I hereby appoint the Chief Executive Officer, the Chief Financial Officer and the General Counsel (or, if none, the Chief Operating Officer), and any of them acting alone and with full power of substitution, to serve as my attorneys-in-fact to arrange for the sale of shares of the Company's common stock in accordance with this Instruction. I agree to execute and deliver such documents, instruments and certificates as may reasonably be required in connection with the sale of the shares of common stock pursuant to this Instruction.

Unless the last box in the definition of Covered RSUs below is checked, if I have previously adopted an automatic sale or sell-to-cover instruction relating to Covered RSUs, this Instruction shall be void *ab initio*.

I hereby certify that, as of the Adoption Date:

(i) I am not prohibited from entering into this Instruction by the Company's insider trading policy or otherwise;

(ii) I am not aware of any material nonpublic information about the Company or its common stock; and

(iii) I am adopting this Instruction in good faith and not as part of a plan or scheme to evade the prohibitions of Rule 10b-5 under the Exchange Act.

_____

Print Name: _____

Adoption Date: _____

Covered RSUs:
The following restricted stock units ("RSUs") are covered by this Instruction:

Any RSUs granted to me by the Company prior to, on or after the Adoption Date that, in each case, (1) are not subject to any prior automatic sale or sell-to-cover instruction and (2) for which the next vesting date is after the Cooling-Off Period referred to above, other than any future granted RSUs which by the terms of the applicable award agreement require the Company to withhold shares for tax withholding obligations in connection with the vesting and settlement of such RSUs, and therefore do not permit sell-to-cover transactions.

The following RSUs are covered by this Instruction *only if* the box below is checked:

☐ With respect to any RSUs, whether or not granted to me by the Company prior to the Adoption Date, that already are subject to an automatic sale or sell-to-cover instruction (a "Prior Instruction"), I elect to have such sales effected pursuant to this Instruction and confirm that doing so does not modify or change the amount, price, or timing of such sales from those provided by the Prior Instruction (and, as a result the Cooling-Off Period referred to above is not applicable to sales pursuant to this Instruction that were previously subject to the Prior Instruction).

Exhibit 10.53

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

PATENT LICENSE AGREEMENT

This Agreement is effective as of March 10, 2016 (the "EFFECTIVE DATE"), between Solid GT, LLC ("LICENSEE") having the address in Article 12 below, and the Regents of the University of Michigan, a constitutional corporation of the state of Michigan ("MICHIGAN") having the address in Article 12 below. LICENSEE and MICHIGAN hereby agree as follows:

ARTICLE 1 – DEFINITIONS

"AFFILIATE" means any corporation, partnership, joint venture or other entity of which a majority of the voting stock or other equity ownership thereof is owned or controlled by, or under common control with, LICENSEE, or which owns or controls a majority of the voting stock or other equity ownership of LICENSEE.

"FIELD OF USE" means all uses.

"FIRST COMMERCIAL SALE" means the first SALE through a bona fide arms length transaction of any LICENSED PRODUCT or first commercial use of any LICENSED PROCESS by LICENSEE or a SUBLICENSEE, excluding the SALE of a LICENSED PRODUCT or use of a LICENSED PROCESS for use in trials, as a sample or that is of temporary availability.

"LICENSED PROCESS(ES)" means any process or method that, but for this Agreement, would comprises an infringement of (including contributory or inducement) a VALID CLAIM in the country in which any such process or method is used or performed, or (b) employs a LICENSED PRODUCT.

"LICENSED PRODUCT(S)" means any product that: (a) but for this Agreement, comprises an infringement of (including contributory or inducement) a VALID CLAIM in the country in which any such product or product part is made, used, imported, offered for SALE or sold; or (b) is manufactured by using a LICENSED PROCESS or is employed to practice a LICENSED PROCESS.

"MICHIGAN," as used in Articles 9 and 10, shall include its Regents, officers, employees, students, and agents.

"NET SALES" means the amount billed or invoiced, and if any amount is not billed or invoiced, the amounts actually received, on Sales, however characterized, by LICENSEE and/or SUBLICENSEES of LICENSED PRODUCTS and uses of LICENSED PROCESSES, less the following deductions (but only to the extent such deductions are otherwise included in NET SALES and are not obtained in view of other consideration received by LICENSEE):

(a) cash discounts actually granted to customers in such invoices for SALE of LICENSED PRODUCTS, but only in amounts customary in the trade;

(b) sales taxes, tariff duties and/or use taxes separately stated in such bills or invoices with reference to particular SALES and actually paid by LICENSEE or SUBLICENSEE to a governmental unit;

(c) actual freight expenses between LICENSEE or SUBLICENSEE and customers, to the extent such expenses are not charged to or reimbursed by customers; or

(d) amounts actually refunded or credited on returns.

Where LICENSEE or SUBLICENSEE receives any consideration other than cash for such transactions, fair market cash value for such consideration, to be agreed upon by the parties hereto, shall be included in NET SALES. Where a product or activity is a LICENSED PRODUCT or LICENSED PROCESS hereunder due to contributory infringement or inducement of infringement, NET SALES shall include SALES of the product or process that constitutes a direct infringement of the PATENT RIGHTS. NET SALES shall not include LICENSED PRODUCT used for pre-clinical or clinical trials, post-marketing trials, samples and indigent patient programs or any other uses of LICENSED PRODUCT not ordinarily included as part of NET SALES for royalty determination purposes.

A sale or transfer to an AFFILIATE or SUBLICENSEE for re-sale by such AFFILIATE or SUBLICENSEE shall not be considered a sale for the purpose of this provision but the resale by such AFFILIATE or SUBLICENSEE shall be a sale for such purposes. Any amounts received LICENSEE, an AFFILIATE or SUBLICENSEE in exchange for LICENSED PRODUCTS or LICENSED PROCESSES transferred or provided to any person or entity for use in testing, clinical trials, or as marketing samples to develop or promote the LICENSED PRODUCTS are not included in the definition of NET SALES.

“PATENT RIGHTS” means MICHIGAN’S legal rights under the patent laws of the United States or relevant foreign countries for all of the following:

(e) the following United States and foreign patent(s) and/or patent application(s), and divisionals, continuations, continuations-in-part (only to the extent that such claims are fully supported under U.S. patent laws by another patent or application in the PATENT RIGHTS), and foreign counterparts of the same:

[**]; and

[**]; and

(f) any renewals, reexaminations, substitutes, supplementary protection certificates and extensions of these patents, and any corresponding foreign counterparts of the same.

“ROYALTY PERIOD(S)” means the six-month periods ending on the last days of June and December each year.

“SALE” means sale, rental, or lease, however characterized, and SOLD means the past tense of SALE.

“SUBLICENSEE(S)” means any person or entity in writing sublicensed, or granted an option for a sublicense, by LICENSEE under this Agreement.

“TERRITORY” means all of the countries of the world.

“VALID CLAIM” means (i) a claim in an issued and unexpired patent included in the PATENT RIGHTS that: (a) has not been held unenforceable, unpatentable or invalid by a decision of a court or other governmental agency of competent jurisdiction, and not subject to appeal, (b) has not been admitted to be invalid or unenforceable through reissue or disclaimer or otherwise, (c) has not been lost through an interference, reexamination, or reissue proceeding; or (ii) a pending claim of a pending patent application included in the PATENT RIGHTS that has not been abandoned or finally rejected without the possibility of appeal or refiling and that has been pending for less than seven years from its priority date.

ARTICLE 2 – GRANT OF LICENSE

2.1MICHIGAN hereby grants to LICENSEE an exclusive license under the PATENT RIGHTS, with the right to grant sublicenses, both subject to the terms and conditions of this Agreement, in the FIELD OF USE and the TERRITORY to make, have made, import, use, market, offer for sale and sell LICENSED PRODUCTS and to practice LICENSED PROCESSES.

2.2Without limiting any other rights it may have, MICHIGAN specifically reserves the right for it and its affiliates to practice and have practiced the PATENT RIGHTS for non-commercial research, public service, internal (including clinical) and/or educational purposes, and the right to grant the same limited non-commercial rights to other non-profit research institutions. For avoidance of doubt, sponsored research on behalf of for-profit entities shall be deemed to be commercial.

2.3This Agreement shall extend until expiration of the last to expire of the PATENT RIGHTS, unless sooner terminated as provided in another specific provision of this Agreement.

2.4LICENSEE agrees that LICENSED PRODUCTS used, leased or sold in the United States shall be manufactured substantially in the United States.

2.5The licenses granted in this Agreement are subject to any rights required to be granted under prior research or sponsorship agreements, or retained by the U.S. government, for example in accordance with Chapter 18 of Title 35 of U.S.C. 200-212 and the regulations thereunder (37 CFR Part 401), when applicable. LICENSEE agrees to comply in all respects, and shall provide MICHIGAN with all reasonably requested information and cooperation for MICHIGAN to comply with applicable provisions of the same and any requirements of any agreements between MICHIGAN and any agency of the U.S. government that provided funding for the subject matter covered by the PATENT RIGHTS.

ARTICLE 3 – CONSIDERATION

3.1LICENSEE shall pay the following to MICHIGAN:

(a) License Issue Fee. A License Issue Fee equal to [**], due within fourteen (14) days from the complete execution of this Agreement.

(b) Annual Maintenance Fee. An Annual Maintenance Fee equal to [**], due within fourteen (14) days of the second (2nd) anniversary of the EFFECTIVE DATE, and within fourteen (14) days of each subsequent anniversary of the EFFECTIVE DATE. The Annual Maintenance Fee shall not be due in any calendar year that the LICENSEE pays Minimum Annual Royalties as described in Section 3.1 (e) or Running Royalties as described in Section 3.1 (b) in an amount equal to or greater than Minimum Annual Royalties.

(c) Running Royalties. Running Royalties equal to [**] of NET SALES. If LICENSEE makes any SALES to any AFFILIATE at a price less than the regular price charged to other parties, the Running Royalties payable to MICHIGAN shall be computed on the basis of the regular price charged to other parties.

(d) Sublicensing Fees. Sublicensing Fees equal to a percentage of any revenue not based on product sales that LICENSEE or SUBLICENSEES (or a designee) is due from or receives from SUBLICENSEES or assignees in consideration for rights under or relating to the PATENT RIGHTS (e.g., license issue fees, maintenance or annual minimum fees, milestone payments, other royalties), but excluding, (a) amounts received for the purchase of securities; (b) payments of loans or other debt obligations, (c) payments made as a reimbursement of costs incurred, such as for patent prosecution costs, (d) amounts received to cover research and development activities related to actual or potential LICENSED PRODUCTS or LICENSED PROCESSES after the effective date of the sublicense agreement, and (e) amounts attributable to intellectual property other than the LICENSED PATENTS, as follows:

1) [**] if the sublicense is entered into on or after the EFFECTIVE DATE and before [**];

2) [**] if the sublicense is entered into at or after [**] and before [**]; and

3) [**] if [**] into at or after [**].

(e) Back Patent Costs. Within fourteen (14) days from complete execution of this Agreement, LICENSEE shall pay MICHIGAN all of the Back Patent Costs which are [**] as of March 10, 2016.

(f) Minimum Annual Royalties. Beginning with the FIRST COMMERCIAL SALE, Minimum Annual Royalties are due for each calendar year within sixty (60) days following the end thereof. Minimum Annual Royalties shall be credited against Running Royalties (and, if paid first, Running Royalties shall be credited against Minimum Annual Royalties) due on NET SALES made during the calendar year for which the Minimum Annual Royalties apply. Minimum Annual Royalties paid in excess of Running Royalties shall not be creditable to amounts due for future years. The Minimum Annual Royalty is [**].

(g) Milestone Payments for LICENSED PRODUCT as follows (for the avoidance of doubt, none of these Milestone Payments shall be required to be paid more than once):

(a) [**]

(b) [**]

(c) [**]

(d) [**]

Milestone payments are non refundable and non-creditable against royalties.

(h) Change of Control. Within thirty (30) days after any CHANGE OF CONTROL, LICENSEE shall pay to MICHIGAN a two million dollar ($2,000,000.00) fee ("CHANGE OF CONTROL FEE"). As used herein, "CHANGE OF CONTROL" shall mean: (a) any consolidation, merger, combination, reorganization or other transaction in which LICENSEE is not the surviving entity, irrespective of whether LICENSEE is maintained as an AFFILIATE or subsidiary of the new controlling entity, or dissolved and absorbed into the new controlling entity; or (b) any transaction or series of related transactions in which the shares of stock or other equity interests of LICENSEE constituting in excess of fifty percent (50%) of the voting power of LICENSEE are exchanged for or converted into other stock or securities, cash and/or any other property; or (c) a sale or other disposition of all or substantially all of the assets of the LICENSEE.

3.2LICENSEE is not obligated to pay multiple royalties if any LICENSED PRODUCT or LICENSED PROCESS is covered by more than one VALID CLAIM or the same LICENSED PRODUCT is covered by VALID CLAIMS in two or more countries. If LICENSEE or its AFFILIATES or SUBLICENSEE enters into a license agreement with a third party that LICENSEE reasonably determines is necessary and procured for the commercialization of a LICENSED PRODUCT or LICENSED PROCESS and according to such license agreement a royalty must be paid to the third party by LICENSEE or its AFFILIATE or SUBLICENSEE based upon commercialization of a LICENSED PRODUCT or LICENSED PROCESS, then the royalty otherwise payable to MICHIGAN pursuant to Section 3.1 may be reduced by [**] of the applicable third party royalty; provided that, in no instance shall the royalty payable to MICHIGAN by LICENSEE ever be reduced below [**] of the otherwise applicable royalty.

3.3Royalty payments shall be made to "The Regents of the University of Michigan" in United States dollars. Payments drawn directly on a U.S. bank may be made by either check to the address in Article 12 or by wire transfer. Any payment drawn on a foreign bank or foreign branch of a U.S. bank shall be made only by wire transfer. Wire transfers shall be made in accordance with the following or any other instructions as may be specified by MICHIGAN. In computing royalties, LICENSEE shall convert any revenues it receives in foreign currency into its equivalent in United States dollars at the most recent exchange rate published in the Wall Street Journal on the last business day of the ROYALTY PERIOD during which such payments are received by LICENSEE, or at such other exchange rate as the parties may agree to in writing.

3.4Royalty payments shall be made on a semi-annual basis with submission of the reports required by Article 4. All amounts due under this Agreement, including amounts due for the payment of patent expenses, shall, if overdue, be subject to a charge of interest [**] until payment, [**] above the prime rate in effect at the JP Morgan Chase Bank, N.A. or its successor bank on the due date (or at the highest allowed rate if a lower rate is required by law) or [**], whichever is greater. The payment of such interest shall not foreclose MICHIGAN from exercising any other rights it may have resulting from any late payment. LICENSEE shall reimburse MICHIGAN for the costs, including reasonable attorney fees, for expenses paid in order to collect any amounts overdue more than 120 days.

3.5All payments made under this Agreement are and shall be non-refundable. MICHIGAN shall have no obligation whatsoever to pay, return, credit, or refund any amounts paid hereunder, except as may be specifically provided herein. By way of example only, notwithstanding the deductions permitted to NET SALES, MICHIGAN shall have no obligation to pay any amounts to LICENSEE even if such deductions should result in a negative amount for NET SALES in any given ROYALTY PERIOD.

3.6The payments required to be paid by LICENSEE to MICHIGAN pursuant to this agreement may be paid with deduction for taxes withheld under the LICENSEE's or, if applicable, its SUBLICENSEE's applicable domestic law. LICENSEE will reasonably assist MICHIGAN to obtain full benefit of any applicable tax treaty to reduce the amount of such withheld taxes. MICHIGAN shall be responsible for the payment of all taxes, duties, levies, and other charges imposed by any taxing authority with respect to the royalties payable to MICHIGAN under this agreement. LICENSEE may withhold or deduct any portion of the payments due to MICHIGAN required under applicable law or regulation of any government entity or authority. LICENSEE shall cooperate reasonably with MICHIGAN in the event MICHIGAN elects to assert, at its own expense, any exemption from any such tax or deduction.

ARTICLE 4 – REPORTS

4.1Until the FIRST COMMERCIAL SALE, by July 31 of each year LICENSEE shall provide to MICHIGAN a written annual report that includes reports on progress since the prior annual report and general future plans regarding: research and development, regulatory approvals, manufacturing, sublicensing, marketing and SALES. Further, LICENSEE shall specifically report to MICHIGAN the FIRST COMMERCIAL SALE within sixty (60) days thereafter, and provide a brief description of the products or services subject of the FIRST COMMERCIAL SALE, and terms thereof.

4.2After the FIRST COMMERCIAL SALE, LICENSEE shall provide semi-annual reports to MICHIGAN. Specifically, as of the end of each ROYALTY PERIOD (and delivered within sixty (60) days after such ROYALTY PERIOD closes, including the close of the ROYALTY PERIOD immediately following any termination of this Agreement), LICENSEE shall report to MICHIGAN for the applicable ROYALTY PERIOD:

(a) number of LICENSED PRODUCTS sold, leased, or distributed, however characterized, by LICENSEE and each SUBLICENSEE.

(b) NET SALES, excluding the deductions provided therefor, of LICENSED PRODUCTS SOLD by LICENSEE and all SUBLICENSEES.

(c) a description and accounting for all LICENSED PROCESSES SOLD, by LICENSEE and all SUBLICENSEES included in NET SALES, excluding the deductions therefor.

(d) deductions applicable as provided in the definition for NET SALES above, and an explanation of the rationale(s) therefor.

(e) Sublicense Fees due on payments from SUBLICENSEES under Paragraph 3.1 above, including supporting figures.

(f) foreign currency conversion rate and calculations (if applicable) and total royalties due.

(g) each milestone under Article 3 or Article 5 having a deadline during the ROYALTY PERIOD, and a specific identification of whether or not it was achieved.

(h) for each sublicense or amendment thereto completed in the particular ROYALTY PERIOD (including agreements under which LICENSEE will have LICENSED PRODUCTS made by a third party): names, addresses, and U.S.P.T.O. Entity Status (as discussed in Paragraph 4.5) of such SUBLICENSEE; the date of each agreement and amendment; the territory of the sublicense; the scope of the sublicense; and the nature, timing and amounts of all fees, royalties to be paid thereunder.

(i) progress on research and development, regulatory approvals, manufacturing, sublicensing, marketing and SALES, and general plans for the future.

(j) the date of first SALE of LICENSED PRODUCTS (or results of LICENSED PROCESSES) in each country and the circumstances thereof.

LICENSEE shall include the amount of all payments due, and the various calculations used to arrive at those amounts, including the quantity, description (nomenclature and type designation as described in Paragraph 4.3 below), country of manufacture and country of SALE or use of LICENSED PRODUCTS and LICENSED PROCESSES.

If no payment is due, LICENSEE shall so report to MICHIGAN that no payment is due. Failure to provide reports as required under this Article 4 shall be a material breach of this Agreement. LICENSEE agrees to reasonably cooperate with MICHIGAN regarding any questions it may have relating to compliance with this Agreement, for example to discuss the information in reports.

4.3LICENSEE shall promptly establish and consistently employ a system of specific nomenclature and type designations for LICENSED PRODUCTS and LICENSED PROCESSES to permit identification and segregation of various types where necessary, and shall require the same of SUBLICENSEES.

4.4LICENSEE shall keep, and shall require SUBLICENSEES to keep, true and accurate records containing data reasonably required for the computation and verification of payments due under this Agreement. LICENSEE shall and it shall require all SUBLICENSEES and those making LICENSED PRODUCTS to: (a) open such records for inspection upon reasonable notice during business hours, and no more than once per year, at MICHIGAN's sole expense, by either MICHIGAN auditor(s) or an independent certified accountant selected by MICHIGAN and reasonably acceptable to LICENSEE, for the purpose of verifying the amount of payments due, and shall provide information to MICHIGAN to facilitate such inspection; and (b) retain such records for six (6) years from date of origination.

The terms of this Article shall survive any termination of this Agreement. MICHIGAN is responsible for all expenses of such inspection, except that if any inspection reveals an underpayment greater than [**] of royalties due MICHIGAN, then LICENSEE shall pay all expenses of that inspection and the amount of the underpayment and interest to MICHIGAN within thirty (30) days of written notice thereof. LICENSEE shall also reimburse MICHIGAN for reasonable expenses required to collect the amount underpaid.

4.5So that MICHIGAN may pay the proper U.S. Patent and Trademark Office fees relating to the PATENT RIGHTS, if LICENSEE, any AFFILIATE, or any SUBLICENSEE (including optionees) does not quality as a "Small Entity" under U.S. patent laws, LICENSEE shall notify MICHIGAN immediately. The parties understand that the changes to LICENSEE's, AFFILIATE's, SUBLICENSEE's, or optionees' businesses that might affect entity status include: acquisitions, mergers, hiring of a total of more than 500 total employees, sublicense agreements, and sublicense options.

ARTICLE 5 – DILIGENCE

5.1LICENSEE shall use commercially reasonable efforts to bring one or more LICENSED PRODUCTS to market, and/or one or more LICENSED PROCESSES to commercial use, through a diligent program for utilizing the PATENT RIGHTS and to continue diligent marketing efforts throughout the life of this Agreement, in each case consistent with prudent business practices and judgment. LICENSEE has the responsibility to do all that is legally required and commercially reasonable to obtain and retain any governmental approvals to manufacture and/or sell LICENSED PRODUCTS and/or use LICENSED PROCESSES for all relevant activities of LICENSEE and SUBLICENSEES. If the commercialization of multiple LICENSED PRODUCTS or LICENSED PROCESSES is commercially reasonable, then the requirement of this paragraph shall apply to all such LICENSED PRODUCTS and/or LICENSED PROCESSES. Further, for the sake of clarity, LICENSEE must make commercially reasonable amounts or levels of LICENSED PRODUCTS and/or LICENSED PROCESSES available.

5.2Without limiting Paragraph 5.1, LICENSEE agrees to reach the following commercialization and research and development milestones for the LICENSED PRODUCTS and LICENSED PROCESSES (together the "MILESTONES") by the following dates:

1) [**]

2) [**]

3) [**]

4) [**]

For the purposes of this Agreement, initiation of a clinical trial shall mean that date upon which the first patient or subject is treated with a LICENSED PRODUCT under a protocol approved by an appropriate drug regulatory agency with a therapeutic agent or process that has been manufactured according to Good Manufacturing Practices (GMP) guidelines provided by the relevant regulatory agency.

