

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

**Form 10-K**

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

For the fiscal year ended December 31, 2020

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: 000-30171

**SANGAMO THERAPEUTICS, INC.**

(Exact name of registrant as specified in its charter)

Delaware  
(State or other jurisdiction of  
incorporation or organization)

68-0359556  
(I.R.S. Employer  
Identification No.)

7000 Marina Blvd.  
Brisbane, California  
(Address of principal executive offices)

94005  
(Zip Code)

(510) 970-6000  
(Registrant's telephone number, including area code)

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

<u>Title of each class</u>	<u>Trading Symbol(s)</u>	<u>Name of each exchange on which registered</u>
Common Stock, par value \$0.01 per share	SGMO	Nasdaq Global Select Market

Securities registered pursuant to Section 12(g) of the Act: None

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 Exchange 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 definition of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	<input checked="" type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

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.

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

The aggregate market value of the common stock held by non-affiliates of the registrant based upon the closing sale price of the common stock on June 30, 2020 (the last business day of the registrant's most recently completed second fiscal quarter), as reported on the Nasdaq Global Select Market was \$1,262,431,735. For purposes of this calculation, directors and executive officers of the registrant have been deemed affiliates. This determination of affiliate status is not necessarily a conclusive determination for other purposes.

As of February 19, 2021, a total of 143,251,243 shares of common stock, \$0.01 par value per share were outstanding.

**DOCUMENTS INCORPORATED BY REFERENCE**

Certain information required by Part III, Items 10-14 of this Form 10-K is incorporated by reference to the registrant's definitive Proxy Statement for the 2021 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission pursuant to Regulation 14A not later than 120 days after the end of the fiscal year covered by this Form 10-K, provided that if such Proxy Statement is not filed within such period, such information will be included in an amendment to this Form 10-K to be filed within such 120-day period.

**TABLE OF CONTENTS**

	<u>Page</u>
<b><u>PART I</u></b>	
Item 1.	<a href="#">Business</a> 5
Item 1A.	<a href="#">Risk Factors</a> 40
Item 1B.	<a href="#">Unresolved Staff Comments</a> 66
Item 2.	<a href="#">Properties</a> 66
Item 3.	<a href="#">Legal Proceedings</a> 66
Item 4.	<a href="#">Mine Safety Disclosures</a> 66
<b><u>PART II</u></b>	
Item 5.	<a href="#">Market for the Registrant’s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities</a> 67
Item 6.	<a href="#">Selected Financial Data</a> 68
Item 7.	<a href="#">Management’s Discussion and Analysis of Financial Condition and Results of Operations</a> 69
Item 7A.	<a href="#">Quantitative and Qualitative Disclosures About Market Risk</a> 77
Item 8.	<a href="#">Financial Statements and Supplementary Data</a> 79
Item 9.	<a href="#">Changes in and Disagreements with Accountants on Accounting and Financial Disclosure</a> 117
Item 9A.	<a href="#">Controls and Procedures</a> 117
Item 9B.	<a href="#">Other Information</a> 120
<b><u>PART III</u></b>	
Item 10.	<a href="#">Directors, Executive Officers and Corporate Governance</a> 120
Item 11.	<a href="#">Executive Compensation</a> 120
Item 12.	<a href="#">Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters</a> 120
Item 13.	<a href="#">Certain Relationships and Related Transactions, and Director Independence</a> 120
Item 14.	<a href="#">Principal Accounting Fees and Services</a> 121
<b><u>PART IV</u></b>	
Item 15.	<a href="#">Exhibits, Financial Statement Schedules</a> 122
Item 16.	<a href="#">Form 10-K Summary</a> 125
<b><u>SIGNATURES</u></b>	

## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

Some statements contained in this report are “forward-looking statements” within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, or the Exchange Act. These statements relate to our future events, including our anticipated operations, research, development and commercialization activities, clinical trials, operating results and financial condition. These forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements. Forward-looking statements may include, but are not limited to, statements about:

- our strategy;
- anticipated research and development of product candidates and potential commercialization of any resulting approved products;
- the initiation, scope, rate of progress, enrollment, anticipated results and timing of our preclinical studies and clinical trials and those of our collaborators or strategic partners;
- the therapeutic and commercial potential of technologies used by us in our product candidates, including our zinc finger protein technology platform, zinc finger nucleases and zinc finger protein transcription factors;
- the expected benefits of the acquisition of Sangamo Therapeutics France S.A.S., or Sangamo France;
- our ability to establish and maintain collaborations and strategic partnerships and realize the expected benefits of such arrangements;
- anticipated revenues from existing and new collaborations and the timing thereof;
- our estimates regarding the impact of the evolving COVID-19 pandemic on our business and operations and the business and operations of our collaborators, including clinical trials and manufacturing, and our ability to manage such impacts;
- our research and development and other expenses;
- our ability to obtain adequate preclinical and clinical supplies of our product candidates from current and potential new suppliers and manufacturers;
- the ability of Sangamo and our collaborators and strategic partners to obtain and maintain regulatory approvals for product candidates;
- our ability to comply with, and the impact of, regulatory requirements, obligations and restrictions on our business and operations;
- our ability to protect our intellectual property and operate our business without infringing upon the intellectual property rights of others, including our ability to obtain rights to the gene transfer technologies required to develop and commercialize our product candidates;
- our estimates regarding the sufficiency of our cash resources and our expenses, capital requirements and need for additional financing, and our ability to obtain additional financing;
- our ability to manage the growth of our business;
- our projected operating and financial performance;
- our operational and legal risks; and
- our plans, objectives, expectations and intentions and any other statements that are not historical facts.