5.3LICENSEE must achieve each MILESTONE on or before the deadline dates indicated above. LICENSEE shall notify MICHIGAN within thirty (30) days after each such deadline as to whether or not such MILESTONE was met. If LICENSEE fails to meet any MILESTONE under this Article by the date of any MILESTONE deadline, LICENSEE will be deemed to be in material breach of this Agreement, and MICHIGAN may terminate the Agreement effective on ninety (90) days' written notice, unless LICENSEE achieves the MILESTONE within this ninety (90) day period.

ARTICLE 6 – SUBLICENSING

6.1LICENSEE shall notify MICHIGAN in writing of every sublicense agreement and each amendment thereto within thirty (30) days after their execution, and indicate the name of the SUBLICENSEE, the territory of the sublicense, the scope of the sublicense, and the nature, timing and amounts of all fees and royalties to be paid thereunder, and whether or not the SUBLICENSEE has greater or fewer than 500 employees. Upon request, LICENSEE shall provide MICHIGAN with a copy of sublicense agreements.

6.2LICENSEE shall not receive from SUBLICENSEES anything of value other than cash payments in consideration for any sublicense under this Agreement, without the express prior written permission of MICHIGAN.

6.3Each sublicense granted by LICENSEE under this Agreement shall provide for its termination upon termination of this Agreement. Each sublicense shall terminate upon termination of this Agreement unless LICENSEE has previously assigned its rights under the sublicense to MICHIGAN and MICHIGAN has agreed at its sole discretion in writing to such assignment.

6.4LICENSEE shall require that all sublicenses: (a) be consistent with the terms and conditions of this Agreement; (b) contain the SUBLICENSEE'S acknowledgment of the disclaimer of warranty and limitation on MICHIGAN's liability, as provided by Article 9 below; and (c) contain provisions under which the SUBLICENSEE accepts duties at least equivalent to those accepted by the LICENSEE in the following Paragraphs: 4.4 (duty to keep records), 10.1 (duty to defend, hold harmless, and indemnify MICHIGAN), 10.3 (duty to maintain insurance), 13.4 (duty to properly mark LICENSED PRODUCTS with patent notices), and 13.6 (duty to restrict the use of MICHIGAN's name).

ARTICLE 7 – PATENT APPLICATIONS AND MAINTENANCE

7.1MICHIGAN shall have the right to control all aspects maintaining the patents that form the basis for the PATENT RIGHTS, including administrative reexaminations and reviews, disputes (including litigation) regarding inventorship and derivation, and interferences. LICENSEE shall fully cooperate with MICHIGAN in activities relating to the PATENT RIGHTS, including said activities. Upon MICHIGAN's request, to the extent permitted by law, LICENSEE shall cooperate with MICHIGAN in applying for patent term extension for any and all patents included in the PATENT RIGHTS. LICENSEE and MICHIGAN will mutually agree on the jurisdictions in which to seek such patent protection.

7.2MICHIGAN shall notify LICENSEE of all information received by MICHIGAN relating to maintenance of the PATENT RIGHTS, and shall make reasonable efforts to allow LICENSEE to review, comment, and advise upon such information. LICENSEE shall hold such information confidential and to use the information only for the purpose of advancing MICHIGAN's PATENT RIGHTS.

7.3LICENSEE shall reimburse MICHIGAN for all fees and costs relating to the activities described in this Article; provided, however, that LICENSEE shall not be responsible to reimburse such fees and cost relating to any country in which LICENSEE has not agreed to seek patent protection. Such reimbursement shall be made within thirty (30) days of receipt of MICHIGAN's invoice and shall be subject to the interest and other requirements specified in Article 4 above.

ARTICLE 8 – ENFORCEMENT

8.1Each party shall promptly advise the other in writing of any known acts of potential infringement of the PATENT RIGHTS by another party. LICENSEE has the first option to police the PATENT RIGHTS against infringement by other parties within the TERRITORY and the FIELD OF USE, including those prior to the EFFECTIVE DATE. LICENSEE shall not file any suit without (a) first performing a thorough, diligent investigation of the merits of such suit, including with respect to the validity and enforceability of the PATENT RIGHTS; (b) there being reasonable legal and economic bases for doing so; and (c) notifying MICHIGAN twenty days before any such filing. This right to police includes filing, prosecuting, and settling all infringement actions at its expense, except that LICENSEE shall make any such settlement only with the advice and consent of MICHIGAN. LICENSEE has the right to file suit using counsel of its choosing, subject to MICHIGAN's approval, which shall not be unreasonably withheld or delayed. LICENSEE may grant to third parties the right to enforce hereunder, but only with the express written permission of MICHIGAN.

8.2If LICENSEE has complied with Paragraph 8.1, MICHIGAN shall provide reasonable assistance to LICENSEE with respect to such actions, but only if LICENSEE promptly reimburses MICHIGAN for out-of-pocket expenses incurred in connection with any such assistance rendered at LICENSEE's request or reasonably required by MICHIGAN, including but not limited to expenses incurred in complying with discovery duties. MICHIGAN retains the right to participate, with counsel of its own choosing and at its own expense, in any action under this Article. LICENSEE shall defend, indemnify and hold harmless MICHIGAN

with respect to any claims asserted by an alleged infringer reasonably related to the enforcement of the PATENT RIGHTS under this Article, including but not limited to antitrust counterclaims and claims for recovery of attorney fees.

8.3MICHIGAN and its employees have a vital interest in lawsuits relating to the validity and enforceability of the PATENT RIGHTS. If a third party files a suit, including as a counterclaim, alleging that any of the PATENT RIGHTS is invalid or unenforceable, then the parties shall jointly control the defense of such claim. Each party shall consult with the other with respect to the defense of such claim, and shall reasonably consider the other party's input. In furtherance of such joint control, at the onset of such claim and as reasonable during the pendency of any such claim, the parties shall meet and confer in good faith to set a plan for handling the defense thereof. The parties expect that in general (a) LICENSEE will have the right to lead daily activities, including but not limited to discovery, relating to the defense and (b) the parties would make joint filings. Notwithstanding, in the event that the parties cannot agree on how to proceed with respect to such claim, MICHIGAN shall have the right to control the defense thereof on either a temporary or permanent basis. LICENSEE shall be responsible for the reasonable costs and fees associated with the activities under this Paragraph 8.3. The parties shall consider reasonable controls on costs and fees as part of an aforementioned meet and confer with respect to the handling of the defense. Notwithstanding, if a third party asserts jurisdiction for any such action solely as the result of acts of MICHIGAN, then MICHIGAN shall be responsible for such reasonable costs and fees. LICENSEE shall have the right to offset [**] of any fees it is responsible for under this Section 8.3 from payments it is required to make to MICHIGAN under Section 3.1 hereof.

8.4If LICENSEE or MICHIGAN recovers damages in patent litigation or settlement thereof, the award shall be applied first to satisfy LICENSEE's (to the extent not offset in accordance with Section 8.3) and MICHIGAN's reasonable expenses and legal fees for the litigation. The remaining balance shall be divided equally between LICENSEE and MICHIGAN. This provision shall control the division of revenues where a sublicense, covenant not to sue, or assignment of rights is granted as part of a settlement of such lawsuit (including prospective rights).

ARTICLE 9 – NO WARRANTIES; LIMITATION ON MICHIGAN'S LIABILITY

9.1MICHIGAN makes no representations or warranties that any claim within the PATENT RIGHTS is or will be held valid, patentable, or enforceable, or that the manufacture, importation, use, offer for SALE, SALE or other distribution of any LICENSED PRODUCTS or LICENSED PROCESSES will not infringe upon any patent or other rights.

9.2**MICHIGAN** MAKES NO REPRESENTATIONS, EXTENDS NO WARRANTIES OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING BUT NOT LIMITED TO THE IMPLIED WARRANTIES OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE, AND ASSUMES NO RESPONSIBILITIES WHATEVER WITH RESPECT TO DESIGN, DEVELOPMENT, MANUFACTURE, USE, **SALE** OR OTHER DISPOSITION BY **LICENSEE** OR **SUBLICENSEES** OF **LICENSED PRODUCTS** OR **LICENSED PROCESSES. LICENSEE** AND **SUBLICENSEES** ASSUME THE ENTIRE

RISK AS TO PERFORMANCE OF **LICENSED PRODUCTS** AND **LICENSED PROCESSES.**

9.3In no event shall MICHIGAN be responsible or liable for any direct, indirect, special, incidental, or consequential damages or lost profits or other economic loss or damage with respect to LICENSED PRODUCTS LICENSED PROCESSES, or the PATENT RIGHTS to LICENSEE, SUBLICENSEES or any other individual or entity regardless of legal or equitable theory. The above limitations on liability apply even though MICHIGAN may have been advised of the possibility of such damage.

9.4LICENSEE shall not make any statements, representations or warranties whatsoever to any person or entity, or accept any liabilities or responsibilities whatsoever from any person or entity that are inconsistent with any disclaimer or limitation included in this Article 9.

ARTICLE 10 – INDEMNITY; INSURANCE

10.1LICENSEE shall defend, indemnify and hold harmless and shall require SUBLICENSEES to defend, indemnify and hold harmless MICHIGAN for and against any and all claims, demands, damages, losses, and expenses of any nature (including attorneys' fees and other litigation expenses), resulting from, but not limited to, death, personal injury, illness, property damage, economic loss or products liability, including errors and omissions, arising from or in connection with, any of the following: (1) Any manufacture, use, SALE or other disposition by LICENSEE, SUBLICENSEES or transferees of LICENSED PRODUCTS or LICENSED PROCESSES; (2) The use by any person of LICENSED PRODUCTS made, used, sold or otherwise distributed by LICENSEE or SUBLICENSEES; and (3) The use or practice by LICENSEE or SUBLICENSEES of any invention or computer software related to the PATENT RIGHTS.

10.2MICHIGAN is entitled to participate at its option and expense through counsel of its own selection, and may join in any legal actions related to any such claims, demands, damages, losses and expenses under Paragraph 10.1 above. LICENSEE shall not settle any such legal action with an admission of liability of MICHIGAN without MICHIGAN's written approval.

10.3Prior to any distribution or commercial use of any LICENSED PRODUCT or use of any LICENSED PROCESS by LICENSEE, LICENSEE shall purchase and maintain in effect commercial general liability insurance, product liability insurance, and errors and omissions insurance which shall protect LICENSEE and MICHIGAN with respect to the events covered by Paragraph 10.1, and LICENSEE shall require the same of any SUBLICENSEE. Each such insurance policy must provide reasonable coverage for all claims with respect to any LICENSED PROCESS used and any LICENSED PRODUCTS manufactured, used, sold, licensed or otherwise distributed by LICENSEE — or, in the case of a SUBLICENSEE's policy, by said SUBLICENSEE — and must specify MICHIGAN as an additional insured. LICENSEE shall furnish certificate(s) of such insurance to MICHIGAN, upon request.

10.4In no event shall either party hereunder be liable to the other for any special, indirect, or consequential damages of any kind whatsoever resulting from any breach or default of this Agreement.

ARTICLE 11 – TERM AND TERMINATION

11.1If LICENSEE ceases to operate its business, or if it files a petition in bankruptcy, has an involuntary petition in bankruptcy filed against LICENSEE that is not dismissed within sixty days after the filing thereof, make a general assignment for the benefit of creditors or liquidates or dissolves, this Agreement shall immediately terminate upon MICHIGAN's attempt to deliver a termination notice to the address for notices provided herein. If LICENSEE makes or attempts to make an assignment for the benefit of creditors, or if proceedings in voluntary or involuntary bankruptcy or insolvency are instituted on behalf of or against LICENSEE, or if a receiver or trustee is appointed for the property of LICENSEE, this Agreement shall automatically terminate. LICENSEE shall notify MICHIGAN of any such event mentioned in this Paragraph as soon as reasonably practicable, and in any event within five days after any such event.

11.2If LICENSEE fails to make any payment due to MICHIGAN, other than amounts subject to a bona fide dispute, upon thirty (30) days' written notice by MICHIGAN, this Agreement shall automatically terminate unless MICHIGAN specifically extends such date in writing or such unpaid amount is paid to MICHIGAN within such thirty (30) day period. Such termination shall not foreclose MICHIGAN from collection of any amounts remaining unpaid or seeking other legal relief.

11.3Upon any material breach or default of this Agreement by LICENSEE (other than as specifically provided herein, the terms of which shall take precedence over the handling of any other material breach or default under this Paragraph), MICHIGAN has the right to terminate this Agreement effective on ninety (90) days' written notice to LICENSEE. Such termination shall become automatically effective upon expiration of the thirty-day period unless LICENSEE cures the material breach or default before the period expires.

11.4LICENSEE has the right to terminate this Agreement at any time on sixty (60) days' written notice to MICHIGAN if LICENSEE prior to the termination date:

(a) pays all amounts due MICHIGAN through the effective date of the termination;

(b) submits a final report of the type described in Paragraph 4.2;

(c) returns any patent documentation (including that exchanged under Article 7) and any other confidential or trade-secret materials provided to LICENSEE by MICHIGAN in connection with this Agreement, or, with prior approval by MICHIGAN, destroys such materials, and certifies in writing that such materials have all been returned or destroyed; and

(d) suspends its manufacture, use and SALE of the LICENSED PROCESS(ES) and LICENSED PRODUCT(S).

11.5Upon any termination of this Agreement, and except as provided herein to the contrary, all rights and obligations of the parties hereunder shall cease, except any previously accrued rights and obligations and further as follows: (a) obligations to pay royalties and other sums, or to transfer equity or other consideration, accruing hereunder up to the day of such termination, whether or not this Agreement provides for a number of days before which actual payment is due and such date is after the day of termination and whether or not a required funding event or other stock transfer trigger has yet been met; (b) MICHIGAN's rights to inspect books and records as described in Article 4, and LICENSEE's obligations to keep such records for the required time; (c) any cause of action or claim of LICENSEE or MICHIGAN accrued or to accrue because of any breach or default by the other party hereunder; (d) the provisions of Articles 1, 9, 10, and 13; and (e) all other terms, provisions, representations, rights and obligations contained in this Agreement that by their sense and context are intended to survive until performance thereof by either or both parties.

Termination by either party hereunder shall not alter or affect any other rights or relief that either party may be entitled to under law.

11.6Upon termination of this Agreement, if LICENSEE has filed patent applications or obtained patents to any modification or improvement to LICENSED PRODUCTS or LICENSED PROCESSES within the scope of the PATENT RIGHTS, LICENSEE agrees upon request to enter into good faith negotiations with MICHIGAN or MICHIGAN's future licensee(s) for the purpose of granting licensing rights to said modifications or improvements in a timely fashion and under commercially reasonable terms.

11.7If LICENSEE or a SUBLICENSEE, or any affiliate thereof, asserts the invalidity or unenforceability of any claim included in the PATENT RIGHTS, including by way of litigation or administrative proceedings, either directly or through any other party, then MICHIGAN shall have the right to immediately terminate this Agreement upon written notice to LICENSEE.

ARTICLE 12 – NOTICES

12.1Any notice, request, or report required or permitted to be given or made under this Agreement by either party is effective when mailed if sent by recognized overnight carrier, certified or registered mail, or electronic mail followed by confirmation by U.S. mail, to the address set forth below or such other address as such party specifies by written notice given in conformity herewith. Any notice, request, or report not so given is not effective until actually received by the other party.

To MICHIGAN:
Office of Technology Transfer
University of Michigan
1600 Huron Parkway, 2nd Floor
Ann Arbor, MI 48109-2590

To LICENSEE:
Solid GT, LLC
One Broadway
Cambridge, MA 02142
Attention: Ilan Ganot, CEO

ARTICLE 13 – MISCELLANEOUS PROVISIONS

13.1This Agreement shall be governed by and construed under the laws of the state of Michigan without regard for principles of choice of law, except that questions affecting the construction and effect of any patent shall be determined by the law of the country in which the patent was granted. Any claims, demands, or actions asserted against MICHIGAN, its Regents, fellows, officers, employees or agents shall only be brought in the Michigan Court of Claims. LICENSEE, its successors, and assigns consent to the jurisdiction of a court with applicable subject matter jurisdiction sitting in the state of Michigan with respect to any claims arising under this agreement or the relationship between the parties.

13.2MICHIGAN and LICENSEE agree that this Agreement sets forth their entire understanding concerning the subject matter of this Agreement. The parties may amend this Agreement from time to time, such as to add new rights, but no modification will be effective unless both MICHIGAN and LICENSEE agree to it in writing.

13.3If a court of competent jurisdiction finds any term of this Agreement invalid, illegal or unenforceable, that term will be curtailed, limited or deleted, but only to the extent necessary to remove the invalidity, illegality or unenforceability, and without in any way affecting or impairing the remaining terms.

13.4LICENSEE agrees to mark the LICENSED PRODUCTS sold in the United States with all applicable United States patent numbers as necessary to meet the requirements of 35 U.S.C. 287 so that the full benefits of patent enforcement may be realized. All LICENSED PRODUCTS shipped to or sold in other countries shall be marked to comply with the patent laws and practices of the countries of manufacture, use and SALE.

13.5No waiver by either party of any breach of this Agreement, no matter how long continuing or how often repeated, is a waiver of any subsequent breach thereof, nor is any delay or omission on the part of either party to exercise or insist on any right, power, or privilege hereunder a waiver of such right, power or privilege. In no event shall any waiver be deemed valid unless it is in writing and signed by an authorized representative of each party.

13.6LICENSEE shall, and shall require its affiliates to, refrain from using and to require SUBLICENSEES to refrain from using the name of MICHIGAN or its employees in publicity or advertising without the prior written approval of MICHIGAN. Reports in scientific literature and presentations of joint research and development work are not publicity. Notwithstanding this provision, without prior written approval of MICHIGAN, LICENSEE and SUBLICENSEES may state publicly that LICENSED PRODUCTS and PROCESSES were developed by LICENSEE based upon an invention(s) developed at the University of Michigan and/or that the PATENT RIGHTS were licensed from the University of Michigan.

13.7LICENSEE agrees to comply with all applicable laws and regulations, including but not limited to all United States laws and regulations controlling the export of commodities and technical data. LICENSEE shall be solely responsible for any violation of such laws and regulations involving LICENSEE or its SUBLICENSEES, and to defend, indemnify and hold harmless MICHIGAN if any legal action of any nature results from any such violation.

13.8The relationship between the parties is that of independent contractor and contractee. Neither party is an agent of the other in connection with the exercise of any rights hereunder, and neither has any right or authority to assume or create any obligation or responsibility on behalf of the other.

13.9LICENSEE may not assign this Agreement without the prior written consent of MICHIGAN and shall not pledge any of the license rights granted in this Agreement as security for any creditor. Any attempted pledge of any of the rights under this Agreement or assignment of this Agreement without the prior consent of MICHIGAN will be void from the beginning. No assignment by LICENSEE will be effective until the intended assignee agrees in writing to accept all of the terms and conditions of this Agreement, and such writing is provided to MICHIGAN. Notwithstanding the foregoing, LICENSEE may, without MICHIGAN's consent, assign its rights under this Agreement to a purchaser of all or substantially all of LICENSEE's business relating to the subject matter of this Agreement, so long as (a) LICENSEE is not in breach of this Agreement and (b) such assignee provides a statement in writing to MICHIGAN that it agrees to accept all the terms and conditions of this Agreement (including obligations existing as of the time of such assignment) in the place of LICENSEE.

13.10If the registration, recordation, or reporting to a national or supranational agency of this Agreement, its terms, or assignment thereof is or becomes required or advisable (e.g., as a prerequisite to enforceability of the Agreement in such nation), LICENSEE shall, at its expense, promptly undertake such action. LICENSEE shall provide prompt notice thereof to MICHIGAN along with copies of relevant documentation.

IN WITNESS WHEREOF, the parties hereto have executed this Agreement in duplicate originals by their duly authorized officers or representatives.

FOR LICENSEE: SOLID GT, LLC	FOR THE REGENTS OF THE UNIVERSITY OF MICHIGAN
By /s/ Ilan Ganot (authorized representative)	By /s/ Ruth L. Rasor Ruth L. Rasor Managing Director of Licensing UM Technology Transfer
Printed Name Ilan Ganot Title CEO Date 3/11/16	Date 14 March 2016

Exhibit 10.54

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

LIFE TECHNOLOGIES CELL LINE LICENSE AGREEMENT

This CELL LINE LICENSE AGREEMENT (the "**AGREEMENT**" or the "**LICENSE**"**)**, effective as of November 20, 2016 (the "**EFFECTIVE DATE**"**)**, by and between Life Technologies Corporation, a Delaware corporation having its principal place of business at 5781 Van Allen Way, Carlsbad, CA 92008 USA **(**"**LICENSOR**"**),** and Solid Biosciences, a Delaware limited liability company having its principal place of business at 161 Third Street, Third Floor, Cambridge, MA 02142 **(**"**LICENSEE**"**)**. Each of LICENSOR and LICENSEE may be referred to herein as a "**PARTY**" and collectively as the "**PARTIES**".

BACKGROUND RECITALS

WHEREAS, LICENSOR has developed certain [**] **(**"**CELLS**", as defined below);

WHEREAS, LICENSEE desires to obtain a non-exclusive license to use such CELLS for producing adeno-associated virus vectors for certain purposes, including, but not limited to, research, development (including human clinical trials), manufacturing and commercial uses; and

WHEREAS, is willing to grant to LICENSEE a non-exclusive license to use CELLS under the terms and conditions set forth hereunder.

AGREEMENT

NOW THEREFORE, in consideration of the premises and of the mutual covenants and agreements contained herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the PARTIES intending to be legally bound agree as follows:

1. Definitions. For the purposes of this AGREEMENT, the terms set forth hereinafter are defined as follows:

1.1"**AFFILIATE**" means with respect LICENSEE, any entity Controlled by LICENSEE, so long as such Control exists and with respect to LICENSOR, any entity Controlling, Controlled by, or under common Control with LICENSOR, for only so long as such control exists. For the purposes of this definition, the word "CONTROL" means the actual power, either directly or indirectly through one or more intermediaries, to direct or cause the direction of the management and policies of such entity, whether by the ownership of more than fifty percent (50%) of the voting stock of such entity, or by contract or otherwise.

1.2"**BUSINESS DAY**" means a day other than Saturday, Sunday or any other day on which commercial banks located in the U.S. are authorized or obligated by LAWS to close.

1.3“**CLAIMS**” has the meaning set forth in Section 11.1.

1.4“**COMMERCIALIZATION**” means any and all activities directed to the preparation for sale of, offering for sale of, or sale of, a LICENSEE PRODUCT, including activities related to making (or having made by SERVICE PROVIDERS), manufacturing, marketing, promoting, distributing and importing such LICENSEE PRODUCT, and interacting with REGULATORY AUTHORITIES regarding any of the foregoing. “**COMMERCIALIZE**” means to engage in COMMERCIALIZATION.

1.5“**CONFIDENTIAL INFORMATION**” has the meaning set forth in Section 5.1.

1.6“**CURE PERIOD**” has the meaning set forth in Section 7.3.

1.7“**DISTRIBUTOR**” means a THIRD PARTY to whom, pursuant to a written agreement with such THIRD PARTY, LICENSEE sells LICENSEE PRODUCTS under LICENSEE’S label for resale to customers. In no event shall any THIRD PARTY have the rights to make modifications to any LICENSEE PRODUCTS or part thereof, including relabeling such LICENSEE PRODUCTS or part thereof, except for placement of the DISTRIBUTOR’S name on such LICENSEE PRODUCT to comply with any country-specific regulatory requirements.

1.8“**FDA**” means the U.S. Food and Drug Administration or any successor agency thereto.

1.9“**FEE(S)**” has the meaning set forth in Section 3.

1.10“**GOVERNMENTAL AUTHORITY**” means any multi-national, federal, state, local, municipal or other government authority of any nature (including any governmental division, subdivision, department, agency, bureau, branch, office, commission, council, court, or other tribunal).

1.11“**LAWS**” means all applicable laws, statutes, rules, regulations, ordinances, compliance guidance in final form, and other pronouncements, all as amended from time to time, having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision, domestic or foreign.

1.12“**LICENSEE PRODUCT(S)**” means genetically engineered adeno-associated virus vectors that (i) are developed and manufactured by LICENSEE or its AFFILIATES, or on behalf of LICENSEE by SERVICE PROVIDER(S), through the use of CELLS, and (ii) are used in the treatment of or in the preparation of the treatment of Duchenne Muscular Dystrophy (DMD).

1.13“**CELLS**” means (i) [**] transferred by LICENSOR to LICENSEE’S designated SERVICE PROVIDER prior to the EFFECTIVE DATE; and (ii) any and all progeny thereof generated by LICENSEE or its AFFILIATES.

1.14“**INDEMNITEES**” has the meaning set forth in Section 11.1.

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1.15“**RIGHTS**” means (i) all proprietary rights of LICENSOR in and to the LICENSOR CELLS including biological materials rights; (ii) all proprietary know-how, trade secrets, data, test results, techniques, procedures, compositions, methods, formulas, protocols and information, in each case, if developed and provided by LICENSOR or its AFFILIATES.

1.16“**NON-COMPLIANT ENTITY**” has the meaning set forth in Section 7.3.

1.17“**PHASE I CLINICAL STUDY**” means a clinical study of a LICENSEE PRODUCT in human volunteers or patients with the endpoint of determining initial tolerance, toxicity, safety, or pharmacokinetic information, which will be deemed commenced when the first volunteer or patient in such study has received his or her initial dose of a LICENSEE PRODUCT.

1.18“**REGULATORY AUTHORIZATION**” means any approval or authorization of any REGULATORY AUTHORITY in a particular jurisdiction that is necessary for the development (including human clinical trials), manufacture, use, storage, import, transport and/or sale of LICENSEE PRODUCTS in such jurisdiction in accordance with LAWS.

1.19“**REGULATORY AUTHORITY**” means any applicable GOVERNMENTAL AUTHORITY involved in granting REGULATORY AUTHORIZATION in any country or jurisdiction, including without limitation, in the U.S., the FDA and any other applicable GOVERNMENTAL AUTHORITY having jurisdiction over the CELLS or over LICENSEE PRODUCTS.

1.20“**REGULATORY FILINGS**” means regulatory applications, submissions, notifications, registrations, REGULATORY AUTHORIZATIONS, or other submissions made to or with a REGULATORY AUTHORITY that are necessary or reasonably desirable in order to research, develop (including human clinical trials), manufacture, market, sell or otherwise commercialize LICENSEE PRODUCTS in a particular country or jurisdiction.

1.21“**SERVICE PROVIDER**” means a THIRD PARTY, including, but not limited to a contractor, subcontractor or contract service organization, that performs services for consideration on behalf of LICENSEE or its AFFILIATE and solely for the benefit of LICENSEE or such AFFILIATE of LICENSEE, and with whom LICENSEE or its AFFILIATE has entered into a written agreement for the provision of services for LICENSEE or its AFFILIATE that employs CELLS, for purposes which include, without limitation, the research, development (including human clinical trials), testing, analysis, expression, assay, manufacture, production and storage of LICENSEE PRODUCTS.

1.22“**TERM**” has the meaning set forth in Section 7.1.

1.23“**TERRITORY**” means worldwide.

1.24“**THIRD PARTY**” means any person or entity other than LICENSOR, AFFILIATES of LICENSOR, LICENSEE, and AFFILIATES of LICENSEE.

1.25“**U.S.**” means the United States of America.

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2. Grant of License; Affiliates; Service Providers; Support.

2.1 Grant of License.

(a) Subject to the terms and conditions of this AGREEMENT and the payment of the applicable FEES, LICENSOR hereby grants to LICENSEE, and LICENSEE hereby accepts from LICENSOR, a worldwide, non-exclusive, royalty-free, non-transferable (except as set forth in Sections 2.1(c) and 2.1(d) hereof) license in the TERRITORY under the RIGHTS for LICENSEE to use the CELLS (i) to develop LICENSEE PRODUCTS and to have SERVICE PROVIDERS develop LICENSEE PRODUCTS for or on behalf of LICENSEE only and (ii) to make, have made by SERVICE PROVIDERS, import, sell and have sold by DISTRIBUTORS such LICENSEE PRODUCTS, all solely under LICENSEE's brand.

(b) Any rights of LICENSEE under this AGREEMENT may be exercised and any obligations of LICENSEE under this AGREEMENT may be performed by any AFFILIATE of LICENSEE to the extent that the AFFILIATE remains an AFFILIATE of LICENSEE. LICENSEE hereby unconditionally guarantees the compliance with and performance by its AFFILIATES of all applicable provisions of this AGREEMENT and will be responsible and jointly and severally liable for all of its and its AFFILIATES' obligations due pursuant to this AGREEMENT. A breach of this AGREEMENT by any of LICENSEE's AFFILIATES will be deemed a breach by LICENSEE.

(c) For clarity, nothing herein will preclude LICENSEE from entering into, or will require consent from LICENSOR with respect to, agreements with any SERVICE PROVIDERS to transfer CELLS to SERVICE PROVIDERS, for use by such SERVICE PROVIDERS for purposes which include, without limitation, the research, development (including human clinical trials), testing, analysis, expression, manufacture, production and storage of LICENSEE PRODUCTS in accordance with the rights granted hereunder, *provided that* each such SERVICE PROVIDER to which CELLS are transferred after the EFFECTIVE DATE agrees in writing (i) only to use such CELLS on behalf of LICENSEE as provided hereunder; (ii) not to transfer CELLS to, or use CELLS on behalf of, any THIRD PARTY; (iii) not to use CELLS for the benefit of such SERVICE PROVIDER other than such use on behalf of LICENSEE hereunder; and (iv) to return to LICENSEE or destroy all CELLS in its possession upon completion or termination of its activities on behalf of LICENSEE, and to certify such return or destruction in writing to LICENSEE (with a copy of such certification provided to LICENSOR upon request). LICENSEE shall promptly notify LICENSOR once it becomes aware that any SERVICE PROVIDER is using CELLS other than as permitted under this AGREEMENT. LICENSEE agrees that its continued employment of a SERVICE PROVIDER when LICENSEE and/or its AFFILIATES are aware or should be aware that such SERVICE PROVIDER is using CELLS other than as permitted hereunder, if not cured as provided under Section 7.3, shall constitute a material breach by LICENSEE under this AGREEMENT. Notwithstanding the foregoing, LICENSEE shall remain responsible for its own and its SERVICE PROVIDERS' performance under this AGREEMENT.

(d) No licenses provided under this AGREEMENT may be sublicensed, assigned, or otherwise transferred by LICENSEE except in accordance with Section 8, below.

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(e) Except as set forth in Section 2.1(c) or Section 2.1(d) hereof, LICENSEE shall have no right to transfer CELLS to any THIRD PARTY under this AGREEMENT.

(f) No other rights are conveyed to LICENSEE by LICENSOR by implication, estoppel or otherwise.

2.2Limitations. LICENSEE acknowledges and agrees that LICENSEE does not acquire any rights hereunder to:

(a) transfer CELLS to any THIRD PARTY other than its SERVICE PROVIDERS or the SERVICE PROVIDERS of its AFFILIATES as specifically set forth in Section 2.1;

(b) sell or offer to sell CELLS to any THIRD PARTY;

(c) directly administer the CELLS into humans or animals; or

(d) direct the replication of or the use of the CELLS for any purpose other than as set forth in Section 2.1 or as otherwise specified herein.

2.3No Implied License. Nothing in this AGREEMENT shall be construed as conferring explicitly or by implication, estoppel or otherwise any license, right or immunity under any rights that LICENSOR (and its successors, AFFILIATES and assigns, and successors, AFFILIATES and assigns of each of the foregoing) now owns or holds a license to, or acquires or obtains a license to in the future, other than the specifically identified RIGHTS for use in connection with LICENSEE PRODUCTS, regardless of whether such other rights are dominant or subordinate to the RIGHTS. Furthermore, LICENSEE and its AFFILIATES have not provided and will not provide, and LICENSOR and its AFFILIATES have not received and will not receive, any consideration except that which is expressly provided herein for the specific rights expressly granted herein. LICENSOR and its AFFILIATES do not represent or warrant that the RIGHTS include all rights owned, licensed or controlled by LICENSOR and/or its AFFILIATES that may pertain to (i) the full breadth and scope of RIGHTS and/or LICENSEE PRODUCTS, (ii) the full breadth and scope of compositions and/or methods LICENSEE and/or its AFFILIATES may employ related to RIGHTS and/or LICENSEE PRODUCTS, (iii) the full breadth and scope of methods and/or compositions an end user customer may employ related to RIGHTS and/or LICENSEE PRODUCTS, and/or (iv) the full breadth and scope of intellectual property that may arise related to RIGHTS and/or LICENSEE PRODUCTS

2.4Regulatory Support. In the event that LICENSEE engages in clinical and regulatory activities directed towards obtaining regulatory approval of a LICENSEE PRODUCT and desires additional assistance or information from LICENSOR with respect to the cell lineage history, testing results or any other cell line documentation relating to the CELLS, then LICENSOR shall, at the expense of LICENSEE, use commercially reasonable efforts to provide such assistance or information.

3. Fees. As consideration for the rights granted hereunder, LICENSEE agrees to pay LICENSOR a non-refundable, non-creditable license fee of [**], due upon the EFFECTIVE DATE and payable within fifteen (15) days thereof ("**LICENSE FEE**"). For clarity, termination

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of this AGREEMENT at any time after the EFFECTIVE DATE shall not relieve LICENSEE of any unfulfilled payment obligations of the LICENSE FEE.

4. Payment. All payments due hereunder shall be payable in U.S. dollars and shall be made by check or wire transfer to the appropriate account as follows:

Payment by check shall be made to:

Thermo Fisher Scientific, Inc.
Life Technologies Corporation
5823 Newton Drive
Carlsbad, CA 92008 USA
Attention: License Management and Contract Compliance (LMCC)

Payment by wire transfer shall be made to:

LICENSEE shall be responsible for any and all bank transfer charges associated with payments required to be made by it or its AFFILIATES under this AGREEMENT.

Any amount not paid by LICENSEE when due will bear interest at an annual rate of [**] over the prime rate offered by Citibank N.A. on the date the payment is due until the due date the payment is made. The payment of such interest shall not prohibit LICENSOR from exercising any other rights it may have as a consequence of the lateness of the payment.

5. Confidentiality; Press Release; Use of Marks.

5.1CONFIDENTIAL INFORMATION. The term "**CONFIDENTIAL INFORMATION**" in this AGREEMENT means all non-public or proprietary information disclosed by or on behalf of a PARTY or its AFFILIATES to the other PARTY pursuant to this AGREEMENT, which may include ideas, inventions, discoveries, concepts, compounds, compositions, formulations, formulas, practices, procedures, processes, methods, knowledge, know-how, trade secrets, technology, inventories, machines, techniques, development, designs, drawings, computer programs, skill, experience, documents, apparatus, results, clinical and regulatory strategies, regulatory documentation, information and submissions pertaining to, or made in association with, filings with any REGULATORY AUTHORITY, data, including pharmacological, toxicological and clinical data, analytical and quality control data, manufacturing data and descriptions, patent and legal data, market data, financial data or descriptions, devices, assays, chemical formulations, specifications, material, product samples and other samples, physical, chemical and biological materials and compounds, and the like, in whatever form or medium, and whether or not designated or marked "confidential" or "proprietary." CONFIDENTIAL INFORMATION shall include the terms and conditions of this AGREEMENT.

5.2Exclusions. Notwithstanding any other provision of this AGREEMENT, CONFIDENTIAL INFORMATION shall not include any item of information that the receiving party demonstrates: (i) became generally available to the public other than as a result of disclosure by the receiving party or its AFFILIATES; (ii) was available to the receiving party and its AFFILIATES on a non-confidential basis prior to the disclosure to the receiving party by

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the disclosing party; (iii) became available to the receiving party or its AFFILIATES on a non-confidential basis from a source other than the disclosing party; *provided that,* such source is not bound by an obligation of confidentiality to the disclosing party; or (iv) was independently developed by the receiving party without use of the CONFIDENTIAL INFORMATION as evidenced by written records.

5.3Non-Use and Confidentiality. The PARTIES shall maintain the CONFIDENTIAL INFORMATION in strict confidence and the PARTIES shall use CONFIDENTIAL INFORMATION only in accordance with the terms and conditions of this AGREEMENT. Each PARTY shall (i) limit its dissemination of CONFIDENTIAL INFORMATION to only those employees and agents of such PARTY and such PARTY's AFFILIATES, who require such CONFIDENTIAL INFORMATION in order to exercise the rights of each PARTY and such PARTY's AFFILIATES under this AGREEMENT and such employees and agents shall be subject to obligations of confidentiality at least as restrictive as those specified herein; and (ii) not disclose, without the prior written consent of the other PARTY, CONFIDENTIAL INFORMATION to any THIRD PARTY other than to (a) an AFFILIATE or SERVICE PROVIDER to the extent required for the purposes of this AGREEMENT, (b) to a REGULATORY AUTHORITY in connection with REGULATORY FILINGS, or (c) to any GOVERNMENTAL AUTHORITY in accordance with LAWS. In the event that the receiving party or anyone to whom it transmits the CONFIDENTIAL INFORMATION pursuant to this AGREEMENT becomes legally required to disclose any such CONFIDENTIAL INFORMATION, the receiving party shall provide the disclosing party with prompt notice of such required disclosure so that the disclosing party may seek a protective order or other appropriate remedy and/or waive compliance with the provisions of this AGREEMENT. In the event that such protective order or other remedy is not obtained, the receiving party shall furnish only that portion of the CONFIDENTIAL INFORMATION which is legally required to be furnished in the written opinion of the receiving party's counsel. The burdens of non-use and confidentiality under this Agreement will continue until terminated by mutual agreement between the PARTIES hereto.

5.4Press Release. Neither PARTY will make any public press release or similar publicity announcement or disclosure that includes the other PARTY's names, logos, trademarks or service marks, or the physical likeness or names of its employees or investigators or other symbols of the other PARTY without the other PARTY's prior written consent.

5.5Limited Use of Marks. LICENSEE shall not, at any time, employ any of the trade names, trademarks, trade dress, slogans, designs, or the like of LICENSOR or its AFFILIATES for any advertising, promotional, or other purposes without prior written permission to do so from LICENSOR; *provided, however,* that LICENSEE may disclose the existence of this AGREEMENT and the name of LICENSOR and its AFFILIATES to LICENSEE's AFFILIATES, SERVICE PROVIDERS and THIRD PARTIES, and to REGULATORY AUTHORITIES in connection with REGULATORY FILINGS and in accordance with the requirements of any GOVERNMENTAL AUTHORITIES (subject to Section 5.3 above).

6. Reservation of Rights. This AGREEMENT shall not limit the rights of LICENSOR or its AFFILIATES in any way regarding RIGHTS. It is specifically understood that as between the PARTIES to this AGREEMENT, LICENSOR reserves the right for itself or through its

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AFFILIATES to exercise its rights in its intellectual property, and to license, sublicense, assign or otherwise transfer such rights to others for any purpose whatsoever, under any terms it so chooses, in its sole discretion or not at all, including terms and conditions that are substantially similar to or different from those in this AGREEMENT. For purposes of clarification, LICENSOR may provide THIRD PARTIES with draft contracts that are substantially similar to this AGREEMENT at its sole discretion.

This AGREEMENT shall also not limit the rights of LICENSEE or its AFFILIATES to license their own intellectual property rights to any THIRD PARTY. For clarity, in order for any THIRD PARTY to use CELLS or any LICENSOR intellectual property rights thereto (including RIGHTS) for commercial purposes (including without limitation pre-IND commercial research), except as set forth in 2.1 (c), such a THIRD PARTY must obtain a separate license from LICENSOR for consideration and on terms and conditions to be determined by LICENSOR, and it is a material condition of this AGREEMENT that LICENSEE inform its clients, potential licensees and other THIRD PARTIES interested in such rights of this requirement in writing with a copy of each such notification to LICENSOR.

7. Term and Termination

7.1TERM. This license is granted to LICENSEE as of the EFFECTIVE DATE, and will continue in perpetuity unless earlier terminated in accordance with this Section 7 (the "**TERM**").

7.2Termination by LICENSEE. LICENSEE may terminate this AGREEMENT without specification of any reason with thirty (30) days' prior written notice to LICENSOR. Any such termination shall become effective at the end of the thirty (30) day notice period.

7.3Termination by LICENSOR.

(i) Upon a material breach or default of a material term under this AGREEMENT by LICENSEE or an AFFILIATE of LICENSEE, including without limitation a failure to pay fees owed as specified in this AGREEMENT, this AGREEMENT may be terminated by LICENSOR upon sixty (60) days prior written notice to LICENSEE (the "**CURE PERIOD**"). Any termination of this AGREEMENT pursuant to this Section 7.3 shall become effective at the end of the CURE PERIOD, unless LICENSEE has cured any such material breach prior to the expiration of such CURE PERIOD.

(ii) In the event that LICENSEE notifies LICENSOR, or LICENSOR becomes independently aware, that any of LICENSEE's AFFILIATES or a particular SERVICE PROVIDER is using CELLS other than as permitted under this AGREEMENT (a "**NON-COMPLIANT ENTITY**"), the rights conveyed by LICENSEE or its AFFILIATES to such NON-COMPLIANT ENTITY under this AGREEMENT may be terminated by LICENSOR upon sixty (60) days' written notice to LICENSEE. Said notice shall become effective at the end of the sixty (60) day period, unless during said period LICENSEE causes the NON-COMPLIANT ENTITY to cure the non-compliant activities, and LICENSEE provides clear written evidence of such cure to LICENSOR.

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(iii)LICENSOR shall have the right to terminate this AGREEMENT immediately at any time upon written notice to LICENSEE in the event that LICENSOR reasonably determines that continued performance under the AGREEMENT may violate any LAWS. LICENSOR shall communicate with LICENSEE regarding the circumstances giving rise to such termination and shall use commercially reasonable efforts to provide LICENSEE with advance notice of such termination. Prior to terminating the AGREEMENT as set forth herein, LICENSOR shall use commercially reasonable efforts to mitigate the potential violation of any LAWS.

Termination by LICENSOR in compliance with this Section 7.3 shall not, in any event, constitute a breach of this AGREEMENT.

7.4Effect of Termination.

(a) Upon the effective date of termination of this AGREEMENT, all rights and licenses granted to LICENSEE and its AFFILIATES by LICENSOR hereunder, including any rights extended by LICENSEE and/or its AFFILIATES to SERVICE PROVIDERS or DISTRIBUTORS shall automatically and immediately terminate and LICENSEE shall immediately stop, and shall cause (if applicable) its AFFILIATES, SERVICE PROVIDERS, and DISTRIBUTORS to immediately stop, exercising the license rights granted to LICENSEE in Section 2.1 of this AGREEMENT.

(b) LICENSEE shall, as soon as practicable, but in any event, within sixty (60) days following the effective date of termination of this AGREEMENT cause its AFFILIATES and SERVICE PROVIDERS to return to LICENSEE or destroy all CELLS in such AFFILIATES' and/or SERVICE PROVIDERS' possession, with certification of such return or destruction in writing to LICENSEE (with a copy of such certification provided to LICENSOR upon request).

(c) Upon termination for any reason of rights conveyed by LICENSEE or its AFFILIATES to any AFFILIATE or SERVICE PROVIDER under this AGREEMENT, which termination does not include termination of the licenses granted to LICENSEE hereunder, LICENSEE shall, within thirty (30) days following the effective date of such termination, cause the terminated AFFILIATE or SERVICE PROVIDER to return to LICENSEE or destroy all CELLS in such AFFILIATE's or SERVICE PROVIDER's possession and to certify such return or destruction in writing to LICENSEE (with a copy of such certification provided to LICENSOR upon request).

(d) All rights and obligations of the PARTIES set forth herein that expressly or by their nature survive the expiration, assignment or termination of this AGREEMENT shall continue in full force and effect subsequent to, and notwithstanding the termination of this AGREEMENT until they are satisfied or by their nature expire and shall bind the PARTIES and their legal representatives, successors, and permitted assigns, including, without limitation (i) Sections 3 and 4 (to the extent that payment obligations existing before expiration or termination of this AGREEMENT remain unmet upon expiration or termination of this AGREEMENT); and (ii) Sections 1, 5, 6, 7.4, 8, 9.5, 9.6, 10, 11 and 12.

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8. Assignment/Transferability.

8.1Assignment by LICENSEE. This AGREEMENT is personal to LICENSEE and neither this AGREEMENT nor any right or obligation hereunder may be assigned or otherwise transferred (whether voluntarily, by operation of law or otherwise, including, without limitation (i) through the acquisition by any person or group, directly or indirectly, of the beneficial ownership of more than fifty percent (50%) of the total voting power of LICENSEE; (ii) through a merger of LICENSEE into another person or entity; and (iii) through the sale, lease or transfer of all or substantially all of the assets of LICENSEE to any person or entity in one or a series of related transactions with any of the foregoing referred to as a **"CHANGE OF CONTROL")** by LICENSEE, without the prior express written consent of LICENSOR, which shall not be unreasonably withheld, except that, without such consent, LICENSEE may transfer this Agreement to SOLID GT, LLC.