In some cases, you can identify forward-looking statements by terms such as: “anticipates,” “believes,” “continues,” “could,” “estimates,” “expects,” “intends,” “may,” “plans,” “seeks,” “should,” “will” and similar expressions intended to identify forward-looking statements. These statements reflect our current views with respect to future events, are based on assumptions and are subject to risks and uncertainties. Given these risks and uncertainties, you should not place undue reliance on these forward-looking statements. We discuss many of these risks in greater detail under the headings “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in this Annual Report on Form 10-K. Except as required by law, we undertake no obligation to update or revise any forward-looking statements to reflect new information or future events or developments. Readers are cautioned not to place undue reliance on the forward-looking statements, which speak only as of the date of this Annual Report on Form 10-K.

## SUMMARY OF RISK FACTORS

Our business involves significant risks. Below is a summary of the material risks that our business faces, which makes an investment in our common stock speculative and risky. This summary does not address all these risks. These risks are more fully described below under the heading “Risk Factors” in Part I, Item 1A of this Annual Report on Form 10-K. Before making investment decisions regarding our common stock, you should carefully consider these risks. The occurrence of any of the events or developments described below could have a material adverse effect on our business, results of operations, financial condition, prospects and stock price. In such event, the market price of our common stock could decline, and you could lose all or part of your investment. In addition, there are also additional risks not described below that are either not presently known to us or that we currently deem immaterial, and these additional risks could also materially impair our business, operations or market price of our common stock.

- We are a clinical-stage biotechnology company with no approved products or product revenues. Our success depends substantially on clinical trial results demonstrating safety and efficacy of our product candidates to the satisfaction of regulatory authorities. Obtaining positive clinical trial results and regulatory approvals is expensive, lengthy, challenging and unpredictable and may never occur for any product candidates.
- Many of our product candidates are based on novel zinc finger protein technologies that have yet to yield any approved commercially viable therapeutic products.
- We have incurred significant operating losses since inception and anticipate continued losses for the foreseeable future. We may never become profitable.
- We require additional capital to fund our operations and continue operating as a viable business. This additional capital may not be available to us on favorable terms or at all.
- We rely heavily on collaborations with larger biopharmaceutical companies to generate revenues and develop, obtain regulatory approvals for and commercialize many of our product candidates. If conflicts arise with our collaborators or if the collaborations terminate for any reason, our revenues and product development efforts would be negatively impacted.
- Biotechnology and genomic medicine are highly competitive businesses. Our competitors may develop rival technologies and products that are superior to or are commercialized more quickly than our technologies and product candidates.
- Manufacturing genomic medicines is complex, expensive, highly regulated and risky. We currently rely heavily on third party manufacturers and have limited experience manufacturing products ourselves. Manufacturing challenges may result in unexpected costs, supply interruptions and harm and delay to our product development efforts.
- Even if we obtain regulatory approvals for our product candidates, our approved products may not gain market acceptance among physicians and patients and adequate coverage and reimbursement from third-party payors and may not demonstrate commercial viability.
- We may not be able to obtain, maintain and enforce necessary and desirable intellectual property protections for our technologies and product candidates in all desired jurisdictions, which could adversely affect the value of our technologies and our product development efforts and could increase the risks of costly, lengthy and distracting litigation with unpredictable results.
- Our success depends on hiring, integrating and retaining additional highly qualified skilled employees and retaining current key executives and employees, which may be challenging given that competition for these individuals is intense.
- The evolving COVID-19 pandemic could continue to adversely impact our business and operations and the business and operations of our collaborators, manufacturers and other business partners. If such impacts become material, our revenues and product development efforts could be negatively impacted.
- The market price of our common stock has been and will likely continue to be volatile, and you could lose all or part of any investment in our common stock.

## PART I

### ITEM 1 – BUSINESS

#### OVERVIEW

We are a clinical-stage genomic medicine company committed to translating ground-breaking science into medicines that transform the lives of patients with serious diseases. We plan to deliver on this mission through (i) development of our clinical and preclinical product candidates, (ii) our novel science and (iii) our in-house manufacturing capabilities.

##### *Our Product Candidates*

Our current clinical-stage product candidates are:

- Giroctocogene fitelparvovec, also known as SB-525, our lead product candidate, is a gene therapy for the treatment of hemophilia A and is currently being evaluated in the registrational Phase 3 AFFINE (efficAcY and saFety Factor vIii geNe thErapy) clinical trial. We are developing giroctocogene fitelparvovec with our collaborator Pfizer Inc., or Pfizer;
- ST-920, our wholly-owned gene therapy product candidate for the treatment of Fabry disease, is currently being evaluated in our Phase 1/2 STAAR clinical study;
- BIVV003, our cell therapy product candidate for the treatment of sickle cell disease, or SCD, is currently being evaluated in our Phase 1/2 PRECIZN-1 clinical study. We are developing BIVV003 with our collaborator Sanofi S.A., or Sanofi; and
- ST-400, our cell therapy product candidate for the treatment of transfusion dependent beta thalassemia, is currently being evaluated in our Phase 1/2 Thales clinical study. We are developing ST-400 with our collaborator Sanofi.

In addition, we expect to initiate clinical studies for two additional product candidates in 2021:

- TX200, our wholly-owned Chimeric Antigen Receptor, or CAR, engineered regulatory T cell, or CAR-Treg, cell therapy product candidate for the treatment of HLA-A2 mismatched kidney transplant rejection; and
- KITE-037, our allogeneic anti-CD19 CAR-T cell therapy product candidate for the treatment of cancer. We are developing KITE-037 with our collaborator Kite Pharma Inc., or Kite, a wholly-owned subsidiary of Gilead Sciences, Inc.