8.2Assignment by LICENSOR. LICENSOR may assign all or any part of its rights and obligations under this AGREEMENT at any time without the consent of LICENSEE or its AFFILIATES or (if applicable) any successor or permitted transferee of LICENSEE to whom this AGREEMENT may have been assigned pursuant to Section 8.1. LICENSEE or (if applicable) any successor or permitted transferee agrees to execute such further acknowledgments or other instruments as LICENSOR may reasonably request in connection with such assignment.

8.3Binding Effect. Any permitted assignment of this AGREEMENT shall be binding on the assignee. Any purported assignment or other transfer of this AGREEMENT other than as expressly set forth in Section 2.1 or this Section 8 shall be null and void.

9. Warranties and Representations; Acknowledgements; Limitation of Liability.

9.1By LICENSOR. LICENSOR represents and warrants that, as of the EFFECTIVE DATE, it has the full right and authority to enter into this AGREEMENT and to grant to LICENSEE the rights granted in Section 2 of this AGREEMENT. For the avoidance of doubt, CELLS were provided "as is" solely for LICENSEE to generate derivatives products. Except as provided in this Section 9.1, LICENSOR makes no representations or warranties concerning the CELLS.

9.2By LICENSEE.

(a) LICENSEE represents, warrants and covenants to LICENSOR that:

(i) LICENSEE has the full right and authority to enter into this AGREEMENT;

(ii) the use of CELLS by LICENSEE, its AFFILIATES and SERVICE PROVIDERS prior to the EFFECTIVE DATE has been in compliance with the non-financial terms and conditions of this AGREEMENT;

(iii)LICENSEE has complied with and shall comply with and require its AFFILIATES and SERVICE PROVIDERS to comply with all (i) LAWS; and (ii)

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requirements of REGULATORY AUTHORITIES in connection with the exercise of the rights granted to LICENSEE by LICENSOR hereunder;

(iv)LICENSEE will not resell CELLS;

(v) LICENSEE has and will maintain the technical and other requisite competencies to determine, and is solely responsible for determining, the suitability of the CELLS purchased from LICENSOR for use by LICENSEE;

(vi)LICENSEE and its AFFILIATES, as applicable, will conduct all necessary tests, comply with all applicable regulatory requirements and obtain all applicable REGULATORY AUTHORIZATIONS, issue all appropriate warnings and information to users, and be responsible for obtaining any required THIRD PARTY intellectual property rights with respect to LICENSEE'S and its AFFILIATES' (a) use of CELLS or RIGHTS and (b) COMMERCIALIZATION of LICENSEE PRODUCTS;

(vii)LICENSEE will adhere to LICENSEE'S procedures, current Good Manufacturing Practices (cGMP) process for manufacturing LICENSEE PRODUCTS, and will conduct all testing needed to ensure the safety, potency and purity of LICENSEE PRODUCTS and compliance with LAWS;

(viii)LICENSEE shall diligently pursue REGULATORY AUTHORIZATION for LICENSEE PRODUCTS and shall not sell or cause to be sold, use or cause to be used such LICENSEE PRODUCTS in any manner requiring such REGULATORY AUTHORIZATION until it is finally obtained; and

(ix)LICENSEE will comply with all applicable anticorruption and antibribery laws and will not knowingly take any action that would cause LICENSOR or any of its AFFILIATES to be in violation of such laws. As part of such compliance, LICENSEE represents that it shall not offer or make any improper payments of money or anything of value to a non-U.S. Government Official in connection with this AGREEMENT. Licensee shall not offer or make improper payments to a THIRD PARTY knowing, or suspecting, that the THIRD PARTY will give the payment, or a portion of it, to a Government Official.

(b) LICENSEE acknowledges and covenants that:

(i) CELLS were originally sold by LICENSOR and its AFFILIATES for research use only and are expressly not qualified for commercial, therapeutic or biotherapeutic purposes by LICENSOR or its AFFILIATES. LICENSEE assumes all responsibility and liability associated with LICENSEE'S use of CELLS for human use;

(ii) CELLS have not been tested by or for LICENSOR for safety or efficacy or any other purpose, unless expressly stated in LICENSOR's catalogues or on the label or other documentation accompanying the CELLS at the time the CELLS were sold to LICENSEE;

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(iii)Except as provided in Section 2.4, LICENSOR has no obligation to develop any cell lineage history, testing results or any other cell line documentation relating to the CELLS;

(iv)Except as provided in Section 2.4, LICENSOR has not provided, nor does any term in this AGREEMENT require it to provide to LICENSEE, its AFFILIATES or any THIRD PARTY, any cell lineage history, testing results or any other cell line documentation relating to the CELLS at any time;

(v) there are gaps in the cell line history of CELLS. Except as provided in Section 2.4, LICENSEE agrees to assume all responsibility for addressing those gaps and documenting the steps it took to address those gaps. LICENSEE agrees to assume all responsibility for testing the CELLS, any derivatives thereof, and any products, including LICENSEE PRODUCTS that LICENSEE or its AFFILIATES offers for sale or sells or services that LICENSEE or its AFFILIATES performs that are manufactured using, derivatives of, or ever came in contact with the CELLS, for viral and/or bacterial contamination or other adventitious agents, and that it will remove any such contamination or adventitious agent from any additional processes, materials, or products that LICENSEE may create using or that came in contact with CELLS;

(vi)LICENSOR developed CELLS and provided CELLS to LICENSEE without expectation that CELLS would be used in humans;

(vii)LICENSEE's use of CELLS may require authorization from or licensure of THIRD PARTY intellectual property or proprietary rights and such THIRD PARTY rights are not herein conferred by LICENSOR to LICENSEE by implication, estoppel, or otherwise; and

(viii)nothing in this AGREEMENT shall be construed as conferring the right to use in advertising, publicity or otherwise any trademarks or any contraction, abbreviation, simulation or adaptation thereof, of LICENSOR, except as expressly set forth herein.

9.3Mutual. Each PARTY represents and warrants to the other PARTY that (i) such PARTY is a company or corporation duly organized, validly existing and in good standing under the laws of the jurisdiction in which it is organized; (ii) such PARTY has the legal power and authority to execute, deliver and perform this AGREEMENT; (iii) the execution, delivery and performance by such PARTY of this AGREEMENT has been duly authorized by all necessary corporate action; (iv) this AGREEMENT constitutes the legal, valid and binding obligation of such PARTY, enforceable against such PARTY in accordance with its terms; and (v) the execution, delivery and performance of this AGREEMENT will not cause or result in a violation of any law, of such PARTY's charter documents, or of any contract by which such PARTY is bound.

9.4Anti-Boycott. Notwithstanding any other provision of this AGREEMENT, neither LICENSEE nor LICENSOR shall be required to take or refrain from taking any action impermissible or penalized under the laws of the United States or any applicable foreign

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jurisdiction, including without limitation the anti-boycott laws administered by the U.S. Commerce and Treasury Departments.

9.5Disclaimer of Other Warranties. EXCEPT AS EXPRESSLY SET FORTH HEREIN, NEITHER LICENSOR NOR ANY OF ITS AFFILIATES MAKES ANY WARRANTIES, EXPRESS OR IMPLIED OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE, OR THAT THE MANUFACTURE, USE, IMPORT OR SALE OF CELLS OR LICENSEE PRODUCTS WILL BE FREE FROM INFRINGEMENT OF ANY PATENT OR OTHER INTELLECTUAL OR PROPRIETARY RIGHTS OF A THIRD PARTY. LICENSOR AND ITS AFFILIATES EXPRESSLY DISCLAIM ANY AND ALL WARRANTIES THAT THE USE OF CELLS, INCLUDING WITHOUT LIMITATION, THE USE OF CELLS IN THE MANUFACTURE OF LICENSEE PRODUCTS OR COMPONENTS THEREOF, THE USE OF THE RIGHTS OR THE USE OR TRANSFER OF SUCH LICENSEE PRODUCTS OR COMPONENTS THEREOF BY OR TO ANY AFFILIATE OR THIRD PARTY (INCLUDING A SERVICE PROVIDER), AND/OR ANY RESULTS OBTAINED BY USING SUCH CELLS, RIGHTS OR LICENSEE PRODUCTS OR COMPONENTS THEREOF, ARE, OR WILL BE, FREE FROM INFRINGEMENT OF ANY PATENT OR OTHER INTELLECTUAL OR PROPRIETARY RIGHTS OF THIRD PARTIES; AND THIS ALLOCATION OF RISK BETWEEN THE PARTIES IS REFLECTED IN THE TERMS OF THE AGREEMENT AND IS AN ESSENTIAL ELEMENT OF THE BARGAIN BETWEEN THE PARTIES.

NEITHER LICENSOR NOR ANY OF ITS AFFILIATES MAKES ANY WARRANTIES, EXPRESS OR IMPLIED, AS TO THE SUITABILITY OF THE CELLS FOR HUMAN USE OR COMMERCIALIZATION.

9.6Indirect Damages. NEITHER LICENSOR NOR ITS AFFILIATES SHALL BE LIABLE HEREUNDER TO LICENSEE, ITS AFFILIATES OR ANY OTHER PERSON OR ENTITY FOR SPECIAL, INCIDENTAL, CONSEQUENTIAL, EXEMPLARY OR OTHER INDIRECT DAMAGES (INCLUDING, BUT NOT LIMITED TO, LOSS OF PROFITS OR LOSS OF USE DAMAGES) ARISING FROM THE MANUFACTURE OR USE OF CELLS OR LICENSEE PRODUCTS, OR THE USE OF THE RIGHTS, OR IN CONNECTION WITH THE PERFORMANCE OF THIS AGREEMENT, EVEN IF LICENSOR AND/OR ITS AFFILIATES HAVE BEEN ADVISED OF THE POSSIBILITY OF SUCH DAMAGES OR LOSSES.

9.7Limitation of Liability. UNDER NO CIRCUMSTANCES SHALL THE TOTAL LIABILITY OF LICENSOR AND ITS AFFILIATES, ARISING OUT OF OR RELATED TO THIS AGREEMENT INCLUDING, REGARDLESS OF THE FORUM AND REGARDLESS OF WHETHER ANY ACTION OR CLAIM IS BASED ON CONTRACT, TORT, OR ANY OTHER LEGAL THEORY, EXCEED THE TOTAL AMOUNT PAID BY LICENSEE COLLECTIVELY TO LICENSOR AND ITS AFFILIATES HEREUNDER (DETERMINED AS OF THE DATE OF ANY FINAL JUDGMENT IN SUCH ACTION).

10. Export Regulations. LICENSEE on behalf of itself and its AFFILIATES hereby agrees to comply with all applicable U.S. export laws administered by the FDA and U.S. export control and economic sanctions laws, regulations, and orders, including without limitation those

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regulations maintained by the U.S. Treasury Department's Office of Foreign Assets Control and the U.S. Commerce Department's Bureau of Industry and Security. Without limiting the foregoing, LICENSEE covenants and agrees that neither it nor its AFFILIATES shall, directly or indirectly, sell, export, re-export, transfer, divert, or otherwise release or dispose of any equipment, product, commodities, services, software, samples, materials, information, technical data, or technology received under this AGREEMENT to or through any individual, entity, or destination, or for use prohibited by the laws or regulations of the U.S. or any other applicable jurisdiction without having obtained prior authorization from the competent GOVERNMENTAL AUTHORITIES as required by all such laws and regulations. LICENSEE's or any of its AFFILIATES' breach of this provision shall constitute cause for immediate termination of this AGREEMENT. LICENSEE agrees to indemnify and hold harmless LICENSOR and its AFFILIATES for LICENSEE's or any of its AFFILIATES' non-compliance with these controls in connection with a breach of this provision.

11. Indemnity; Insurance.

11.1 Indemnification by LICENSEE. LICENSEE shall defend, indemnify and hold LICENSOR, AFFILIATES, and its and their respective officers, directors, employees and agents (the **"INDEMNITEES"),** harmless from and against all liability, damages, expenses (including reasonable attorneys' and expert witness fees and expenses), recoveries and losses resulting from any death, personal injury, illness or property damage (collectively, **"LOSSES")** resulting from any claims (including any claims for infringement or misappropriation of intellectual property), demands, actions, suits or proceedings (collectively, **"CLAIMS")** brought by a THIRD PARTY to the extent that such CLAIMS arise out of, are based on, or result from (i) the replication or use of CELLS by LICENSEE, its AFFILIATES or SERVICE PROVIDERS; (ii) the use of RIGHTS by LICENSEE, its AFFILIATES or SERVICE PROVIDERS; (iii) breach by LICENSEE or any of its AFFILIATES or SERVICE PROVIDERS of any representation, warranty or covenant made by LICENSEE in this AGREEMENT or (iv) any use, sale, or import of LICENSEE PRODUCTS, including but not limited to, use or reliance upon such LICENSEE PRODUCTS or RIGHTS, by LICENSEE, its AFFILIATES, SERVICE PROVIDERS and/or its or their DISTRIBUTORS or customers.

11.2 Indemnification Procedures. INDEMNITEES shall give written notice to LICENSEE in a reasonably timely manner after learning of such CLAIM. INDEMNITEES shall provide LICENSEE with reasonable assistance, at LICENSEE's expense, in connection with the defense of the CLAIM for which indemnity is being sought. INDEMNITEES may participate in and monitor such defense with counsel of its own choosing at its sole expense; *provided, however,* that LICENSEE shall have the right to assume and conduct the defense of the CLAIM with counsel of its choice. LICENSEE shall not settle any CLAIM without the prior written consent of the INDEMNITEES, not to be unreasonably withheld or delayed, unless the settlement involves only the payment of money. INDEMNITEES shall not settle any CLAIM without the prior written consent of LICENSEE. If LICENSEE does not assume and conduct the defense of the CLAIM as provided above, (i) INDEMNITEES may defend against, and consent to the entry of any judgment or enter into any settlement with respect to, the CLAIM in any manner the INDEMNITEES may deem reasonably appropriate (and INDEMNITEES need not consult with, or obtain any consent from, LICENSEE in connection therewith); and (ii)

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LICENSEE will remain responsible to indemnify the INDEMNITEES as provided in this Section 11.

11.3Insurance. LICENSEE will maintain the following insurance policies:

(a) Research and Development. As of the EFFECTIVE DATE of this AGREEMENT and until the date on which LICENSEE obtains a REGULATORY AUTHORIZATION to COMMERCIALIZE LICENSEE PRODUCT, LICENSEE will maintain in effect commercial general liability coverage, covering LICENSEE'S obligations under this AGREEMENT with limits not less than [**].

(b) Clinical Trials Insurance. From the first day LICENSEE commences clinical trials using materials manufactured using CELLS ("**LICENSEE'S CLINICAL TRIAL(S)**") and for at least five (5) years of consistent coverage (tail coverage for claims-made policy) after termination of this Agreement, LICENSEE will maintain in effect clinical trial insurance coverage with limits and policy terms required by local LAWS in the territories where the LICENSEE'S CLINICAL TRIALS are taking place and not less than:

(i) [**] upon commencement of PHASE I CLINICAL STUDY; and

(ii) [**] upon commencement of any LICENSEE'S CLINICAL TRIALS beyond PHASE I CLINICAL TRIAL.

(c) Insurance upon COMMERCIALIZATION. Prior to or upon the grant of a REGULATORY AUTHORIZATION to COMMERCIALIZE LICENSEE PRODUCT, LICENSEE will maintain commercial general liability and product liability insurance, covering therapeutic products and LICENSEE'S obligations under the terms of this AGREEMENT, including its indemnification obligations and costs for defense, for any claims arising from bodily injury and property damage regarding the use of CELLS with limits not less than [**]. This insurance policy will be maintained until the later of: (i) the expiration of any applicable statute of limitations, (ii) five (5) years following termination of this AGREEMENT, or (iii) five (5) years following the last sale of LICENSEE PRODUCTS.

(d) The insurance policies, or certificates issued to LICENSEE evidencing such insurance coverage shall:

(i) Name as additional insured each of LICENSOR and its AFFILIATES;

(ii) Be primary and non-contributing with, and not in excess of, any other insurance available to LICENSOR;

(iii)Have reasonable and customary deductible amounts compared to other similar companies in the biotechnology and biopharmaceutical industry;

(iv)Be issued by responsible insurance carriers licensed to do business in the state in which the project is located, and with a rating of not less than A-, as rated in the most currently available "Best's Insurance Guide;"

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(e) LICENSEE will also maintain locally admitted commercial general liability and/or other clinical trial coverage and product liability insurance, covering therapeutic products and LICENSEE'S obligations under the terms of this AGREEMENT, in any other territories where (i) LICENSEE operates, (ii) LICENSEE'S CLINICAL TRIALS are taking place, or (iii) LICENSEE PRODUCTS are manufactured, COMMERCIALIZED, or used, as required by applicable LAWS. Such insurance policies shall name as additional insured each of LICENSOR and its AFFILIATES, if such additional insured language is customary in these territories.

(f) Certificates of insurance evidencing the coverage as required by this Section 11.3 will be delivered to LICENSOR by LICENSEE upon request. LICENSEE will notify LICENSOR if the insurance policy is cancelled, suspended, non-renewed, terminated, or materially altered, within thirty (30) days from such change.

(g) LICENSEE is solely responsible to ensure it maintains the appropriate insurance and level of coverage as required herein. In the event of a failure to carry and maintain the levels of insurance required herein or a failure to remedy any non-conformity, LICENSOR will be entitled to treat such failure as a material breach of the Agreement, in addition to all other rights and remedies available to LICENSOR.

12. General.

12.1Entire Agreement. This AGREEMENT constitutes the entire AGREEMENT between LICENSOR and LICENSEE as to the subject matter hereof, and all prior negotiations, representations, agreements and understandings are merged into, extinguished by and completely expressed by this AGREEMENT. This AGREEMENT may be modified or amended only by a writing executed by authorized officers of both of the PARTIES.

12.2Notices. Any notice required or permitted to be given by this AGREEMENT shall be given in writing in English by postpaid, first class, registered or certified mail, or by courier or facsimile, properly addressed to the other PARTY at the respective address as follows:

If to LICENSOR:

Thermo Fisher Scientific, Inc.
Life Technologies Corporation
5823 Newton Drive
Carlsbad, CA 92008 USA
Attention: License Management & Contract Compliance (LMCC)

With a copy, which shall not constitute notice to:

Thermo Fisher Scientific, Inc.
Life Technologies Corporation
5823 Newton Drive
Carlsbad, CA 92008 USA
Attention: Contracts Department
Fax Number: 750-476-6326

If to LICENSEE:
Solid Biosciences
161 First St. 3rd floor
Cambridge, MA 02142

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Either PARTY may change its address by providing notice to the other PARTY. Unless otherwise specified herein, any notice given in accordance with the foregoing shall be deemed given within four (4) BUSINESS DAYS after the day of mailing, or one (1) BUSINESS DAY after the date of delivery to the courier, as the case may be.

12.3Governing Law. This AGREEMENT shall be interpreted and enforced in accordance with laws of the State of California in the United States of America, without regard to its conflicts of laws rules, provided, that those matters pertaining to the validity or enforceability of patent rights shall be interpreted and enforced in accordance with the laws of the territory in which such patent rights exist. The parties expressly agree that the application of the United Nations Convention on Contracts for the International Sale of Goods (1980) is specifically excluded and shall NOT apply to this Agreement.

12.4Compliance with LAWS. Each PARTY agrees to comply with LAWS in exercising its rights and performing its obligations under this AGREEMENT. Nothing in this AGREEMENT shall be construed so as to require the commission of any act contrary to law, and wherever there is any conflict between any provision of this AGREEMENT or concerning the legal right of the PARTIES to enter into this AGREEMENT and any statute, law, ordinance or treaty, the latter shall prevail, but in such event the affected provisions of this AGREEMENT shall be curtailed and limited only to the extent necessary to bring it within the applicable legal requirements.

12.5Injunctive Relief. Notwithstanding anything herein seemingly to the contrary, either PARTY may seek injunctive relief from a court of competent jurisdiction to prevent or limit damage to that PARTY's CONFIDENTIAL INFORMATION or otherwise preserve the status quo pending the proceeding.

12.6Relationship of Parties. The relationship of the PARTIES is that of independent contractors, and nothing herein shall be construed as establishing one PARTY or its AFFILIATES as the agent, legal representative, joint venturer, partner, employee, or servant of the other PARTY or its AFFILIATES. Except as set forth herein, neither PARTY shall have any right, power or authority to assume, create or incur any expense, liability or obligation, express or implied, on behalf of the other PARTY or its AFFILIATES. No PARTY shall hold itself out as being the agent, legal representative, joint venturer, partner, employee, or servant of the other PARTY or its AFFILIATES or as having authority to represent or act for the other PARTY or its AFFILIATES in any capacity whatsoever, except as authorized herein.

12.7Force Majeure. If the performance of this AGREEMENT or any obligation hereunder (except for the payment of money) is prevented, restricted or interfered with by reason of fire or other casualty or accident, strikes or labor disputes, inability to procure raw materials, power or supplies, war, invasion, civil commotion or other violence, compliance with any order of any governmental authorities or any other act or conditions whatsoever beyond the reasonable control of either PARTY hereto, the PARTY so affected upon giving a prompt notice to the other PARTY shall be excused from such performance to the extent of such prevention, restriction or interference; provided, however, that the PARTY so affected shall use commercially reasonable

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efforts to avoid or remove such causes of non-performance and shall continue performance hereunder with the utmost dispatch whenever such causes are removed, to the extent commercially reasonable.

12.8Unenforceabilitv. If any provision of this AGREEMENT is held to be unenforceable for any reason, it shall be adjusted rather than voided, if possible, in order to achieve the intent of the PARTIES to the extent possible. In any event, all other provisions of this AGREEMENT shall be deemed valid and enforceable to the full extent possible.

12.9Waiver, Modifications and Amendments. The failure of any PARTY to insist on the performance of any obligation hereunder shall not be deemed to be a waiver of such obligation. Waiver of any breach of any provision hereof shall not be deemed a waiver of any other breach of such provision or any other provision on such occasion or any succeeding occasion. No waiver, modification, release or amendment of any obligation under or provision of this AGREEMENT shall be valid or effective unless in writing and signed by the PARTIES.