Moreover, we are focusing our preclinical development in emerging areas for us including CAR-Treg cell therapies for autoimmune disorders and genome engineering for neurological diseases. Indications for our other preclinical programs include neurodevelopmental disorders, cancer, inflammatory bowel disease, or IBD, tauopathies such as Alzheimer's and neurodegenerative diseases such as amyotrophic lateral sclerosis, or ALS, multiple sclerosis, or MS, and Huntington's disease, some of which we are developing with our collaborators Biogen MA, Inc. and Biogen International GmbH, which we refer to together as Biogen, Kite, Novartis Institutes for BioMedical Research, Inc., or Novartis, Pfizer and Takeda Pharmaceutical Company Limited, or Takeda.

Our multiple collaborations with biopharmaceutical companies bring us important financial and strategic benefits and reinforce the potential of our research and development efforts and our zinc finger protein, or ZFP, technology platform. They leverage our collaborators' therapeutic and clinical expertise and commercial resources with the goal to bring our medicines more rapidly to patients. We believe these collaborations reflect the value of our ZFP technology platform and will potentially expand the addressable markets of our product candidates. To date, we have received approximately \$815.0 million in upfront licensing fees, milestone payments and proceeds from sale of our common stock to collaborators and have the right to earn up to \$7.0 billion in future milestone payments from our collaborations, in addition to potential product royalties.

##### *Our Novel Science*

We are a leader in the research and development of ZFPs, which are naturally occurring sequence specific DNA binding proteins found in humans and other species. We have developed a proprietary synthetic ZFP technology platform with potential clinical utility in (i) genome editing and genome regulation, which we refer to together as genome engineering and (ii) gene-edited cell therapy, which we refer to as cell therapy.

Our strategy is to translate our differentiated and versatile ZFP technology platform to product candidates with best- or first-in-class clinical potential. For example, ZFPs can be engineered to make zinc finger nucleases, or ZFNs, which are proteins that can be used to edit genomes by specifically modifying DNA sequences by knocking in or knocking out select genes. ZFPs can also be engineered to make ZFP transcription factors, or ZFP-TFs, which are proteins that can be used to regulate genomes by selectively increasing or decreasing gene expression.

In the process of developing these genome engineering technologies, we have additionally accrued significant scientific, manufacturing and development capabilities, as well as related know-how, that are broadly applicable to the field of gene therapy, which we have used to develop our gene therapy product candidates.

Finally, we have also leveraged our ZFP technology platform and technologies obtained through acquisitions to become a leader in researching and developing CAR-Treg product candidates for the treatment of autoimmune and inflammatory diseases in broad patient populations, including kidney transplant rejection, MS and IBD. CAR-Tregs are considered to have enhanced suppressive function over polyclonal Tregs due to the antigen-specificity introduced by the CAR.

#### *Our In-House Manufacturing*

We believe that our current and future in-house manufacturing capacity provides us a competitive advantage. We currently operate an in-house adeno-associated virus, or AAV, manufacturing facility in our Brisbane, California headquarters, and we are building cell therapy manufacturing facilities in Brisbane, California and Valbonne, France, which we expect to be operational by the end of 2021. Our manufacturing strategy is to provide greater flexibility, quality and control by building a balanced and necessary capacity achieved through our in-house manufacturing and contract manufacturing organization, or CMO, partnerships, investing in manufacturing processes and analytics and developing a strong supply chain.

### **Business Updates**

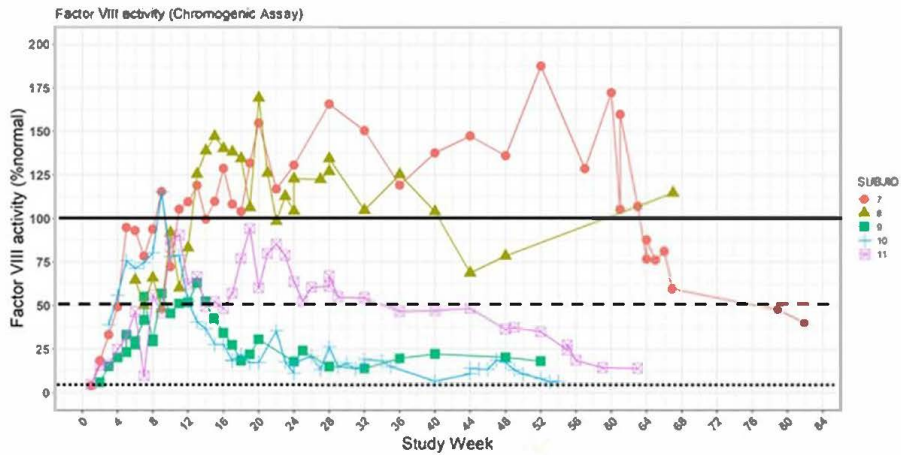
#### *Giroctocogene Fitelparvovec –Hemophilia A*

In December 2020, we and Pfizer jointly announced updated follow-up data from the Phase 1/2 Alta study of giroctocogene fitelparvovec, our investigational gene therapy for the treatment of severe hemophilia A. As of the August 31, 2020 cutoff date, all five patients in the high dose 3e13 vg/kg cohort had at least one year of follow-up, with 85 weeks of follow-up for the longest treated patient. The results showed that all five patients showed sustained factor VIII, or FVIII, activity levels, with a group median FVIII activity of 56.9% and a group geometric mean FVIII activity of 70.4% via chromogenic assay from week nine to 52. Steady-state FVIII activity was achieved for all patients in this high dose cohort within nine weeks of treatment with giroctocogene fitelparvovec, with no bleeding events and no FVIII infusions (beyond three weeks post-infusion) within the first year. More detailed information about FVIII activity levels in the high dose cohort as of the August 31, 2020 cutoff date are presented in the tables below. As of the cutoff date, one patient had one target joint bleed requiring FVIII therapy, occurring after week 52.