12.10Headings. Headings used herein are for descriptive purposes only and shall not control or alter the meaning of this AGREEMENT as set forth in the text.

12.11Severability. Should one or more of the provisions contained in this AGREEMENT be held invalid, illegal or unenforceable by a court or tribunal with jurisdiction to do so, then the validity, legality and enforceability of the remaining provisions contained herein shall not be affected or impaired thereby, unless the absence of the invalidated provision(s) adversely affect the PARTIES' substantive rights. In such instance, the PARTIES shall use their best efforts to replace the invalid, illegal or unenforceable provision(s) with valid, legal and enforceable provision(s) which, insofar as practical, implement the purposes of this AGREEMENT.

12.12Counterparts. This AGREEMENT may be signed in one or more counterparts, all of which together shall constitute one and the same AGREEMENT, binding on the PARTIES as if such PARTIES had signed the same document. The execution and delivery of this Agreement by either Party hereto by facsimile transmission or e-mail delivery of a ".pdf' or similarly formatted data file will constitute valid execution and delivery of this Agreement.

IN WITNESS WHEREOF, the PARTIES intending to be legally bound have caused this AGREEMENT to be executed by their respective duly authorized representatives as of the EFFECTIVE DATE.

For LICENSOR:	**For LICENSEE:**
Life Technologies Corporation	**Solid Biosciences**
By: /s/ Deborah Day Barbara	By: /s/ Ilan Ganot
(signature)	(signature)

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Name: Deborah Day Barbara (please print)	Name: llan Ganot (please print)
Title: Leader, Therapeutic Licensing	Title: CEO
Date: 12/01/16	Date:

"

Exhibit 10.55

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

LICENSE AGREEMENT

This License Agreement is entered into as of this 23rd day of June, 2016 (the "Effective Date"), by and between **Solid GT, LLC,** a company organized under the laws of Delaware and having an address at 161 First Street, Suite #300, Cambridge, MA 02142 ("Licensee") and **President and Fellows of Harvard College,** an educational and charitable corporation existing under the laws and the constitution of the Commonwealth of Massachusetts, having a place of business at Richard A. and Susan F. Smith Campus Center, Suite 727, 1350 Massachusetts Avenue, Cambridge, Massachusetts 02138 ("Harvard").

WHEREAS, certain Biological Material (as defined below) was developed in research conducted by Harvard researcher [**];

WHEREAS, Harvard is committed to the policy that ideas or creative works produced at Harvard should be used for the greatest possible public benefit, and believes that every reasonable incentive should be provided for the prompt introduction of such ideas into public use, all in a manner consistent with the public interest;

WHEREAS, Licensee desires to obtain a non-exclusive license to use the Biological Material and associated Technology Transfer Material (as defined below) to produce Viruses (as defined below) and to use Viruses to manufacture Products for sale; and

WHEREAS, Harvard desires to grant such a license to Licensee in accordance with the terms and conditions of this Agreement;

NOW, THEREFORE, the parties hereto, intending to be legally bound, hereby agree as follows:

1. Definitions.

Whenever used in this Agreement with an initial capital letter, the terms defined in this Article 1, whether used in the singular or the plural, will have the meanings specified below.

1.1. "Affiliate" means, with respect to a person, organization or entity, any person, organization or entity controlling, controlled by or under common control with, such person, organization or entity. For purposes of this definition only, "control" of another person, organization or entity will mean the possession, directly or indirectly, of the power to direct or cause the direction of the activities, management or policies of such person, organization or entity, whether through the ownership of voting securities, by contract or otherwise. Without limiting the foregoing, control will be presumed to exist when a person, organization or entity (a) owns or directly controls fifty percent (50%) or more of the outstanding voting stock or other ownership interest of the other organization or entity or (b) possesses, directly or indirectly, the

power to elect or appoint fifty percent (50%) or more of the members of the governing body of the other organization or entity. The parties acknowledge that in the case of certain entities organized under the laws of certain countries outside of the United States, the maximum percentage ownership permitted by law for a foreign investor may be less than fifty percent (50%), and that in such cases such lower percentage will be substituted in the preceding sentence.

1.2. "Aggregate Consideration" means the amount equal to:

1.2.1. in the case of an Asset Sale, the sum of (a) all cash and the fair market value of all securities or other property transferred to Licensee or Licensee's direct or indirect parent company at the time of the transaction, less all current and long-term liabilities (but not contingent liabilities) of Licensee that are not discharged or assumed by the buyer (or its affiliates) in connection with the Asset Sale and (b) all cash and the fair market value of all securities and other property for Trailing Consideration payable to Licensee or Licensee's direct or indirect parent company, when and if, actually paid; or

1.2.2. in the case of a Merger or Stock Sale, the sum of (a) all cash and the fair market value of all securities and other property transferred to the stockholders of Licensee or Licensee's direct or indirect parent company (and any option holders or warrant holders) in return for their stock (or options or warrants) in Licensee or Licensee's direct or indirect parent company at the time of the transaction and (b) all cash and the fair market value of all securities and other property transferred to the stockholders of Licensee or Licensee's direct or indirect parent company (and any option holders or warrant holders) for Trailing Consideration payable to the holders of Licensee's or Licensee's direct or indirect parent company's securities, when and if actually paid.

The valuation of any securities or other property shall be determined by reference to the operative transaction agreement for a respective Merger, Stock Sale or Asset Sale; provided that, if no such valuation is readily determinable from such operative transaction agreement, then for securities for which there is an active public market:

(a) if traded on a securities exchange or the NASDAQ Stock Market, the value shall be deemed to be the average of the closing prices of the securities on such exchange or market over the 30-period ending three days prior to the closing of such transaction; or

(b) if actively traded over-the-counter, the value shall be deemed to be the average of the closing bid prices over the 30-day period ending three days prior to the closing of such transaction.

The method of valuation of securities subject to investment letters or other similar restrictions on free marketability shall take into account an appropriate discount from the market value as determined pursuant to clause (a) or (b) immediately above so as to reflect the approximate fair market value thereof.

For securities for which there is no active public market, the value shall be the fair market value thereof as either (a) determined in good faith by the board of directors of Licensee or Licensee's direct or indirect parent company, as the case may be, (b) approved by

Harvard, such approval not to be unreasonably withheld or (c) determined by a third party appraiser appointed and paid for by Licensee.

1.3. "Biological Material" means any material listed in Exhibit 1.3 to this Agreement, together with all progeny, mutants, replicates and derivatives (modified or unmodified) thereof; provided, however that in no event shall a Virus or a Product be deemed Biological Material.

1.4. "Calendar Quarter" means each of the periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 and December 31 during the Term.

1.5. "Change of Control" means (a) a merger, share exchange or other reorganization in which Licensee (or its successor) is a constituent party **("Merger"),** (b) the acquisition, in a single transaction or series of related transactions, by a person or entity or a group of related persons or entities, of a majority of the voting power of Licensee (or such successor) from persons holding (either directly or indirectly) securities of Licensee (or such successor) (**"Stock Sale"**), or (c) the sale, lease, transfer, or other disposition, in a single transaction or series of related transactions of all or substantially all of the assets of Licensee (or such successor) (or that portion of its assets related to the subject matter of this Agreement)(**"Asset Sale")**, in which, for each of (a), (b) and (c), the security holders of Licensee (or such successor) that control a majority of the voting power of Licensee (or such successor) prior to such transaction do not control, directly or indirectly, a majority of the voting power of the acquiring, surviving or successor entity, as the case may be; provided however, that (1) a transaction in which working capital is raised through the non-public issuance of equity in Licensee to investors shall not constitute a "Change of Control" and (2) Licensee's merger, combination or other transaction with its direct or indirect parent company or other Affiliate of Licensee shall not constitute a "Change of Control".

1.6. "FDA" means the United States Food and Drug Administration.

1.7. "Field" means the treatment of Duchenne Muscular Dystrophy.

1.8. "Major Country" means the United States, Japan, Germany, Italy, Spain, France and the United Kingdom, or the European Union, as a whole.

1.9. "Market Countries" means:

(a) All current and future Organization for Economic Cooperation and Development (OECD) countries, presently consisting of Australia, Austria, Belgium, Canada, Czech Republic, Denmark, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Italy, Republic of Korea, Japan, Luxembourg, Mexico, the Netherlands, New Zealand, Norway, Poland, Portugal, Spain, Sweden, Switzerland, Turkey, the UK, and the United States; and

(b) All current and future members of the European Union not otherwise members of the OECD; and

(c) People's Republic of China, India, Malaysia, Russian Federation, Singapore, and Taiwan.

1.10. "Marketing Approval" means all approvals from the relevant Regulatory Authority of a Major Country necessary to market and sell a Product in such country or territory.

1.11. "Net Sales" means the gross amount billed or invoiced by or on behalf of Licensee or its Sublicensees (in each case, the "Invoicing Entity") on sales, leases or other transfers of Products, less the following to the extent applicable with respect to such sales, leases or other transfers and not previously deducted from the gross invoice price: (a) customary trade, quantity or cash discounts to the extent actually allowed and taken; (b) amounts actually repaid or credited by reason of rejection or return of any previously sold, leased or otherwise transferred Products; (c) customer freight, shipping, transportation, delivery, packaging and/or cost of insurance prepaid charges that are paid or actually allowed by or on behalf of the Invoicing Entity; and (d) to the extent separately stated on purchase orders, invoices or other documents of sale, any sales, use, value added or similar taxes, tariffs, custom duties or other similar governmental charges levied directly on the production, sale, transportation, delivery or use of a Product that are paid by or on behalf of the Invoicing Entity, but not including any tax levied with respect to income; provided that:

1.11.1.in any transfers of Products between an Invoicing Entity and an Affiliate of such Invoicing Entity not for the purpose of resale by such Affiliate, Net Sales will be equal to the fair market value of the Products so transferred, assuming an arm's length transaction made in the ordinary course of business, and

1.11.2.in the event that an Invoicing Entity receives non-cash consideration for any Products or in the case of transactions not at arm's length with a non-Affiliate of an Invoicing Entity, Net Sales will be calculated based on the fair market value of such consideration or transaction, assuming an arm's length transaction made in the ordinary course of business;

1.11.3.sales of Products to Public Sector entities in Non-Suit Countries for end use solely in Non-Suit Countries will not be deemed Net Sales; and

1.11.4.sales of Products by an Invoicing Entity to its Affiliate or a Sublicensee for resale by such Affiliate or Sublicensee will not be deemed Net Sales. Instead, Net Sales will be determined based on the gross amount billed or invoiced by such Affiliate or Sublicensee upon resale of such Products to a third party purchaser.

1.12. "Non-Royalty Income" means any payments or other consideration that Licensee or any of its Affiliates receives in connection with a Sublicense or Strategic Partnership, including without limitation, fees, milestone payments, agreement maintenance fees, and other payments, but specifically excluding (a) royalties based on Net Sales, (b) amounts received from a Sublicensee or Strategic Partner to cover reasonable, fully-burdened costs incurred or to be incurred by Licensee in the performance of research or development activities after the Effective Date, (c) amounts received from a Sublicensee or Strategic Partner as reimbursement for out-of- pocket costs incurred by Licensee in the preparation, filing, prosecution and maintenance of the Patent Rights, or (d) consideration for the issuance of equity interests in Licensee to the extent the amount paid for such equity does not exceed its fair market value. If Licensee or its Affiliate receives non-cash consideration in connection with a Sublicense or Strategic Partnership, or in the case of transactions not at arm's length,

Non-Royalty Income will be calculated based on the fair market value of such consideration or transaction, at the time of the transaction, assuming an arm's length transaction made in the ordinary course of business. To the extent Licensee receives compensation for both a grant of a Sublicense of rights to the Biological Material and/or the Technology Transfer Material under Section 2.1 and the grant of other rights or licenses to intellectual property other than a Sublicense of rights granted under Section 2.1, such compensation will be reasonably apportioned between that amount attributable to the Sublicense of rights under Section 2.1, which shall be deemed Non-Royalty Income, and that amount attributable to the grant of other rights or licenses in such other intellectual property, which shall be excluded from Non-Royalty Income, such apportionment to be reasonably agreed upon by the Parties.

1.13. "Non-Suit Countries" means all countries other than Market Countries.

1.14. "Product" means any product produced by a Virus or through the use of the Biological Material or Technology Transfer Material.

1.15. "Public Sector" will include:

(a) the sovereign government of a country;

(b) agencies of the United Nations and the World Health Organization;

(c) non-profit organizations which are members of the International Committee of the Red Cross and Red Crescent;

(d) international charitable agencies (also known as Non-Governmental Agencies) including but not limited to Oxfam, Medecins Sans Frontieres, and so forth;

(e) non-profit organizations substantially supported by philanthropic organizations including but not limited to the Bill and Melinda Gates Foundation, the Rockefeller Foundation and so forth, specifically including global product development and distribution public-private partnerships.

1.16. "Regulatory Authority" means any applicable government regulatory authority involved in granting approvals for the manufacturing and marketing of a Product, including, in the United States, the FDA.

1.17. "Strategic Partner" means any entity that agrees to compensate Licensee or its Affiliate in exchange for: Licensee's or its Affiliate's practice of the Patent Rights and/or development of Products, on behalf of or in collaboration with such entity, including without limitation, for commercialization and development activities for Products. Any entity which meets the foregoing criteria, that also receives a Sublicense shall be considered a Sublicensee, and not a Strategic Partner, for the purposes of this Agreement.

1.18. "Strategic Partnership" means any agreement with a Strategic Partner.

1.19. "Sublicense" shall mean (a) any right granted, license given or agreement entered into by Licensee to or with any other person or entity (including strategic or development

partnerships), under or with respect to or permitting any use or exploitation of the Biological Material and/or the Technology Transfer Material for the purpose of producing Viruses to be used in the production of Products, or otherwise permitting the development, manufacture, marketing, distribution, use and/or sale of Products; (b) any option or other right granted by Licensee to any other person or entity to negotiate for or receive any of the rights described under clause (a); or (c) any standstill or similar obligation undertaken by Licensee toward any other person or entity not to grant any of the rights described in clause (a) or (b) to any third party; in each case regardless of whether such grant of rights, license given or agreement entered into is referred to or is described as a sublicense.

1.20. "Sublicensee" shall mean any person or entity granted a Sublicense.

1.21. "Technology Transfer Material" means the methods, protocols and other information listed in Exhibit 1.21 hereto.

1.22. "Term" means the term of this Agreement as set forth in Section 6.1.

1.23. "Third Party" means any person or entity other than Harvard, Licensee and Licensee's Affiliates.

1.24. "Trailing Consideration" means any payments due for any deferred or contingent aggregate consideration payable to Licensee, its direct or indirect parent company, or its security holders, as the case may be, with respect to an Asset Sale, Merger or Stock Sale, including, without limitation, any post-closing milestone payment, escrow or holdback of consideration.

1.25. "Virus" means any virus produced by Licensee or a Sublicensee through use of the Biological Material that does not contain any Biological Material or any functional portion or functional fragment thereof.

2. License.

2.1. License Grant. Subject to the terms and conditions set forth in this Agreement, Harvard hereby grants to Licensee a non-exclusive, royalty-bearing, worldwide license, sublicensable solely in accordance with Section 2.2, to use the Biological Material and the Technology Transfer Material solely to produce Viruses solely to make and sell Products for use in the Field.

2.2. Sublicenses.

2.2.1.Sublicense Grant. Licensee will be entitled to grant Sublicenses to Third Parties under the license granted pursuant to Section 2.1 subject to the terms of this Section 2.2; provided that in each case such grant is made (a) in conjunction with a license to technology owned or controlled by Licensee (other than the Biological Material and the Technology Transfer Material) that is included in or useful for the making of Products, and (b) solely for the manufacture of Viruses solely to make and sell Products for use in the Field. Affiliates of Licensee shall be permitted to exercise such right as a Sublicensee only with Harvard's prior

written consent, not to be unreasonably withheld or delayed; and provided, further, that Licensee shall ensure that any such Affiliate complies with the terms of this Section 2.2.

2.2.2.Sublicense Agreements. Sublicenses shall be granted pursuant to written agreements, which will be subject and subordinate to the terms and conditions of this Agreement. Such Sublicense agreements will contain, among other things, the following:

2.2.2.1.all provisions necessary to ensure Licensee's ability to perform its obligations under this Agreement;

2.2.2.2.a section substantially the same as Article 5 of this Agreement, which also will state that the Indemnitees (as defined in Section 5.1) are intended third party beneficiaries of such Sublicense agreement for the purpose of enforcing such indemnification;

2.2.2.3.a provision clarifying that, in the event of termination of the license set forth in Section 2.1 (in whole or in part), any existing Sublicense agreement shall terminate to the extent of such terminated license; provided, however, that, for each Sublicensee, upon termination of the license, if the Sublicensee is not then in breach of the Sublicense agreement such that Licensee would have the right to terminate such Sublicense agreement, such Sublicensee shall have the right to obtain a direct license from Harvard on the same terms and conditions as set forth herein, which direct license shall not impose any representations, warranties, obligations or liabilities on Harvard that are not included in this Agreement;

2.2.2.4.a provision clarifying that the Sublicensee shall only be entitled to sublicense its rights under such Sublicense agreement on the terms set forth in this Section 2.2; and

2.2.2.5.a provision prohibiting the Sublicensee from assigning the Sublicense agreement without the prior written consent of Harvard, except that Sublicensee may assign the Sublicense agreement to a successor in connection with the merger, consolidation or other reorganization of the Sublicensee, or the sale of all or substantially all of its assets or that portion of its business to which the Sublicense agreement relates; provided, however, that any permitted assignee agrees in writing to be bound by the terms of such Sublicense agreement.

2.2.3.Delivery of Sublicense Agreement. Licensee shall furnish Harvard with a fully executed copy of any Sublicense agreement, redacted with respect to matters not relevant to Harvard's interest, promptly after its execution (or promptly after the Effective Date with respect to Sublicense agreements entered into prior to the Effective Date). Harvard shall keep all such agreements and their terms confidential and shall use them solely for the purpose of monitoring Licensee's and Sublicensees' compliance with their obligations hereunder and enforcing Harvard's rights under this Agreement.

2.2.4.Breach by Sublicensee. During the term of this Agreement, Licensee shall be responsible for any breach of a Sublicense agreement by a Sublicensee that results in a material breach of this Agreement. Licensee may elect (a) to cure such breach in accordance with Section 6.2.2 of this Agreement or (b) to enforce its rights by terminating such Sublicense agreement in accordance with the terms thereof.

2.3. Biological Material.

2.3.1. As between the parties hereto, subject to the the terms herein, including without limitation, the license granted to Licensee pursuant to Section 2.1 and any Sublicenses granted to Sublicensees pursuant to Section 2.2, all rights, title and interest in and to all Biological Material, and any intellectual property applying thereto, shall be owned solely and exclusively by Harvard.

2.3.2. Licensee shall not use Biological Material other than in accordance with the rights expressly granted to it hereunder. Except in connection with Sublicenses granted pursuant to Section 2.2, Licensee shall not sell or otherwise transfer any Biological Material to any third party.

2.3.3. The Biological Material is provided only for use in animals or *in vitro.* THE BIOLOGICAL MATERIAL SHALL NOT BE USED IN HUMANS.

2.3.4. Licensee shall inform Harvard's Office of Technology Development (at 1350 Massachusetts Avenue, Richard A. and Susan F. Smith Campus Center, Suite 727, Cambridge, MA 02138, (617) 495-3067, Attn: Chief Technology Development Officer) of any Biological Material created by Licensee that is different from, and a modification to, the Biological Material listed in Exhibit 1.3, and upon Harvard's request, shall provide samples of such material to Harvard.

2.3.5. Licensee shall deliver to Harvard between [**] and [**] of the product of Licensee's expansion of the Biological Material, at Licensee's expense, no later than August 15, 2016.

2.3.6. As between the parties hereto, all right, title and interest in and to all Viruses, Products, and any intellectual property applying thereto or to the production thereof, shall be owned solely and exclusively by Licensee. For the avoidance of doubt, nothing herein prohibits or is intended to prohibit the use of Products in humans.

2.4. No Other Grant of Rights. Except as expressly provided herein, nothing in this Agreement will be construed to confer any ownership interest, license or other rights upon Licensee by implication, estoppel or otherwise as to any technology, intellectual property rights, products or biological materials of Harvard, or any other entity, regardless of whether such technology, intellectual property rights, products or biological materials are dominant, subordinate or otherwise related to any Biological Material or Technology Transfer Material.

3. Consideration for Grant of License.

3.1. License Issuance Fee. Within thirty (30) days after the Effective Date, Licensee shall pay Harvard a non-refundable license issuance fee in the amount of [**]. Such license issuance fee shall be creditable against any royalty amounts payable under Section 3.3 below with respect to Products sold in calendar year 2016.

3.2. License Maintenance Fee. Licensee shall pay Harvard a non-refundable annual license maintenance fee as follows: [**] for each (full or partial) calendar year prior to Marketing Approval; [**] for the first full calendar year after Marketing Approval; [**] for the

second full calendar year after Marketing Approval; and [**] for the third full calendar year after Marketing Approval and each calendar year thereafter; provided, that if Licensee grants rights to the Biological Material and/or the Technology Transfer Material to a Sublicensee or a Strategic Partner, the annual maintenance fee payable to Harvard as set forth above shall thereafter be [**]. Each such annual maintenance fee shall be due and payable on January 2nd of the calendar year to which such fee applies. Each annual license maintenance fee shall be creditable against any royalty amounts payable under Section 3.3 below with respect to Products sold in the same calendar year that such annual license maintenance fee applies.

3.3. Royalty on Net Sales. Licensee shall pay Harvard an amount equal to (i) [**] of Net Sales with respect to the first [**] of cumulative Net Sales, and (ii) [**] of Net Sales with respect to cumulative Net Sales in excess of [**].

3.4. Non-Royalty Income Fee. Licensee will pay Harvard an amount equal to [**] of all Non-Royalty Income.

3.5. Milestone Payments. With respect to each Product, Licensee will pay Harvard [**] within [**] after first [**] of such Product.

3.6. Change of Control Payment. [**]

3.7. Late Payment. Any payment by Licensee that is not paid on or before the date such payment is due under this Agreement will bear interest at the lower of (a) [**] and (b) the maximum rate allowed by law. Interest will accrue beginning on the first day following the due date for payment and will be compounded quarterly. Payment of such interest by Licensee shall not limit, in any way, Harvard's right to exercise any other remedies Harvard may have as a consequence of the lateness of any payment.

3.8. Payment Method. Each payment due to Harvard under this Agreement shall be paid by check or wire transfer of funds to Harvard's account in accordance with written instructions provided by Harvard. If made by wire transfer, such payments shall be marked so as to refer to this Agreement. All payments due under this Agreement will be paid in U.S. Dollars. Conversion of foreign currency to U.S. Dollars will be made at the conversion rate existing in the United States (as reported in the *Wall Street Journal*) on the last working day of the applicable Calendar Quarter. Such payments will be without deduction of exchange, collection or other charges.

3.9. Reporting. Within thirty (30) days after the conclusion of each Calendar Quarter commencing with the first Calendar Quarter in which Net Sales are generated or Non-Royalty Income is received, Licensee shall deliver to Harvard a report containing the following information (in each instance, with a Product-by-Product breakdown):

3.9.1.the number of units of Products sold, leased or otherwise transferred by Invoicing Entities for the applicable Calendar Quarter (with a Product-by-Product breakdown);

3.9.2.the gross amount billed or invoiced for Products sold, leased or otherwise transferred by Invoicing Entities during the applicable Calendar Quarter;

3.9.3.a calculation of Net Sales for the applicable Calendar Quarter, including an itemized listing of allowable deductions;

3.9.4.a detailed accounting of all Non-Royalty Income received during the applicable Calendar Quarter; and

3.9.5.the total amount payable to Harvard in U.S. Dollars on Net Sales and Non- Royalty Income for the applicable Calendar Quarter, together with the exchange rates used for conversion.

Each such report shall be certified on behalf of Licensee as true, correct and complete in all material respects. If no amounts are due to Harvard for a particular Calendar Quarter, the report shall so state.

3.10. Records. Licensee shall maintain, and shall cause its Affiliates and Sublicensees to maintain, complete and accurate records of Products that are made, used, sold, leased or transferred under this Agreement, any amounts payable to Harvard in relation to such Products, and all Non-Royalty Income received by Licensee and its Affiliates, which records shall contain sufficient information to permit Harvard to confirm the accuracy of any reports or notifications delivered to Harvard under Section 3.9. Licensee, its Affiliates and/or its Sublicensees, as applicable, shall retain such records relating to a given Calendar Quarter for at least five (5) years after the conclusion of that Calendar Quarter, during which time Harvard will have the right, at its expense, to cause an independent, certified public accountant (or, in the event of a non-financial audit, other appropriate auditor) to inspect such records during normal business hours for the purposes of verifying the accuracy of any reports and payments delivered under this Agreement and Licensee's compliance with the terms hereof. Such accountant or other auditor, as applicable, shall not disclose to Harvard any information other than information relating to the accuracy of reports and payments delivered under this Agreement. The parties shall reconcile any underpayment or overpayment within thirty (30) days after the accountant delivers the results of the audit. If any audit performed under this Section 3.10 reveals an underpayment in excess of [**] in any calendar year, Licensee shall reimburse Harvard for all amounts incurred in connection with such audit. Harvard may exercise its rights under this Section 3.10 only once every year per audited entity and only with reasonable prior notice to the audited entity.

4. Warranties; Limitation of Liability.

4.1. Compliance with Law. Licensee represents and warrants that it will comply, and that it will ensure that its Affiliates comply, with all local, state and international laws and regulations relating to the Biological Material and to the development, manufacture, use, sale and importation of Viruses and Products. Without limiting the foregoing, Licensee represents and warrants that it will comply with all United States export control laws and regulations with respect to Biological Material and any Viruses and Products developed or made through the use thereof.

4.2. Harvard Representations and Warranties. Harvard hereby represents and warrants to Licensee that Harvard has the authority to grant the license herein to the

Biological Material and the Technology Transfer Material and Harvard has not granted to any Third Party any rights that would conflict with this Agreement.

4.3. No Warranty.

4.3.1.HARVARD MAKES NO REPRESENTATIONS OR WARRANTIES WHATSOEVER AS TO THE COMMERCIAL OR SCIENTIFIC VALUE OF THE BIOLOGICAL MATERIAL. HARVARD MAKES NO REPRESENTATION OR WARRANTY THAT THE USE OF THE BIOLOGICAL MATERIAL, TECHNOLOGY TRANSFER MATERIAL, OR THE DEVELOPMENT, MANUFACTURE, USE, SALE OR IMPORTATION OF ANY VIRUS OR PRODUCT, OR ANY ELEMENT THEREOF, WILL NOT INFRINGE THE PATENT OR PROPRIETARY RIGHTS OF ANY THIRD PARTY.

4.3.2.EXCEPT AS OTHERWISE EXPRESSLY PROVIDED IN THIS AGREEMENT, NEITHER PARTY MAKES ANY WARRANTY WITH RESPECT TO ANY TECHNOLOGY, PATENTS, MATERIALS (INCLUDING BIOLOGICAL MATERIAL), GOODS, SERVICES, RIGHTS, TECHNOLOGY TRANSFER MATERIAL OR OTHER SUBJECT MATTER OF THIS AGREEMENT AND EACH PARTY HEREBY DISCLAIMS WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE AND NONINFRINGEMENT WITH RESPECT TO ANY AND ALL OF THE FOREGOING.

4.3.3.ALL BIOLOGICAL MATERIAL IS EXPERIMENTAL IN NATURE AND SHALL BE USED WITH PRUDENCE AND APPROPRIATE CAUTION SINCE NOT ALL OF THEIR CHARACTERISTICS ARE KNOWN.

4.4. Limitation of Liability.

4.4.1. Except with respect to matters for which Licensee is obligated to indemnify Harvard under Article 5, neither party will be liable to the other with respect to any subject matter of this Agreement under any contract, negligence, strict liability or other legal or equitable theory for (a) any indirect, incidental, consequential or punitive damages or lost profits or (b) cost of procurement of substitute goods, technology or services.

4.4.2. Harvard's aggregate liability for all damages of any kind arising out of or relating to this Agreement or its subject matter under any contract, negligence, strict liability or other legal or equitable theory shall not exceed the amounts paid to Harvard under this Agreement.

5. Indemnification and Insurance.

5.1. Indemnity.

5.1.1. Licensee shall indemnify, defend and hold harmless Harvard and its current and former directors, governing board members, trustees, officers, faculty, medical and professional staff, employees, students, and agents and their respective successors, heirs and assigns (collectively, the "Indemnitees") from and against any claim, liability, cost, expense, damage, deficiency, loss or obligation of any kind or nature (including reasonable attorneys' fees and other costs and expenses of litigation) by or owed to a third party, based upon, arising out of, or otherwise relating to the activities of Licensee and Sublicensees under this Agreement, including

any cause of action relating to product liability concerning any product, process, or service made, used, sold or performed pursuant to any right or license granted under this Agreement (collectively, the "Claims"); provided, however that Licensee's indemnification obligations hereunder shall not apply to any Claim to the extent that it is attributable to the gross negligence or willful misconduct of any Indemnitee.

5.1.2. Licensee shall, at its own expense, provide attorneys reasonably acceptable to Harvard to defend against any actions brought or filed against any Indemnitee hereunder with respect to the subject of indemnity contained herein, whether or not such actions are rightfully brought. Any Indemnitee seeking indemnification hereunder shall promptly notify Licensee of such Claim; provided further that any failure of or delay in such notification shall not affect Licensee's indemnification obligation unless and to the extent such failure or delay is materially prejudicial to Licensee. The Indemnitees shall provide Licensee, at Licensee's expense, with reasonable assistance and full information with respect to such Claim and give Licensee sole control of the defense of any Claim. Neither Licensee nor Harvard shall settle any Claim without the prior written consent of the other, which consent shall not be unreasonably withheld.

5.2. Insurance.

5.2.1. Beginning at the time any Product is being commercially distributed or sold by Licensee, or by an Affiliate or agent of Licensee, Licensee shall, at its sole cost and expense, procure and maintain commercial general liability insurance in amounts not less than [**] per incident and [**] annual aggregate and naming the Indemnitees as additional insureds. Such commercial general liability insurance shall provide: (a) product liability coverage and (b) broad form contractual liability coverage for Licensee's indemnification obligations under this Agreement.

5.2.2. If Licensee elects to self-insure all or part of the limits described above in Section 5.2.1 (including deductibles or retentions that are in excess of [**] annual aggregate) such self-insurance program must be acceptable to Harvard and CRICO/RMF (Harvard's insurer) in their sole discretion. The minimum amounts of insurance coverage required shall not be construed to create a limit of Licensee's liability with respect to its indemnification obligations under this Agreement.

5.2.3. Licensee shall provide Harvard with written evidence of such insurance upon request of Harvard. Licensee shall provide Harvard with written notice at least fifteen (15) days prior to the cancellation, non-renewal or material change in such insurance. If Licensee does not obtain replacement insurance providing comparable coverage within thirty (30) days after such notice, Harvard shall have the right to terminate this Agreement effective at the end of such thirty (30) day period without notice or any additional waiting periods.

5.2.4. Licensee shall maintain such commercial general liability insurance beyond the expiration or termination of this Agreement during: (a) the period that any Product is being commercially distributed or sold by Licensee, or an Affiliate or agent of Licensee; and (b) a reasonable period after the period referred to in (a) above which in no event shall be less than [**].

6. Term and Termination.

6.1. Term. The term of this Agreement shall commence on the Effective Date and, unless earlier terminated as provided in this Article 6, shall continue in full force and effect for fifteen (15) years thereafter (the "Initial Term"). After the Initial Term, this Agreement shall renew for successive three-year periods (the Initial Term and such period, the "Term") unless either Party gives the other Party written notice of its desire to terminate the Agreement no less than sixty (60) days prior to the expiration of the Initial Term or any applicable successive period during the Term.

6.2. Termination.

6.2.1.Termination Without Cause. Licensee may terminate this Agreement upon sixty (60) days prior written notice to Harvard.

6.2.2.Termination for Default.

6.2.2.1. In the event that either party commits a material breach of its obligations under this Agreement and fails to cure that breach within ninety (90) days after receiving written notice thereof, the other party may terminate this Agreement immediately upon written notice to the party in breach.

6.2.2.2. If Licensee defaults in its obligations under Section 5.2 to procure and maintain insurance or, if Licensee has in any event failed to comply with the notice requirements contained therein and fails to cure that default within thirty (30) days after receiving written notice thereof, Harvard may terminate this Agreement immediately upon written notice to Licensee.

6.2.3.Bankruptcy. Harvard may terminate this Agreement upon notice to Licensee if Licensee becomes insolvent, is adjudged bankrupt, applies for judicial or extra-judicial settlement with its creditors, makes an assignment for the benefit of its creditors, voluntarily files for bankruptcy or has a receiver or trustee (or the like) in bankruptcy appointed by reason of its insolvency, or in the event an involuntary bankruptcy action is filed against Licensee and not dismissed within ninety (90) days, or if Licensee becomes the subject of liquidation or dissolution proceedings or otherwise discontinues business.

6.3. Effect of Termination.

6.3.1.Termination of Rights. Upon any termination or expiration of this Agreement, (a) the rights and licenses granted to Licensee under Article 2 shall terminate, (b) all rights in and to and under the Biological Material and Technology Transfer Material will revert to Harvard and, subject to Section 6.3.2, neither Licensee nor its Sublicensees may make any further use or exploitation of any Biological Material or Technology Transfer Material and (c) any existing Sublicense shall terminate; provided, however, that, for each Sublicensee, upon termination of the license, if the Sublicensee is not then in breach of the Sublicense agreement such that Licensee would have the right to terminate such Sublicense agreement, such Sublicensee shall have the right to obtain a direct license from Harvard on the same terms and conditions as set forth herein, which shall not impose any representations, warranties, obligations or liabilities on

Harvard that are not included in this Agreement. Furthermore, in the event of any termination or expiration of this Agreement, Licensee shall destroy, and shall cause its agents and Sublicensees to destroy, all Biological Material under their control or in their possession.

6.3.2.Accruing Obligations. Termination or expiration of this Agreement shall not relieve the parties of obligations accruing prior to such termination or expiration, including obligations to pay amounts accruing hereunder up to the date of termination or expiration. After the date of termination or expiration (except in the case of termination by Harvard pursuant to Section 6.2), Licensee and its Sublicensees (a) may sell Products then in stock and (b) may complete the production of Products then in the process of production and sell the same; provided that, in the case of both (a) and (b), Licensee shall maintain insurance in accordance with the requirements of Section 5.2.

6.4. Survival. The parties' respective rights, obligations and duties under Articles 4, 5 and 7 and Sections 2.2.2.3 (but only with respect to a Sublicensee's right to obtain a direct license from Harvard), 2.3, 3.1, 3.6, 6.3, 6.4, as well as any rights, obligations and duties which by their nature extend beyond the expiration or termination of this Agreement, shall survive any expiration or termination of this Agreement.

7. Miscellaneous.

7.1. No Security Interest. Licensee shall not enter into any agreement under which Licensee grants to or otherwise creates in any third party a security interest in this Agreement or any of the rights granted to Licensee herein. Any grant or creation of a security interest purported or attempted to be made in violation of the terms of this Section 7.1 shall be null and void and of no legal effect.

7.2. Use of Name. Except as provided below, Licensee shall not, and shall ensure that its Affiliates and Sublicensees shall not, use or register the name "Harvard" (alone or as part of another name) or any logos, seals, insignia or other words, names, symbols or devices that identify Harvard or any Harvard school, unit, division or affiliate ("Harvard Names") for any purpose except with the prior written approval of, and in accordance with restrictions required by, Harvard. Without limiting the foregoing, Licensee shall, and shall ensure that its Affiliates and Sublicensees shall, cease all use of Harvard Names on the termination or expiration of this Agreement except as otherwise approved by Harvard. This restriction shall not apply to any information required by law to be disclosed to any governmental entity.

7.3. Entire Agreement. This Agreement is the sole agreement with respect to the subject matter hereof and except as expressly set forth herein, supersedes all other agreements and understandings between the parties with respect to the same.

7.4. Notices. Unless otherwise specifically provided, all notices required or permitted by this Agreement shall be in writing and may be delivered personally, or may be sent by facsimile, overnight delivery or certified mail, return receipt requested, to the following addresses, unless the parties are subsequently notified of any change of address in accordance with this Section 7.4:

If to Licensee (invoices only):	Solid GT, LLC 161 First Street, Suite #300 Cambridge, MA 02142 Fax: Email: Phone: Attn:
If to Licensee (all other notices):	Solid GT, LLC 161 First Street, Suite #300 Cambridge, MA 02142 Fax: Email: Phone: Attn:
If to Harvard:	Office of Technology Development Harvard University Richard A. and Susan F. Smith Campus Center 727 1350 Massachusetts Avenue Cambridge, Massachusetts 02138 Fax: Attn.: Chief Technology Development Officer

Any notice shall be deemed to have been received as follows: (a) by personal delivery, upon receipt; (b) by facsimile or overnight delivery, one business day after transmission or dispatch; (c) by certified mail, as evidenced by the return receipt. If notice is sent by facsimile, a confirming copy of the same shall be sent by mail to the same address.

7.5. Governing Law and Jurisdiction. This Agreement will be governed by, and construed in accordance with, the substantive laws of the Commonwealth of Massachusetts, without giving effect to any choice or conflict of law provision, except that questions affecting the construction and effect of any patent shall be determined by the law of the country in which the patent shall have been granted. Any action, suit or other proceeding arising under or relating to this Agreement (a "Suit") shall be brought in a court of competent jurisdiction in the Commonwealth of Massachusetts, and the parties hereby consent to the sole jurisdiction of the state and federal courts sitting in the Commonwealth of Massachusetts. Each party agrees not to raise any objection at any time to the laying or maintaining of the venue of any Suit in any of the specified courts, irrevocably waives any claim that Suit has been brought in any inconvenient forum and further irrevocably waives the right to object, with respect to any Suit, that such court does not have any jurisdiction over such party.

7.6. Binding Effect. This Agreement shall be binding upon and inure to the benefit of the parties and their respective legal representatives, successors and permitted assigns.

7.7. Headings. Section and subsection headings are inserted for convenience of reference only and do not form a part of this Agreement.

7.8. Counterparts. The parties may execute this Agreement in two or more counterparts, each of which shall be deemed an original, but both of which together shall constitute one and the same instrument. Transmission by facsimile or electronic mail of an executed counterpart of this Agreement shall be deemed to constitute due and sufficient delivery of such counterpart. If by electronic mail, the executed Agreement must be delivered in a .pdf format.

7.9. Amendment; Waiver. This Agreement may be amended, modified, superseded or canceled, and any of the terms may be waived, only by a written instrument executed by each party or, in the case of waiver, by the party waiving compliance. The delay or failure of either party at any time or times to require performance of any provisions hereof shall in no manner affect the rights at a later time to enforce the same. No waiver by either party of any condition or of the breach of any term contained in this Agreement, whether by conduct, or otherwise, in any one or more instances, shall be deemed to be, or considered as, a further or continuing waiver of any such condition or of the breach of such term or any other term of this Agreement.

7.10. No Agency or Partnership. Nothing contained in this Agreement shall give either party the right to bind the other, or be deemed to constitute either party as agent for or partner of the other or any third party.

7.11. Assignment and Successors. This Agreement may not be assigned by either party without the consent of the other, which consent shall not be unreasonably withheld, except that each party may, without such consent, assign this Agreement and the rights, obligations and interests of such party to any purchaser of all or substantially all of its assets to which the subject matter of this Agreement relates, or to any successor corporation resulting from any merger or consolidation of such party with or into such corporation; provided, in each case, that the assignee agrees in writing to be bound by the terms of this Agreement. Any assignment purported or attempted to be made in violation of the terms of this Section 7.11 shall be null and void and of no legal effect.

7.12. Force Majeure. Except for monetary obligations hereunder, neither party will be responsible for delays resulting from causes beyond the reasonable control of such party, including fire, explosion, flood, war, strike, or riot, provided that the nonperforming party uses commercially reasonable efforts to avoid or remove such causes of nonperformance and continues performance under this Agreement with reasonable dispatch whenever such causes are removed.

7.13. Interpretation. Each party hereto acknowledges and agrees that: (a) it and/or its counsel reviewed and negotiated the terms and provisions of this Agreement and has contributed to its revision; (b) the rule of construction to the effect that any ambiguities are resolved against the drafting party shall not be employed in the interpretation of this Agreement; (c) the terms and provisions of this Agreement shall be construed fairly as to both parties hereto and not in favor of or against either party, regardless of which party was generally responsible

for the preparation of this Agreement; and (d) the use of "include," "includes," or "including" herein shall not be limiting and "or" shall not be exclusive.

7.14. Severability. If any provision of this Agreement is or becomes invalid or is ruled invalid by any court of competent jurisdiction or is deemed unenforceable, it is the intention of the parties that the remainder of this Agreement shall not be affected.

IN WITNESS WHEREOF, the parties have caused this Agreement to be executed by their duly authorized representatives as of the date first written above.

President and Fellows of Harvard College		**Solid GT, LLC**	
By:	/s/ Meghan D. McCollum Fenno	By:	/s/ llan Ganot
Name:	Meghan D. McCollum Fenno	Name:	llan Ganot
Title:	Director of Technology Transactions Office of Technology Development Harvard University	Title:	CEO

Exhibit 10.56

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

LICENSE AGREEMENT

This License Agreement is entered into as of this 3rd day of August, 2017 (the "Effective Date"), by and between **Solid Biosciences, LLC**, a company organized under the laws of Delaware and having an address at 161 First Street, Suite #300, Cambridge, MA 02142 ("Licensee") and **President and Fellows of Harvard College,** an educational and charitable corporation existing under the laws and the constitution of the Commonwealth of Massachusetts, having a place of business at Richard A. and Susan F. Smith Campus Center, Suite 727, 1350 Massachusetts Avenue, Cambridge, Massachusetts 02138 ("Harvard").

WHEREAS, certain Biological Material (as defined below) was developed in research conducted by Harvard researcher [**];

WHEREAS, Harvard is committed to the policy that ideas or creative works produced at Harvard should be used for the greatest possible public benefit, and believes that every reasonable incentive should be provided for the prompt introduction of such ideas into public use, all in a manner consistent with the public interest;

WHEREAS, Licensee desires to obtain a non-exclusive license to use the Biological Material and associated Technology Transfer Material (as defined below) to produce Viruses (as defined below) and to use Viruses to manufacture Products for sale; and

WHEREAS, Harvard desires to grant such a license to Licensee in accordance with the terms and conditions of this Agreement;

NOW, THEREFORE, the parties hereto, intending to be legally bound, hereby agree as follows:

1. Definitions.

Whenever used in this Agreement with an initial capital letter, the terms defined in this Article 1, whether used in the singular or the plural, will have the meanings specified below.

1.1."Affiliate" means, with respect to a person, organization or entity, any person, organization or entity controlling, controlled by or under common control with, such person, organization or entity. For purposes of this definition only, "control" of another person, organization or entity will mean the possession, directly or indirectly, of the power to direct or cause the direction of the activities, management or policies of such person, organization or entity, whether through the ownership of voting securities, by contract or otherwise. Without limiting the foregoing, control will be presumed to exist when a person, organization or entity (a) owns or directly controls fifty percent (50%) or more of the outstanding voting stock or other ownership interest of the other organization or entity or (b) possesses, directly or indirectly, the power to elect or appoint fifty percent (50%) or more of the members of the governing body of

the other organization or entity. The parties acknowledge that in the case of certain entities organized under the laws of certain countries outside of the United States, the maximum percentage ownership permitted by law for a foreign investor may be less than fifty percent (50%), and that in such cases such lower percentage will be substituted in the preceding sentence.

1.2.“Aggregate Consideration” means the amount equal to:

1.2.1 in the case of an Asset Sale, the sum of (a) all cash and the fair market value of all securities or other property transferred to Licensee or Licensee’s direct or indirect parent company at the time of the transaction, less all current and long-term liabilities (but not contingent liabilities) of Licensee that are not discharged or assumed by the buyer (or its affiliates) in connection with the Asset Sale and (b) all cash and the fair market value of all securities and other property for Trailing Consideration payable to Licensee or Licensee’s direct or indirect parent company, when and if, actually paid; or

1.2.2 in the case of a Merger or Stock Sale, the sum of (a) all cash and the fair market value of all securities and other property transferred to the stockholders of Licensee or Licensee’s direct or indirect parent company (and any option holders or warrant holders) in return for their stock (or options or warrants) in Licensee or Licensee’s direct or indirect parent company at the time of the transaction and (b) all cash and the fair market value of all securities and other property transferred to the stockholders of Licensee or Licensee’s direct or indirect parent company (and any option holders or warrant holders) for Trailing Consideration payable to the holders of Licensee’s or Licensee’s direct or indirect parent company’s securities, when and if actually paid.