Giroctocogene fitelparvovec was generally well tolerated. One patient in the high dose cohort had a treatment-related serious adverse event of hypotension (grade 3) and fever (grade 2), with symptoms of headache and tachycardia, which occurred six hours post-infusion with giroctocogene fitelparvovec, and which fully resolved within 24 hours. No other treatment-related serious adverse events were reported as of the cutoff date. Among the five patients in the high dose cohort, four received corticosteroids for liver enzyme (alanine aminotransferase, or ALT) elevations. Three patients had subsequent ALT elevations that responded to corticosteroids. All episodes of ALT elevations fully resolved with oral corticosteroids, and as of the cutoff date, no participants were on corticosteroids and no corticosteroid use had been initiated after week 52. Pfizer expects to present two-year follow-up data from the Alta study within the next year.

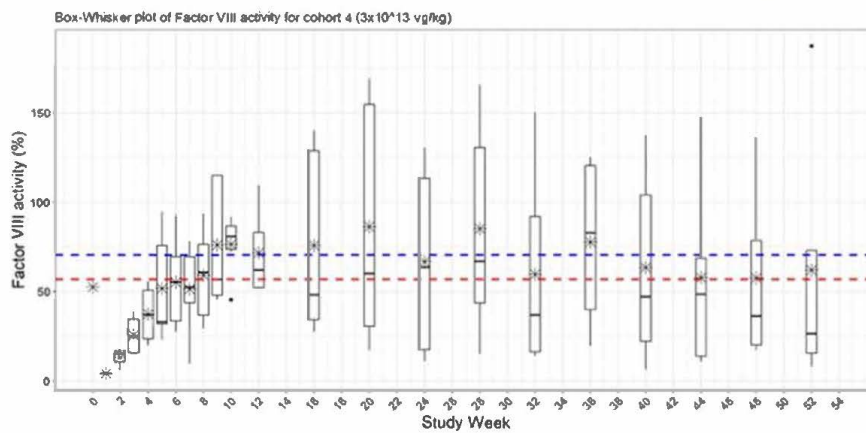
## Efficacy – Cohort 4 (3e13 vg/kg)

FVIII Activity as Measured at Central Laboratory With Chromogenic Assay



Latest available FVIII values from August 2020 data cut.  
FVIII, factor VIII; vg, vector genomes.

## Efficacy – Central Laboratory, Chromogenic Assay



-- Mean FVIII activity from week 9 to week 52 (based on group mean): 70.4% (SD=7.3%)  
-- Median FVIII activity from week 9 to week 52 (based on group median): 56.9% (SD=14.2%)  
 \* Mean (box and whisker plot)    — Median (box and whisker plot)    • Outliers

Week 52: n=4 participants  
1 participant had FVIII measured at week 67 instead of week 52 due to COVID-19 restrictions

Data cut: 31 August 2020.  
FVIII, factor VIII; vg, vector genomes; SD, standard deviation.

In October 2020, we and Pfizer jointly announced that we dosed the first participant in our registrational Phase 3 AFFINE clinical trial of giroctocogene fitelparvovec. AFFINE is a global Phase 3, open-label, multicenter, single arm trial evaluating the efficacy and safety of a single infusion of giroctocogene fitelparvovec in more than 60 adult (ages 18-64 years) male patients with moderately severe to severe hemophilia A. The primary endpoint is impact on annual bleed rate, or ABR, through 12 months following treatment with giroctocogene fitelparvovec, compared to ABR on FVIII replacement therapy collected in the Phase 3 lead-in study period. Pfizer anticipates a pivotal data readout from this trial in 2022. We received a \$30.0 million milestone from Pfizer in connection with the initiation of the AFFINE trial and have the potential to earn up to \$245.0 million in future milestone payments plus tiered royalties of 14%-20% on potential future product sales.

### *ST-920 – Fabry Disease*

In August and September 2020, we dosed the first two patients in our Phase 1/2 STAAR study evaluating our ST-920 gene therapy product candidate to treat Fabry disease, a rare inherited metabolic disease. These first two patients comprise the first of three dose-escalation cohorts in the study. In February 2021, we dosed the first patient in the second cohort. STAAR is an open-label, multicenter, dose-ascending clinical study evaluating the safety and tolerability of ST-920 in adult males with classic Fabry disease. We expect to present initial clinical data from the STAAR study by the end of 2021.

### *BIVV003 – Sickle Cell Disease & ST-400 – Transfusion Dependent Beta Thalassemia*

By the end of 2021, we and Sanofi expect to present clinical data from our Phase 1/2 PRECIZN-1 study evaluating our BIVV003 cell therapy product candidate for the treatment of sickle cell disease. We anticipate presenting at the same time follow-up clinical data from our Phase 1/2 Thales study evaluating ST-400, our cell therapy product candidate for the treatment of transfusion dependent beta thalassemia.

### *TX200 – HLA-A2 Mismatched Kidney Transplant Rejection*

We continue to seek the regulatory approvals necessary to initiate our Phase 1/2 STEADFAST clinical study evaluating our TX200 CAR-Treg cell therapy product candidate to treat HLA-A2 mismatched kidney transplant rejection. We expect to initiate the study in the second half of 2021. We expect that dosing of the first patient in this study will occur several months after initiation and patient enrollment.