The valuation of any securities or other property shall be determined by reference to the operative transaction agreement for a respective Merger, Stock Sale or Asset Sale; provided that, if no such valuation is readily determinable from such operative transaction agreement, then for securities for which there is an active public market:

(a) if traded on a securities exchange or the NASDAQ Stock Market, the value shall be deemed to be the average of the closing prices of the securities on such exchange or market over the 30-period ending three days prior to the closing of such transaction; or

(b) if actively traded over-the-counter, the value shall be deemed to be the average of the closing bid prices over the 30-day period ending three days prior to the closing of such transaction.

The method of valuation of securities subject to investment letters or other similar restrictions on free marketability shall take into account an appropriate discount from the market value as determined pursuant to clause (a) or (b) immediately above so as to reflect the approximate fair market value thereof.

For securities for which there is no active public market, the value shall be the fair market value thereof as either (a) determined in good faith by the board of directors of Licensee or Licensee’s direct or indirect parent company, as the case may be, (b) approved by

Harvard, such approval not to be unreasonably withheld or (c) determined by a third party appraiser appointed and paid for by Licensee.

1.3.“Biological Material” means any material listed in Exhibit 1**.3** to this Agreement, together with all progeny, mutants, replicates and derivatives (modified or unmodified) thereof; provided, however that in no event shall a Virus or a Product be deemed Biological Material.

1.4.“Calendar Quarter” means each of the periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 and December 31 during the Term.

1.5.“Change of Control” means (a) a merger, share exchange or other reorganization in which Licensee (or its successor) is a constituent party (“**Merger**”), (b) the acquisition, in a single transaction or series of related transactions, by a person or entity or a group of related persons or entities, of a majority of the voting power of Licensee (or such successor) from persons holding (either directly or indirectly) securities of Licensee (or such successor) **(“Stock Sale”),** or (c) the sale, lease, transfer, or other disposition, in a single transaction or series of related transactions of all or substantially all of the assets of Licensee (or such successor) (or that portion of its assets related to the subject matter of this Agreement)(“**Asset Sale**”), in which, for each of (a), (b) and (c), the security holders of Licensee (or such successor) that control a majority of the voting power of Licensee (or such successor) prior to such transaction do not control, directly or indirectly, a majority of the voting power of the acquiring, surviving or successor entity, as the case may be; provided however, that (1) a transaction in which working capital is raised through the non-public issuance of equity in Licensee to investors shall not constitute a “Change of Control” and (2) Licensee’s merger, combination or other transaction with its direct or indirect parent company or other Affiliate of Licensee shall not constitute a “Change of Control”.

1.6.“FDA” means the United States Food and Drug Administration.

1.7.“Field” means the treatment of Duchenne Muscular Dystrophy.

1.8.“Major Country” means the United States, Japan, Germany, Italy, Spain, France and the United Kingdom, or the European Union, as a whole.

1.9.“Market Countries” means:

(a) All current and future Organization for Economic Cooperation and Development (OECD) countries, presently consisting of Australia, Austria, Belgium, Canada, Czech Republic, Denmark, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Italy, Republic of Korea, Japan, Luxembourg, Mexico, the Netherlands, New Zealand, Norway, Poland, Portugal, Spain, Sweden, Switzerland, Turkey, the UK, and the United States; and

(b) All current and future members of the European Union not otherwise members of the OECD; and

(c) People's Republic of China, India, Malaysia, Russian Federation, Singapore, and Taiwan.

1.10."Marketing Approval" means all approvals from the relevant Regulatory Authority of a Major Country necessary to market and sell a Product in such country or territory.

1.11."Net Sales" means the gross amount billed or invoiced by or on behalf of Licensee or its Sublicensees (in each case, the "Invoicing Entity") on sales, leases or other transfers of Products, less the following to the extent applicable with respect to such sales, leases or other transfers and not previously deducted from the gross invoice price: (a) customary trade, quantity or cash discounts to the extent actually allowed and taken; (b) amounts actually repaid or credited by reason of rejection or return of any previously sold, leased or otherwise transferred Products; (c) customer freight, shipping, transportation, delivery, packaging and/or cost of insurance prepaid charges that are paid or actually allowed by or on behalf of the Invoicing Entity; and (d) to the extent separately stated on purchase orders, invoices or other documents of sale, any sales, use, value added or similar taxes, tariffs, custom duties or other similar governmental charges levied directly on the production, sale, transportation, delivery or use of a Product that are paid by or on behalf of the Invoicing Entity, but not including any tax levied with respect to income; provided that:

1.11.1 in any transfers of Products between an Invoicing Entity and an Affiliate of such Invoicing Entity not for the purpose of resale by such Affiliate, Net Sales will be equal to the fair market value of the Products so transferred, assuming an arm's length transaction made in the ordinary course of business, and

1.11.2 in the event that an Invoicing Entity receives non-cash consideration for any Products or in the case of transactions not at arm's length with a non-Affiliate of an Invoicing Entity, Net Sales will be calculated based on the fair market value of such consideration or transaction, assuming an arm's length transaction made in the ordinary course of business;

1.11.3 sales of Products to Public Sector entities in Non-Suit Countries for end use solely in Non-Suit Countries will not be deemed Net Sales; and

1.11.4 sales of Products by an Invoicing Entity to its Affiliate or a Sublicensee for resale by such Affiliate or Sublicensee will not be deemed Net Sales. Instead, Net Sales will be determined based on the gross amount billed or invoiced by such Affiliate or Sublicensee upon resale of such Products to a third party purchaser.

1.12."Non-Royalty Income" means any payments or other consideration that Licensee or any of its Affiliates receives in connection with a Sublicense or Strategic Partnership, including without limitation, fees, milestone payments, agreement maintenance fees, and other payments, but specifically excluding (a) royalties based on Net Sales, (b) amounts received from a Sublicensee or Strategic Partner to cover reasonable, fully-burdened costs incurred or to be incurred by Licensee in the performance of research or development activities after the Effective Date, (c) amounts received from a Sublicensee or Strategic Partner as reimbursement for out-of-pocket costs incurred by Licensee in the preparation, filing,

prosecution and maintenance of the Patent Rights, or (d) consideration for the issuance of equity interests in Licensee to the extent the amount paid for such equity does not exceed its fair market value. If Licensee or its Affiliate receives non-cash consideration in connection with a Sublicense or Strategic Partnership, or in the case of transactions not at arm's length, Non-Royalty Income will be calculated based on the fair market value of such consideration or transaction, at the time of the transaction, assuming an arm's length transaction made in the ordinary course of business. To the extent Licensee receives compensation for both a grant of a Sublicense of rights to the Biological Material and/or the Technology Transfer Material under Section 2.1 and the grant of other rights or licenses to intellectual property other than a Sublicense of rights granted under Section 2.1, such compensation will be reasonably apportioned between that amount attributable to the Sublicense of rights under Section 2.1, which shall be deemed Non-Royalty Income, and that amount attributable to the grant of other rights or licenses in such other intellectual property, which shall be excluded from Non-Royalty Income, such apportionment to be reasonably agreed upon by the Parties.

1.13.“Non-Suit Countries” means all countries other than Market Countries.

1.14.“Product” means any product produced by a Virus or through the use of the Biological Material or Technology Transfer Material.

1.15.“Public Sector” will include:

(a) the sovereign government of a country;

(b) agencies of the United Nations and the World Health Organization;

(c) non-profit organizations which are members of the International Committee of the Red Cross and Red Crescent;

(d) international charitable agencies (also known as Non-Governmental Agencies) including but not limited to Oxfam, Medecins Sans Frontieres, and so forth;

(e) non-profit organizations substantially supported by philanthropic organizations including but not limited to the Bill and Melinda Gates Foundation, the Rockefeller Foundation and so forth, specifically including global product development and distribution public-private partnerships.

1.16.“Regulatory Authority” means any applicable government regulatory authority involved in granting approvals for the manufacturing and marketing of a Product, including, in the United States, the FDA.

1.17.“Strategic Partner” means any entity that agrees to compensate Licensee or its Affiliate in exchange for: Licensee's or its Affiliate's practice of the Patent Rights and/or development of Products, on behalf of or in collaboration with such entity, including without limitation, for commercialization and development activities for Products. Any entity which meets the foregoing criteria, that also receives a Sublicense shall be considered a Sublicensee, and not a Strategic Partner, for the purposes of this Agreement.

1.18.“Strategic Partnership” means any agreement with a Strategic Partner.

1.19.“Sublicense” shall mean (a) any right granted, license given or agreement entered into by Licensee to or with any other person or entity (including strategic or development partnerships), under or with respect to or permitting any use or exploitation of the Biological Material and/or the Technology Transfer Material for the purpose of producing Viruses to be used in the production of Products, or otherwise permitting the development, manufacture, marketing, distribution, use and/or sale of Products; (b) any option or other right granted by Licensee to any other person or entity to negotiate for or receive any of the rights described under clause (a); or (c) any standstill or similar obligation undertaken by Licensee toward any other person or entity not to grant any of the rights described in clause (a) or (b) to any third party; in each case regardless of whether such grant of rights, license given or agreement entered into is referred to or is described as a sublicense.

1.20.“Sublicensee” shall mean any person or entity granted a Sublicense.

1.21.“Technology Transfer Material” means the methods, protocols and other information listed in Exhibit 1.21 hereto.

1.22.“Term” means the term of this Agreement as set forth in Section 6.1.

1.23.“Third Party” means any person or entity other than Harvard, Licensee and Licensee’s Affiliates.

1.24.“Trailing Consideration” means any payments due for any deferred or contingent aggregate consideration payable to Licensee, its direct or indirect parent company, or its security holders, as the case may be, with respect to an Asset Sale, Merger or Stock Sale, including, without limitation, any post-closing milestone payment, escrow or holdback of consideration.

1.25.“Virus” means any virus produced by Licensee or a Sublicensee through use of the Biological Material that does not contain any Biological Material or any functional portion or functional fragment thereof.

2. License.

2.1.License Grant. Subject to the terms and conditions set forth in this Agreement, Harvard hereby grants to Licensee a non-exclusive, royalty-bearing, worldwide license, sublicensable solely in accordance with Section 2.2, to use the Biological Material and the Technology Transfer Material solely to produce Viruses solely to make and sell Products for use in the Field.

2.2.Sublicenses.

2.2.1 Sublicense Grant. Licensee will be entitled to grant Sublicenses to Third Parties under the license granted pursuant to Section 2.1 subject to the terms of this Section 2.2; provided that in each case such grant is made (a) in conjunction with a license to technology owned or controlled by Licensee (other than the Biological Material and the Technology

Transfer Material) that is included in or useful for the making of Products, and (b) solely for the manufacture of Viruses solely to make and sell Products for use in the Field. Affiliates of Licensee shall be permitted to exercise such right as a Sublicensee only with Harvard's prior written consent, not to be unreasonably withheld or delayed; and provided, further, that Licensee shall ensure that any such Affiliate complies with the terms of this Section 2.2.

2.2.2 Sublicense Agreements. Sublicenses shall be granted pursuant to written agreements, which will be subject and subordinate to the terms and conditions of this Agreement. Such Sublicense agreements will contain, among other things, the following:

2.2.2.1all provisions necessary to ensure Licensee's ability to perform its obligations under this Agreement;

2.2.2.2a section substantially the same as Article 5 of this Agreement, which also will state that the Indemnitees (as defined in Section 5.1) are intended third party beneficiaries of such Sublicense agreement for the purpose of enforcing such indemnification;

2.2.2.3a provision clarifying that, in the event of termination of the license set forth in Section 2.1 (in whole or in part), any existing Sublicense agreement shall terminate to the extent of such terminated license; provided, however, that, for each Sublicensee, upon termination of the license, if the Sublicensee is not then in breach of the Sublicense agreement such that Licensee would have the right to terminate such Sublicense agreement, such Sublicensee shall have the right to obtain a direct license from Harvard on the same terms and conditions as set forth herein, which direct license shall not impose any representations, warranties, obligations or liabilities on Harvard that are not included in this Agreement;

2.2.2.4a provision clarifying that the Sublicensee shall only be entitled to sublicense its rights under such Sublicense agreement on the terms set forth in this Section 2.2; and

2.2.2.5a provision prohibiting the Sublicensee from assigning the Sublicense agreement without the prior written consent of Harvard, except that Sublicensee may assign the Sublicense agreement to a successor in connection with the merger, consolidation or other reorganization of the Sublicensee, or the sale of all or substantially all of its assets or that portion of its business to which the Sublicense agreement relates; provided, however, that any permitted assignee agrees in writing to be bound by the terms of such Sublicense agreement.

2.2.3 Delivery of Sublicense Agreement. Licensee shall furnish Harvard with a fully executed copy of any Sublicense agreement, redacted with respect to matters not relevant to Harvard's interest, promptly after its execution (or promptly after the Effective Date with respect to Sublicense agreements entered into prior to the Effective Date). Harvard shall keep all such agreements and their terms confidential and shall use them solely for the purpose of monitoring Licensee's and Sublicensees' compliance with their obligations hereunder and enforcing Harvard's rights under this Agreement.

2.2.4 Breach by Sublicensee. During the term of this Agreement, Licensee shall be responsible for any breach of a Sublicense agreement by a Sublicensee that results in a material breach of this Agreement. Licensee may elect (a) to cure such breach in accordance

with Section 6.2.2 of this Agreement or (b) to enforce its rights by terminating such Sublicense agreement in accordance with the terms thereof.

2.3.Biological Material.

2.3.1 As between the parties hereto, subject to the the terms herein, including without limitation, the license granted to Licensee pursuant to Section 2.1 and any Sublicenses granted to Sublicensees pursuant to Section 2.2, all rights, title and interest in and to all Biological Material, and any intellectual property applying thereto, shall be owned solely and exclusively by Harvard.

2.3.2 Licensee shall not use Biological Material other than in accordance with the rights expressly granted to it hereunder. Except in connection with Sublicenses granted pursuant to Section 2.2, Licensee shall not sell or otherwise transfer any Biological Material to any third party.

2.3.3 The Biological Material is provided only for use in animals or *in vitro*. THE BIOLOGICAL MATERIAL SHALL NOT BE USED IN HUMANS.

2.3.4 Licensee shall inform Harvard's Office of Technology Development (at 1350 Massachusetts Avenue, Richard A. and Susan F. Smith Campus Center, Suite 727, Cambridge, MA 02138, (617) 495-3067, Attn: Chief Technology Development Officer) of any Biological Material created by Licensee that is different from, and a modification to, the Biological Material listed in Exhibit 1.3, and upon Harvard's request, shall provide samples of such material to Harvard.

2.3.5 As between the parties hereto, all right, title and interest in and to all Viruses, Products, and any intellectual property applying thereto or to the production thereof, shall be owned solely and exclusively by Licensee. For the avoidance of doubt, nothing herein prohibits or is intended to prohibit the use of Products in humans.

2.4.No Other Grant of Rights. Except as expressly provided herein, nothing in this Agreement will be construed to confer any ownership interest, license or other rights upon Licensee by implication, estoppel or otherwise as to any technology, intellectual property rights, products or biological materials of Harvard, or any other entity, regardless of whether such technology, intellectual property rights, products or biological materials are dominant, subordinate or otherwise related to any Biological Material or Technology Transfer Material.

3. Consideration for Grant of License.

3.1.License Issuance Fee. Within thirty (30) days after the Effective Date, Licensee shall pay Harvard a non-refundable license issuance fee in the amount of [**]. Such license issuance fee shall be creditable against any royalty amounts payable under Section 3.3 below with respect to Products sold in calendar year 2017.

3.2.License Maintenance Fee. Licensee shall pay Harvard a non-refundable annual license maintenance fee as follows: [**] for each (full or partial) calendar year prior to Marketing Approval; [**] for the first full calendar year after Marketing Approval; [**] for the

second full calendar year after Marketing Approval; and [**] for the third full calendar year after Marketing Approval and each calendar year thereafter; provided, that if Licensee grants rights to the Biological Material and/or the Technology Transfer Material to a Sublicensee or a Strategic Partner, the annual maintenance fee payable to Harvard as set forth above shall thereafter be [**]. Each such annual maintenance fee shall be due and payable on January 2nd of the calendar year to which such fee applies. Each annual license maintenance fee shall be creditable against any royalty amounts payable under Section 3.3 below with respect to Products sold in the same calendar year that such annual license maintenance fee applies.

3.3.Royalty on Net Sales. Licensee shall pay Harvard an amount equal to (i) [**] of Net Sales with respect to the first [**] of cumulative Net Sales, and (ii) [**] of Net Sales with respect to cumulative Net Sales in excess of [**].

3.4.Non-Royalty Income Fee. Licensee will pay Harvard an amount equal to [**] of all Non-Royalty Income.

3.5.Milestone Payments. With respect to each Product, Licensee will pay Harvard [**] within [**] after first [**] of such Product.

3.6.Change of Control Payment. [**]

3.7.Late Payment. Any payment by Licensee that is not paid on or before the date such payment is due under this Agreement will bear interest at the lower of (a) [**] per month and (b) the maximum rate allowed by law. Interest will accrue beginning on the first day following the due date for payment and will be compounded quarterly. Payment of such interest by Licensee shall not limit, in any way, Harvard's right to exercise any other remedies Harvard may have as a consequence of the lateness of any payment.

3.8.Payment Method. Each payment due to Harvard under this Agreement shall be paid by check or wire transfer of funds to Harvard's account in accordance with written instructions provided by Harvard. If made by wire transfer, such payments shall be marked so as to refer to this Agreement. All payments due under this Agreement will be paid in U.S. Dollars. Conversion of foreign currency to U.S. Dollars will be made at the conversion rate existing in the United States (as reported in the *Wall Street Journal)* on the last working day of the applicable Calendar Quarter. Such payments will be without deduction of exchange, collection or other charges.

3.9.Reporting. Within thirty (30) days after the conclusion of each Calendar Quarter commencing with the first Calendar Quarter in which Net Sales are generated or Non-Royalty Income is received, Licensee shall deliver to Harvard a report containing the following information (in each instance, with a Product-by-Product breakdown):

3.9.1 the number of units of Products sold, leased or otherwise transferred by Invoicing Entities for the applicable Calendar Quarter (with a Product-by-Product breakdown);

3.9.2 the gross amount billed or invoiced for Products sold, leased or otherwise transferred by Invoicing Entities during the applicable Calendar Quarter;

3.9.3 a calculation of Net Sales for the applicable Calendar Quarter, including an itemized listing of allowable deductions;

3.9.4 a detailed accounting of all Non-Royalty Income received during the applicable Calendar Quarter; and

3.9.5 the total amount payable to Harvard in U.S. Dollars on Net Sales and Non-Royalty Income for the applicable Calendar Quarter, together with the exchange rates used for conversion.

Each such report shall be certified on behalf of Licensee as true, correct and complete in all material respects. If no amounts are due to Harvard for a particular Calendar Quarter, the report shall so state.

3.10.Records. Licensee shall maintain, and shall cause its Affiliates and Sublicensees to maintain, complete and accurate records of Products that are made, used, sold, leased or transferred under this Agreement, any amounts payable to Harvard in relation to such Products, and all Non-Royalty Income received by Licensee and its Affiliates, which records shall contain sufficient information to permit Harvard to confirm the accuracy of any reports or notifications delivered to Harvard under Section 3.9. Licensee, its Affiliates and/or its Sublicensees, as applicable, shall retain such records relating to a given Calendar Quarter for at least five (5) years after the conclusion of that Calendar Quarter, during which time Harvard will have the right, at its expense, to cause an independent, certified public accountant (or, in the event of a non-financial audit, other appropriate auditor) to inspect such records during normal business hours for the purposes of verifying the accuracy of any reports and payments delivered under this Agreement and Licensee's compliance with the terms hereof. Such accountant or other auditor, as applicable, shall not disclose to Harvard any information other than information relating to the accuracy of reports and payments delivered under this Agreement. The parties shall reconcile any underpayment or overpayment within thirty (30) days after the accountant delivers the results of the audit. If any audit performed under this Section 3.10 reveals an underpayment in excess of [**] in any calendar year, Licensee shall reimburse Harvard for all amounts incurred in connection with such audit. Harvard may exercise its rights under this Section 3.10 only once every year per audited entity and only with reasonable prior notice to the audited entity.

4. Warranties; Limitation of Liability.

4.1.Compliance with Law. Licensee represents and warrants that it will comply, and that it will ensure that its Affiliates comply, with all local, state and international laws and regulations relating to the Biological Material and to the development, manufacture, use, sale and importation of Viruses and Products. Without limiting the foregoing, Licensee represents and warrants that it will comply with all United States export control laws and regulations with respect to Biological Material and any Viruses and Products developed or made through the use thereof.

4.2.Harvard Representations and Warranties. Harvard hereby represents and warrants to Licensee that Harvard has the authority to grant the license herein to the Biological

Material and the Technology Transfer Material and Harvard has not granted to any Third Party any rights that would conflict with this Agreement.

4.3.No Warranty.

4.3.1 HARVARD MAKES NO REPRESENTATIONS OR WARRANTIES WHATSOEVER AS TO THE COMMERCIAL OR SCIENTIFIC VALUE OF THE BIOLOGICAL MATERIAL. HARVARD MAKES NO REPRESENTATION OR WARRANTY THAT THE USE OF THE BIOLOGICAL MATERIAL, TECHNOLOGY TRANSFER MATERIAL, OR THE DEVELOPMENT, MANUFACTURE, USE, SALE OR IMPORTATION OF ANY VIRUS OR PRODUCT, OR ANY ELEMENT THEREOF, WILL NOT INFRINGE THE PATENT OR PROPRIETARY RIGHTS OF ANY THIRD PARTY.

4.3.2 EXCEPT AS OTHERWISE EXPRESSLY PROVIDED IN THIS AGREEMENT, NEITHER PARTY MAKES ANY WARRANTY WITH RESPECT TO ANY TECHNOLOGY, PATENTS, MATERIALS (INCLUDING BIOLOGICAL MATERIAL), GOODS, SERVICES, RIGHTS, TECHNOLOGY TRANSFER MATERIAL OR OTHER SUBJECT MATTER OF THIS AGREEMENT AND EACH PARTY HEREBY DISCLAIMS WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE AND NONINFRINGEMENT WITH RESPECT TO ANY AND ALL OF THE FOREGOING.

4.3.3 ALL BIOLOGICAL MATERIAL IS EXPERIMENTAL IN NATURE AND SHALL BE USED WITH PRUDENCE AND APPROPRIATE CAUTION SINCE NOT ALL OF THEIR CHARACTERISTICS ARE KNOWN.

4.4.Limitation of Liability.

4.4.1 Except with respect to matters for which Licensee is obligated to indemnify Harvard under Article 5, neither party will be liable to the other with respect to any subject matter of this Agreement under any contract, negligence, strict liability or other legal or equitable theory for (a) any indirect, incidental, consequential or punitive damages or lost profits or (b) cost of procurement of substitute goods, technology or services.

4.4.2 Harvard's aggregate liability for all damages of any kind arising out of or relating to this Agreement or its subject matter under any contract, negligence, strict liability or other legal or equitable theory shall not exceed the amounts paid to Harvard under this Agreement.

5. Indemnification and Insurance.

5.1.Indemnity.

5.1.1 Licensee shall indemnify, defend and hold harmless Harvard and its current and former directors, governing board members, trustees, officers, faculty, medical and professional staff, employees, students, and agents and their respective successors, heirs and assigns (collectively, the "Indemnitees") from and against any claim, liability, cost, expense, damage, deficiency, loss or obligation of any kind or nature (including reasonable attorneys' fees and other costs and expenses of litigation) by or owed to a third party, based upon, arising out of,

or otherwise relating to the activities of Licensee and Sublicensees under this Agreement, including any cause of action relating to product liability concerning any product, process, or service made, used, sold or performed pursuant to any right or license granted under this Agreement (collectively, the "Claims"); provided, however that Licensee's indemnification obligations hereunder shall not apply to any Claim to the extent that it is attributable to the gross negligence or willful misconduct of any Indemnitee.

5.1.2 Licensee shall, at its own expense, provide attorneys reasonably acceptable to Harvard to defend against any actions brought or filed against any Indemnitee hereunder with respect to the subject of indemnity contained herein, whether or not such actions are rightfully brought. Any Indemnitee seeking indemnification hereunder shall promptly notify Licensee of such Claim; provided further that any failure of or delay in such notification shall not affect Licensee's indemnification obligation unless and to the extent such failure or delay is materially prejudicial to Licensee. The Indemnitees shall provide Licensee, at Licensee's expense, with reasonable assistance and full information with respect to such Claim and give Licensee sole control of the defense of any Claim. Neither Licensee nor Harvard shall settle any Claim without the prior written consent of the other, which consent shall not be unreasonably withheld.

5.2.Insurance.

5.2.1 Beginning at the time any Product is being commercially distributed or sold by Licensee, or by an Affiliate or agent of Licensee, Licensee shall, at its sole cost and expense, procure and maintain commercial general liability insurance in amounts not less than [**] per incident and [**] annual aggregate and naming the Indemnitees as additional insureds. Such commercial general liability insurance shall provide: (a) product liability coverage and (b) broad form contractual liability coverage for Licensee's indemnification obligations under this Agreement.

5.2.2 If Licensee elects to self-insure all or part of the limits described above in Section 5.2.1 (including deductibles or retentions that are in excess of [**] annual aggregate) such self-insurance program must be acceptable to Harvard and CRICO/RMF (Harvard's insurer) in their sole discretion. The minimum amounts of insurance coverage required shall not be construed to create a limit of Licensee's liability with respect to its indemnification obligations under this Agreement.

5.2.3 Licensee shall provide Harvard with written evidence of such insurance upon request of Harvard. Licensee shall provide Harvard with written notice at least fifteen (15) days prior to the cancellation, non-renewal or material change in such insurance. If Licensee does not obtain replacement insurance providing comparable coverage within thirty (30) days after such notice, Harvard shall have the right to terminate this Agreement effective at the end of such thirty (30) day period without notice or any additional waiting periods.

5.2.4 Licensee shall maintain such commercial general liability insurance beyond the expiration or termination of this Agreement during: (a) the period that any Product is being commercially distributed or sold by Licensee, or an Affiliate or agent of Licensee; and (b)

a reasonable period after the period referred to in (a) above which in no event shall be less than [**].

6. Term and Termination.

6.1.Term. The term of this Agreement shall commence on the Effective Date and, unless earlier terminated as provided in this Article 6, shall continue in full force and effect for fifteen (15) years thereafter (the "Initial Term"). After the Initial Term, this Agreement shall renew for successive three-year periods (the Initial Term and such period, the "Term") unless either Party gives the other Party written notice of its desire to terminate the Agreement no less than sixty (60) days prior to the expiration of the Initial Term or any applicable successive period during the Term.

6.2.Termination.

6.2.1 Termination Without Cause. Licensee may terminate this Agreement upon sixty (60) days prior written notice to Harvard.

6.2.2 Termination for Default.

6.2.2.1 In the event that either party commits a material breach of its obligations under this Agreement and fails to cure that breach within ninety (90) days after receiving written notice thereof, the other party may terminate this Agreement immediately upon written notice to the party in breach.

6.2.2.2 If Licensee defaults in its obligations under Section 5.2 to procure and maintain insurance or, if Licensee has in any event failed to comply with the notice requirements contained therein and fails to cure that default within thirty (30) days after receiving written notice thereof, Harvard may terminate this Agreement immediately upon written notice to Licensee.

6.2.3 Bankruptcy. Harvard may terminate this Agreement upon notice to Licensee if Licensee becomes insolvent, is adjudged bankrupt, applies for judicial or extra-judicial settlement with its creditors, makes an assignment for the benefit of its creditors, voluntarily files for bankruptcy or has a receiver or trustee (or the like) in bankruptcy appointed by reason of its insolvency, or in the event an involuntary bankruptcy action is filed against Licensee and not dismissed within ninety (90) days, or if Licensee becomes the subject of liquidation or dissolution proceedings or otherwise discontinues business.

6.3.Effect of Termination.

6.3.1 Termination of Rights. Upon any termination or expiration of this Agreement, (a) the rights and licenses granted to Licensee under Article 2 shall terminate, (b) all rights in and to and under the Biological Material and Technology Transfer Material will revert to Harvard and, subject to Section 6.3.2, neither Licensee nor its Sublicensees may make any further use or exploitation of any Biological Material or Technology Transfer Material and (c) any existing Sublicense shall terminate; provided, however, that, for each Sublicensee, upon termination of the license, if the Sublicensee is not then in breach of the Sublicense agreement

such that Licensee would have the right to terminate such Sublicense agreement, such Sublicensee shall have the right to obtain a direct license from Harvard on the same terms and conditions as set forth herein, which shall not impose any representations, warranties, obligations or liabilities on Harvard that are not included in this Agreement. Furthermore, in the event of any termination or expiration of this Agreement, Licensee shall destroy, and shall cause its agents and Sublicensees to destroy, all Biological Material under their control or in their possession.

6.3.2 Accruing Obligations. Termination or expiration of this Agreement shall not relieve the parties of obligations accruing prior to such termination or expiration, including obligations to pay amounts accruing hereunder up to the date of termination or expiration. After the date of termination or expiration (except in the case of termination by Harvard pursuant to Section 6.2), Licensee and its Sublicensees (a) may sell Products then in stock and (b) may complete the production of Products then in the process of production and sell the same; provided that, in the case of both (a) and (b), Licensee shall maintain insurance in accordance with the requirements of Section 5.2.

6.4.Survival. The parties' respective rights, obligations and duties under Articles 4, 5 and 7 and Sections 2.2.2.3 (but only with respect to a Sublicensee's right to obtain a direct license from Harvard), 2.3, 3.1, 3.6, 6.3, 6.4, as well as any rights, obligations and duties which by their nature extend beyond the expiration or termination of this Agreement, shall survive any expiration or termination of this Agreement.

7. Miscellaneous.

7.1.No Security Interest. Licensee shall not enter into any agreement under which Licensee grants to or otherwise creates in any third party a security interest in this Agreement or any of the rights granted to Licensee herein. Any grant or creation of a security interest purported or attempted to be made in violation of the terms of this Section 7.1 shall be null and void and of no legal effect.

7.2.Use of Name. Except as provided below, Licensee shall not, and shall ensure that its Affiliates and Sublicensees shall not, use or register the name "Harvard" (alone or as part of another name) or any logos, seals, insignia or other words, names, symbols or devices that identify Harvard or any Harvard school, unit, division or affiliate ("Harvard Names") for any purpose except with the prior written approval of, and in accordance with restrictions required by, Harvard. Without limiting the foregoing, Licensee shall, and shall ensure that its Affiliates and Sublicensees shall, cease all use of Harvard Names on the termination or expiration of this Agreement except as otherwise approved by Harvard. This restriction shall not apply to any information required by law to be disclosed to any governmental entity.

7.3.Entire Agreement. This Agreement is the sole agreement with respect to the subject matter hereof and except as expressly set forth herein, supersedes all other agreements and understandings between the parties with respect to the same.

7.4.Notices. Unless otherwise specifically provided, all notices required or permitted by this Agreement shall be in writing and may be delivered personally, or may be sent by

facsimile, overnight delivery or certified mail, return receipt requested, to the following addresses, unless the parties are subsequently notified of any change of address in accordance with this Section 7.4:

If to Licensee (invoices only):	Solid Biosciences, LLC 161 First Street, Suite #3A Cambridge, MA 02142 Fax: Email: Phone: Attn:
If to Licensee (all other notices):	Solid Biosciences, LLC 161 First Street, Suite #3A Cambridge, MA 02142 Fax: Email: Phone: Attn:
If to Harvard:	Office of Technology Development Harvard University 1350 Massachusetts Avenue Cambridge, Massachusetts 02138 Fax: Attn.:

Any notice shall be deemed to have been received as follows: (a) by personal delivery, upon receipt; (b) by facsimile or overnight delivery, one business day after transmission or dispatch; (c) by certified mail, as evidenced by the return receipt. If notice is sent by facsimile, a confirming copy of the same shall be sent by mail to the same address.

7.5.Governing Law and Jurisdiction. This Agreement will be governed by, and construed in accordance with, the substantive laws of the Commonwealth of Massachusetts, without giving effect to any choice or conflict of law provision, except that questions affecting the construction and effect of any patent shall be determined by the law of the country in which the patent shall have been granted. Any action, suit or other proceeding arising under or relating to this Agreement (a "Suit") shall be brought in a court of competent jurisdiction in the Commonwealth of Massachusetts, and the parties hereby consent to the sole jurisdiction of the state and federal courts sitting in the Commonwealth of Massachusetts. Each party agrees not to raise any objection at any time to the laying or maintaining of the venue of any Suit in any of the specified courts, irrevocably waives any claim that Suit has been brought in any inconvenient

forum and further irrevocably waives the right to object, with respect to any Suit, that such court does not have any jurisdiction over such party.

7.6.Binding Effect. This Agreement shall be binding upon and inure to the benefit of the parties and their respective legal representatives, successors and permitted assigns.

7.7.Headings. Section and subsection headings are inserted for convenience of reference only and do not form a part of this Agreement.

7.8.Counterparts. The parties may execute this Agreement in two or more counterparts, each of which shall be deemed an original, but both of which together shall constitute one and the same instrument. Transmission by facsimile or electronic mail of an executed counterpart of this Agreement shall be deemed to constitute due and sufficient delivery of such counterpart. If by electronic mail, the executed Agreement must be delivered in a .pdf format.

7.9.Amendment; Waiver. This Agreement may be amended, modified, superseded or canceled, and any of the terms may be waived, only by a written instrument executed by each party or, in the case of waiver, by the party waiving compliance. The delay or failure of either party at any time or times to require performance of any provisions hereof shall in no manner affect the rights at a later time to enforce the same. No waiver by either party of any condition or of the breach of any term contained in this Agreement, whether by conduct, or otherwise, in any one or more instances, shall be deemed to be, or considered as, a further or continuing waiver of any such condition or of the breach of such term or any other term of this Agreement.

7.10.No Agency or Partnership. Nothing contained in this Agreement shall give either party the right to bind the other, or be deemed to constitute either party as agent for or partner of the other or any third party.

7.11.Assignment and Successors. This Agreement may not be assigned by either party without the consent of the other, which consent shall not be unreasonably withheld, except that each party may, without such consent, assign this Agreement and the rights, obligations and interests of such party to any purchaser of all or substantially all of its assets to which the subject matter of this Agreement relates, or to any successor corporation resulting from any merger or consolidation of such party with or into such corporation; provided, in each case, that the assignee agrees in writing to be bound by the terms of this Agreement. Any assignment purported or attempted to be made in violation of the terms of this Section 7.11 shall be null and void and of no legal effect.

7.12.Force Majeure. Except for monetary obligations hereunder, neither party will be responsible for delays resulting from causes beyond the reasonable control of such party, including fire, explosion, flood, war, strike, or riot, provided that the nonperforming party uses commercially reasonable efforts to avoid or remove such causes of nonperformance and continues performance under this Agreement with reasonable dispatch whenever such causes are removed.

7.13.Interpretation. Each party hereto acknowledges and agrees that: (a) it and/or its counsel reviewed and negotiated the terms and provisions of this Agreement and has contributed

to its revision; (b) the rule of construction to the effect that any ambiguities are resolved against the drafting party shall not be employed in the interpretation of this Agreement; (c) the terms and provisions of this Agreement shall be construed fairly as to both parties hereto and not in favor of or against either party, regardless of which party was generally responsible for the preparation of this Agreement; and (d) the use of "include," "includes," or "including" herein shall not be limiting and "or" shall not be exclusive.

7.14.Severability. If any provision of this Agreement is or becomes invalid or is ruled invalid by any court of competent jurisdiction or is deemed unenforceable, it is the intention of the parties that the remainder of this Agreement shall not be affected.

IN WITNESS WHEREOF, the parties have caused this Agreement to be executed by their duly authorized representatives as of the date first written above.

President and Fellows of Harvard College

By: /s/ Jordan B. Grant

Name: Jordan B. Grant

Title: Director of Technology Transactions
Office of Technology Development
Harvard University

Solid Biosciences, LLC

By: /s/ Ilan Ganot

Name: Ilan Ganot

Title: CEO

Exhibit 21.1

Subsidiaries of Solid Biosciences Inc.

Name of Subsidiary	Jurisdiction of Organization
AavantiBio, Inc.	Delaware
Solid Biosciences Securities Corporation	Massachusetts

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We hereby consent to the incorporation by reference in the Registration Statements on Form S-3 (Nos. 333-276764, 333-269424, 333-258859, 333-254310, and 333-233594) and Form S-8 (Nos. 333-272456, 333-270765, 333-269162, 333-268643, 333-265351, 333-258856, 333-241370 and 333-222763) of Solid Biosciences Inc. of our report dated March 13, 2024 relating to the financial statements, which appears in this Form 10-K.

/s/ PricewaterhouseCoopers LLP
Boston, Massachusetts
March 13, 2024

Exhibit 31.1

CERTIFICATION OF PERIODIC REPORT UNDER SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Alexander Cumbo, certify that:

1. I have reviewed this Annual Report on Form 10-K of Solid Biosciences Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 13, 2024

By: /s/ Alexander Cumbo

Alexander Cumbo
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PERIODIC REPORT UNDER SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Kevin Tan, certify that:

1. I have reviewed this Annual Report on Form 10-K of Solid Biosciences Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 13, 2024

By: /s/ Kevin Tan

Kevin Tan
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of Solid Biosciences Inc. (the "Company") on Form 10-K for the year ended December 31, 2023 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Alexander Cumbo, Chief Executive Officer of the Company, certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: March 13, 2024

By: /s/ Alexander Cumbo
Alexander Cumbo
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of Solid Biosciences Inc. (the "Company") on Form 10-K for the year ended December 31, 2023 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Kevin Tan, Chief Financial Officer of the Company, certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: March 13, 2024

By: /s/ Kevin Tan
Kevin Tan
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 97.1

SOLID BIOSCIENCES INC.

Dodd-Frank Compensation Recovery Policy

This Compensation Recovery Policy (this "Policy") is adopted by Solid Biosciences Inc. (the "Company") in accordance with Nasdaq Listing Rule 5608 ("Rule 5608"), which implements Rule 10D-1 under the Securities Exchange Act of 1934, as amended (the "Exchange Act") (as promulgated pursuant to Section 954 of the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010). This Policy is effective as of October 2, 2023 (the "Effective Date").

1. Definitions

(a) **"Accounting Restatement"** means a requirement that the Company prepare an accounting restatement due to the material noncompliance of the Company with any financial reporting requirement under the U.S. federal securities laws, including any required accounting restatement to correct an error in previously issued financial statements that is material to the previously issued financial statements, or that would result in a material misstatement if the error were corrected in the current period or left uncorrected in the current period. Changes to the Company's financial statements that do not represent error corrections are not an Accounting Restatement, including: (A) retrospective application of a change in accounting principle; (B) retrospective revision to reportable segment information due to a change in the structure of the Company's internal organization; (C) retrospective reclassification due to a discontinued operation; (D) retrospective application of a change in reporting entity, such as from a reorganization of entities under common control; and (E) retrospective revision for stock splits, reverse stock splits, stock dividends or other changes in capital structure.

(b) **"Committee"** means the Compensation Committee of the Company's Board of Directors (the "Board").

(c) **"Covered Person"** means a person who served as an Executive Officer at any time during the performance period for the applicable Incentive-Based Compensation.

(d) **"Erroneously Awarded Compensation"** means the amount of Incentive-Based Compensation that was Received that exceeds the amount of Incentive-Based Compensation that otherwise would have been Received had the amount of Incentive-Based Compensation been determined based on the restated amounts, computed without regard to any taxes paid by the Covered Person or by the Company on the Covered Person's behalf. For Incentive-Based Compensation based on stock price or total shareholder return, where the amount of Erroneously Awarded Compensation is not subject to mathematical recalculation directly from the information in an Accounting Restatement, the amount of Erroneously Awarded Compensation will be based on a reasonable estimate by the Committee of the effect of the Accounting Restatement on the stock price or total shareholder return upon which the Incentive-Based Compensation was Received. The Company will maintain documentation of the determination of that reasonable estimate and provide such documentation to Nasdaq.

(e) **"Executive Officer"** means the Company's officers as defined in Rule 16a-1(f) under the Exchange Act.

(f) **"Financial Reporting Measures"** means (A) measures that are determined and presented in accordance with the accounting principles used in preparing the Company's financial statements, and any measures that are derived wholly or in part from such measures (whether or not such measures are presented within the Company's financial statements or included in a filing made with the U.S. Securities and Exchange Commission), (B) stock price and (C) total shareholder return.

(g) **"Incentive-Based Compensation"** means any compensation that is granted, earned, or vested based wholly or in part upon the attainment of a Financial Reporting Measure.

(h) Incentive-Based Compensation is deemed to be **"Received"** in the Company's fiscal period during which the Financial Reporting Measure specified in the applicable Incentive-Based Compensation award is attained, even if the payment or grant of the Incentive-Based Compensation occurs after the end of that period or is subject to additional time-based vesting requirements.

(i) **"Recovery Period"** means the three completed fiscal years immediately preceding the earlier of: (A) the date the Board, a committee of the Board, or the officer or officers of the Company authorized to take such action if Board action is not required, concludes, or reasonably should have concluded, that the Company is required to prepare an Accounting Restatement; or (B) the date a court, regulator, or other legally authorized body directs the Company to prepare an Accounting Restatement. In addition, if there is a change in the Company's fiscal year end, the Recovery Period will also include any transition period to the extent required by Rule 5608.

2. Recovery of Erroneously Awarded Compensation

Subject to the terms of this Policy and the requirements of Rule 5608, if the Company is required to prepare an Accounting Restatement, the Company will attempt to recover, reasonably promptly from each Covered Person, any Erroneously Awarded Compensation that was Received by such Covered Person during the Recovery Period pursuant to Incentive-Based Compensation that is subject to this Policy.

3. Interpretation and Administration

(a) Role of the Committee. This Policy will be interpreted by the Committee in a manner that is consistent with Rule 5608 and any other applicable law and will otherwise be interpreted in the business judgment of the Committee. All decisions and interpretations of the Committee that are consistent with Rule 5608 will be final and binding.

(b) Compensation Not Subject to this Policy. This Policy does not apply to Incentive-Based Compensation that was Received before the Effective Date. With respect to any Covered Person, this Policy does not apply to Incentive-Based Compensation that was Received by such Covered Person before beginning service as an Executive Officer.

(c) Determination of Means of Recovery. Subject to the requirement that recovery be made reasonably promptly, the Committee will determine the appropriate means of recovery, which may vary between Covered Persons or based on the nature of the applicable

Incentive-Based Compensation, and which may involve, without limitation, establishing a deferred repayment plan or setting off against current or future compensation otherwise payable to the Covered Person. Recovery of Erroneously Awarded Compensation will be made without regard to income taxes paid by the Covered Person or by the Company on the Covered Person's behalf in connection with such Erroneously Awarded Compensation.

(d) Determination That Recovery is Impracticable. The Company is not required to recover Erroneously Awarded Compensation if a determination is made by the Committee that either (A) after the Company has made and documented a reasonable attempt to recover such Erroneously Awarded Compensation, the direct expense paid to a third party to assist in enforcing this Policy would exceed the amount to be recovered or (B) recovery of such Erroneously Awarded Compensation would likely cause an otherwise tax-qualified retirement plan, under which benefits are broadly available to employees of the Company, to fail to meet the requirements of Section 401(a)(13) or 411(a) of the Internal Revenue Code and regulations thereunder.

(e) No Indemnification or Company-Paid Insurance. The Company will not indemnify any Covered Person against the loss of Erroneously Awarded Compensation and will not pay or reimburse any Covered Person for the purchase of a third-party insurance policy to fund potential recovery obligations.

(f) Interaction with Other Clawback Provisions. The Company will be deemed to have recovered Erroneously Awarded Compensation in accordance with this Policy to the extent the Company actually receives such amounts pursuant to any other Company policy, program or agreement, pursuant to Section 304 of the Sarbanes-Oxley Act or otherwise.

(g) No Limitation on Other Remedies. Nothing in this Policy will be deemed to limit the Company's right to terminate employment of any Covered Person, to seek recovery of other compensation paid to a Covered Person, or to pursue other rights or remedies available to the Company under applicable law.

Adopted by the Board on November 12, 2023.