### *Genome Engineering – ALS & FTLT*

We are developing genome engineering product candidates with Pfizer that use ZFP-TFs to treat ALS and frontotemporal lobar degeneration, or FTLT, linked to mutations of the *C9ORF72* gene. In September 2020, we completed our research obligations under the collaboration, and in November 2020, we received a \$5.0 million milestone from Pfizer associated with this program.

### *Genome Engineering – Prion Disease*

We continue to advance our wholly-owned preclinical genome engineering program in prion disease, a fatal and incurable neurodegenerative disease caused by the misfolding of the prion protein encoded by the gene *PRNP*.

## **INTRODUCTION TO OUR TECHNOLOGY**

Our strategy is to translate our differentiated and versatile ZFP technology platforms to product candidates with best- or first-in-class clinical potential. We believe that the versatility and flexibility of our technology platforms enables us to design therapeutic approaches to resolve the underlying genetic or cellular causes of disease, using whichever technology is best suited to deliver that treatment. Our current emerging areas of focus in preclinical studies include genome regulation with our ZFP technology platform in the central nervous system, or CNS, diseases and CAR-Treg cell therapy for autoimmune diseases.

### **ZFPs: Naturally Occurring Sequence Specific DNA Binding Proteins in Humans**

ZFPs are naturally occurring sequence specific DNA binding proteins in humans that recognize and bind to a specific DNA sequence within or near a particular gene and causes expression of that gene to be “turned on” (activated) or “turned off” (repressed). ZFPs are the most common class of such naturally occurring proteins in a wide range of organisms from yeast to humans. Functional domains may be added to ZFPs that enable genome editing (with enzymes such as nucleases or integrases) or genome regulation (with activators and repressors) at a specific genomic site determined by the ZFP DNA-binding domain.



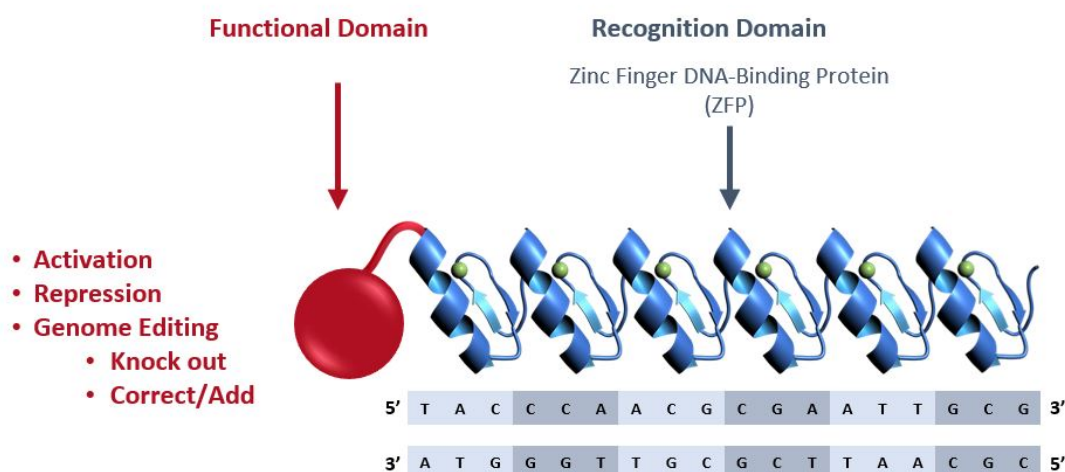


Figure 1:

*Schematic of the two-domain structure of a ZFP and its therapeutic functional domain*

Consistent with the structure of natural ZFPs, we take a modular approach to the design of the proteins that we engineer. The ZFP portion of our engineered proteins, the DNA-recognition domain, is typically composed of four to six zinc fingers. Each individual finger recognizes and binds to a three or four base pair sequence of DNA and multiple fingers can be linked together to recognize longer stretches of DNA, thereby improving specificity. By modifying the amino acid sequence of a ZFP, we can engineer novel ZFPs capable of recognizing the unique DNA sequences of a chosen genomic target. The engineered ZFP DNA-binding domain is then linked to a functional domain. The ZFP DNA-binding domain brings this functional domain to the target of interest. Our ability to use our highly specific ZFPs to precisely target a DNA sequence to a gene of interest provides us with a range of genome editing and genome regulation functionalities that can be applied to multiple cell types.

Our engineered ZFPs can be attached to a cleavage domain of a restriction endonuclease, an enzyme that cuts DNA, creating a ZFN. When a pair of ZFNs binds DNA in the correct orientation and spacing, a cut is introduced into the DNA sequence between the ZFP binding sites. DNA binding by both ZFNs is necessary for cleavage, and the two halves of the endonuclease must be present in the correct orientation to interact with one another in order to mediate DNA cleavage. This break in the DNA triggers a natural process of DNA repair within the cell. This endogenous DNA repair process may be harnessed to achieve one of several outcomes that may be therapeutically useful (Figure 2). If cells are treated with ZFNs alone, the repair process joins the two ends of the broken DNA together and frequently results in the loss (deletion) or addition (insertion) of a small amount of genetic material at the site of the break. These insertions and deletion events are collectively known as “indels”. These disrupt the target DNA sequence and result in the expression of a truncated or non-functional protein from the targeted gene, effectively “knocking out” the gene function. ZFN-mediated genome editing can be used to disrupt genes that are involved in disease pathology. We are using ZFN-mediated genome editing of the *BCL11A* erythroid specific enhancer, or ESE, in hematopoietic stem progenitor cells, or HSPCs, as the basis of a potential long-lasting and once only treatment for beta thalassemia (ST-400) and SCD (BIVV003).

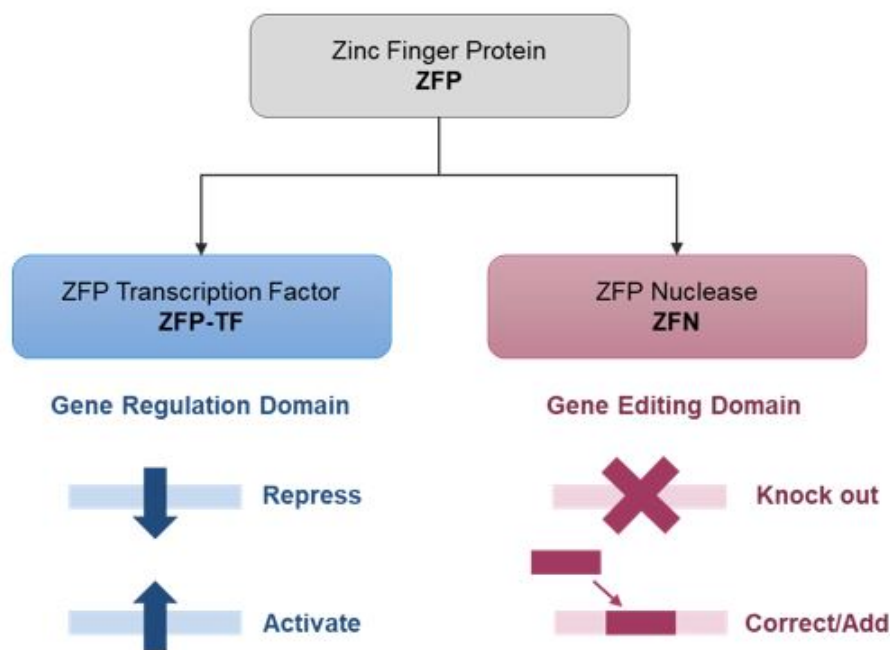


Figure 2:

*Schematic of ZFP genome editing and genome regulation*

In contrast, if cells with a mutation in a particular gene are treated not only with ZFNs, but also with an additional DNA sequence that encodes the correct gene sequence (referred to as a “donor” DNA) and with ZFNs that recognize and bind to sequences flanking the mutation, the cell’s repair machinery can use the donor DNA as a template to correct the mutated gene. This ZFN-mediated gene correction enables the corrected gene to be expressed in its natural chromosomal context and may provide a novel approach for the precise repair of DNA sequence mutations responsible for certain monogenic diseases. In addition to providing a donor sequence that encodes a complete gene, a new copy of a gene can also be precisely added into the genome at a specific location. The ability to precisely place a gene-sized segment of DNA specifically into a pre-determined location in the genome broadens the range of mutations of a gene that can be corrected in a single step. It also reduces the insertional mutagenesis concerns associated with traditional integrating gene replacement approaches such as lentiviruses, in which the insertion of a new corrective copy of the gene typically occurs at random locations in the genome.

We are also evaluating ZFP-TFs, which have the potential to regulate the expression of a target gene (Figure 2). For instance, attaching an activation domain to a ZFP will cause a target gene to be expressed at increased levels, relative to an untreated cell. Alternatively, a repression domain causes the gene to be downregulated or completely turned off. We have several preclinical programs evaluating the potential of ZFP-TFs that have been designed to down regulate the expression of genes as potential treatments for CNS diseases, including a collaboration agreement with Biogen for Alzheimer’s disease and Parkinson’s disease, a collaboration with Takeda for Huntington’s disease and a collaboration with Pfizer for ALS. We also have a preclinical collaboration with Novartis evaluating the potential of ZFP-TFs to upregulate expression of genes as a potential treatment for autism spectrum disorders and intellectual disability.

### **ZFPs Provide Opportunity to Develop a New Class of Human Therapeutics**

We believe that our ZFP technology provides a unique and proprietary basis for a broad new class of drugs that have differentiated technical advantages over small-molecule drugs, protein pharmaceuticals, RNA-based therapeutics, conventional gene therapy approaches and other gene and genome editing platforms, potentially enabling us to develop therapies that address a broad range of unmet medical needs.

We can generate highly specific ZFNs for genome editing and ZFP-TFs for genome regulation using a range of proprietary methods. We are developing delivery strategies to administer these therapeutics, including using mRNA, AAV, adenovirus, plasmid, lipid nanoparticles and direct injection into brain tissue or into the cerebrospinal fluid. As more genes and DNA sequences are linked to specific diseases, we believe that the clinical breadth and scope of our ZFP therapeutic reagents will continue to expand.

### CAR-Tregs Have Potential to Address Autoimmune and Inflammatory Diseases

Tregs are a type of white blood cell and act as the key regulators of the immune system. Their natural role is to maintain immune homeostasis and prevent undesirable immune reactions to autoantigens (autoimmunity) or to antigens that are normally tolerated (food antigens, inhaled antigens, contact antigens and bacterial flora antigens). Tregs play the role of ‘peacekeepers’ containing other T cells before they become harmful to the organism, ensuring the immune system does not mistakenly attack healthy organs while still protecting the body from harm, e.g., from viruses and bacteria.

We are genetically re-programming Tregs *ex vivo* to add a CAR, to give Tregs the ability to target a specific protein, called an antigen. CAR-Tregs are thus re-programmed to recognize and accumulate in specific tissues where the antigen is being expressed and an immune-mediated disorder is occurring. Our preclinical research shows that CAR-Tregs can inhibit overactive immune cells within the body. Moreover, they have the potential to induce long-term immune tolerance – a state of non-reactivity by the immune system to a particular auto-antigen. We aim to develop therapies that can induce and restore immune tolerance to address a wide range of inflammatory and autoimmune diseases.

CARs are composed of three main parts (see Figure 3):

- The extracellular section is composed of a single chain variable fragment, or scFv, typically derived from a monoclonal antibody and designed to recognize the target antigen, and a spacer or hinge to add spatial flexibility.
- The transmembrane domain anchors the CAR in the plasma membrane.
- The intracellular section, made of signaling and co-stimulatory domains, transmits an intracellular signal upon recognition of the antigen by the scFv.

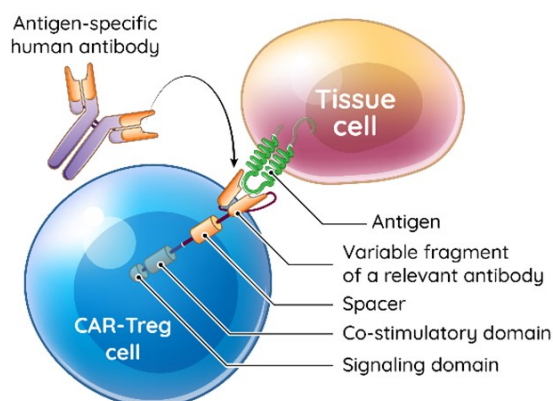


Figure 3:

*Schematic of CAR-Treg cell recognizing antigen on tissue cell, with detailed CAR structure*

We carefully select the CAR target antigen for each autoimmune or inflammatory indication. Our CAR-Treg cells are designed to be active only at the site of inflammation, ensuring specific and selective action. For instance, for a CNS disease such as MS, we want to make sure that the target antigen is localized in the CNS. The target antigen may in some instances be linked to the disease etiology.

A major feature of Tregs is that they can act via multiple mechanisms to mediate suppression. Their mechanism of action can be mediated upon cell contact, through soluble factors, metabolism disruption and/or cytolysis. The proposed mechanism of action for CAR-Tregs is shown in Figure 4.

- Following IV administration, CAR-Tregs are expected to migrate toward inflamed tissues due to Tregs' natural ability to migrate towards inflammatory tissues.
- Subsequently, CAR-Treg are expected to bind to their specific antigen, leading to the proliferation and activation of CAR-Treg cells.
- This activation is expected to allow Tregs to exert their natural anti-inflammatory and immuno-suppressive activities, acting through multiple molecular and cellular targets.

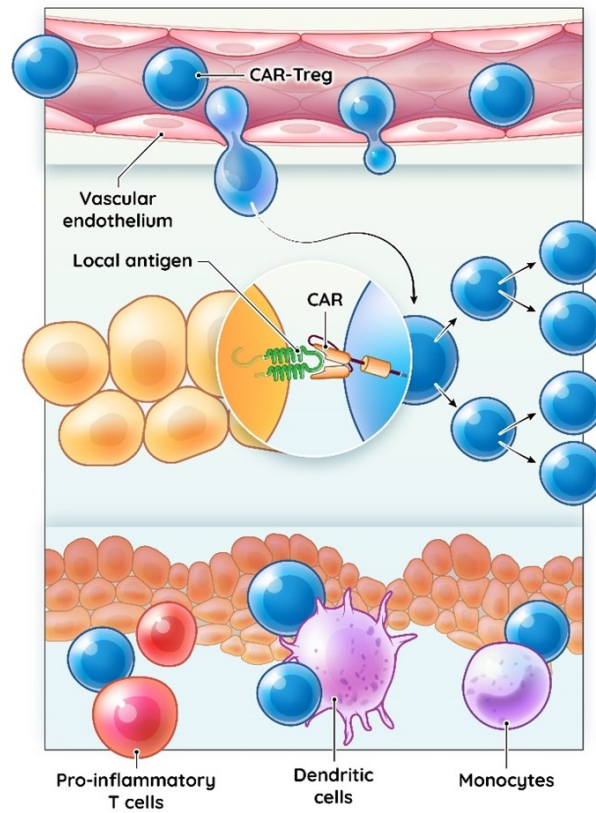


Figure 4:

*Schematic of the proposed mechanism of actions of CAR-Treg cells*

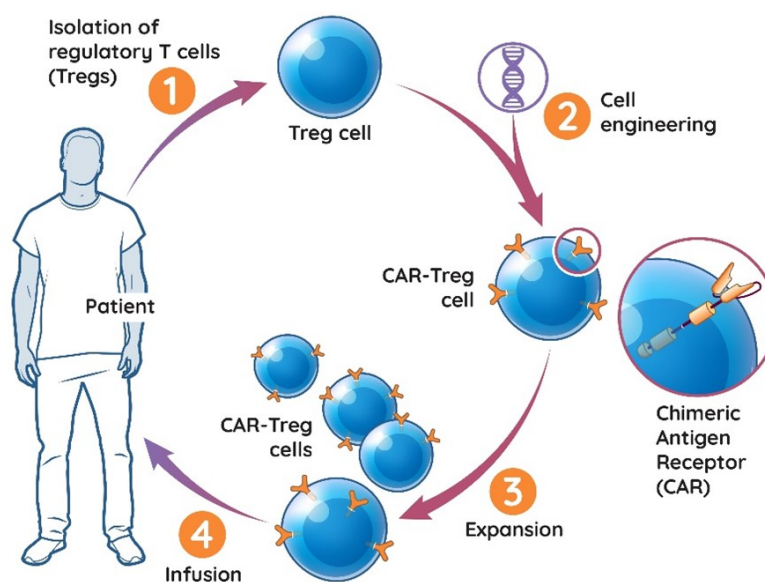


Figure 5:

*Schematic of our autologous CAR-Treg approach*

Our most advanced CAR-Treg product candidate, TX200, is being studied for the prevention of immune-mediated rejection following HLA-A2 mismatched kidney transplantation. TX200 is an autologous CAR-Treg cell therapy product candidate. An autologous cell therapy is made using cells from the same person as the recipient of the cells, as shown on Figure 5. The patient's Tregs are collected before transplant, genetically engineered with a CAR, and then injected back into the same patient. As a result of this detailed process, we expect dosing of patients will occur several months after their enrollment. The CAR in TX200 is designed to recognize the HLA-A2 protein present on the transplanted kidney.

We plan to initiate a clinical study in 2021 to evaluate the safety, tolerability, and mechanism of action of TX200 in patients who have received a kidney transplant. Our STEADFAST clinical study will help us understand how CAR-Tregs work in humans and may provide broader proof of concept for genetically modified cell therapy using Tregs.

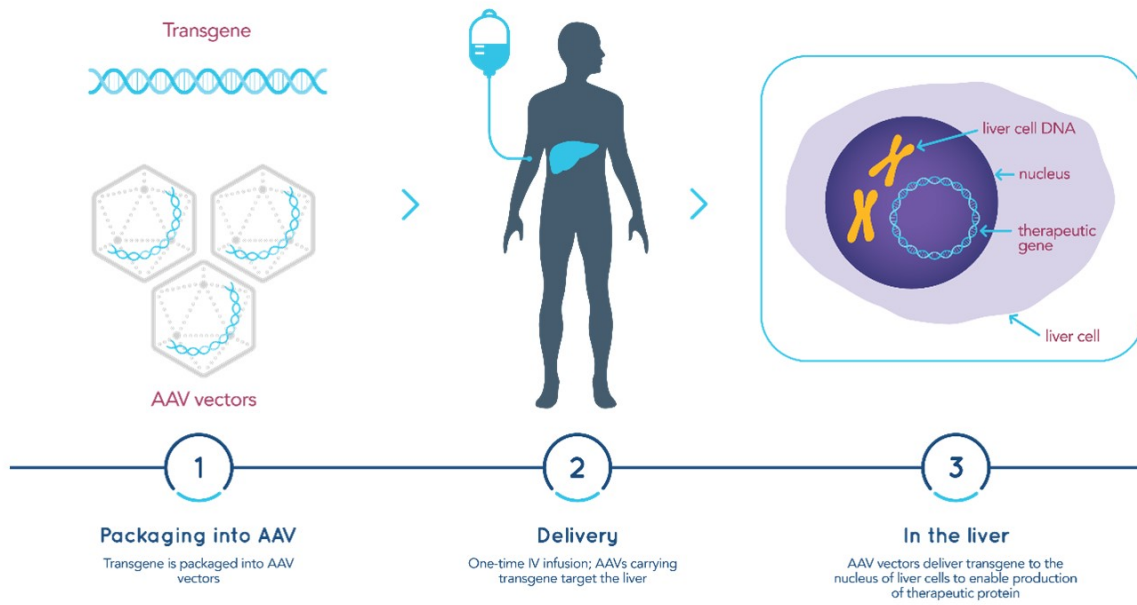
We are convinced of the fundamental impact of our CAR-Treg approach and are initiating the next step with the goal to make approach available to larger group of patients. Accordingly, we are developing ZFN-edited allogeneic Treg therapies. Allogeneic cell therapies are donor derived, made using cells from a different person to the recipient of the cells, as opposed to autologous cell therapies. We believe that allogeneic therapies are the future of cell therapy and will overcome the challenges of autologous approaches such as scale and manufacturing. If we are able to demonstrate proof-of-concept of autologous TX200, we anticipate follow-on allogeneic programs for kidney transplant rejection, IBD such as Crohn's disease and MS, all of which are currently in preclinical development. There is tremendous potential from there to go into many other large autoimmune indications such as rheumatoid arthritis or diabetes.

### **Gene Therapy Introduces Genes into a Patient's Cells to Treat Genetic Diseases**

In the process of developing our ZFP technologies, we have refined our understanding of gene therapies. Gene therapy is the treatment of disease by delivery of a new gene into a patient's cells to replace an incorrect or damaged gene. Most often, gene therapy works by introducing a corrected copy of a defective gene into the patient's cells, without removing or modifying DNA. The goal of gene therapy is to treat, or potentially cure, a genetic disease by adding back a normal copy of the gene responsible for the disease.

In gene therapy, we can deliver a therapeutic gene by engineering a deactivated virus to deliver DNA for a human therapeutic protein rather than viral proteins. One virus that is commonly used in gene therapy is adeno-associated virus, or AAV. AAV is a naturally occurring virus that infects humans but is not known to cause disease. Engineered AAV has been used as a delivery method for gene therapy in many clinical trials in the U.S. and Europe and has been found thus far to be

generally well-tolerated without major side effects. A gene encoding a therapeutic protein can be packaged into AAV and delivered to cells in tissues such as the liver, the eye, the brain or the heart. Once inside the cell, the gene is unpacked from the virus coat, or capsid, and can then enable that cell to make the therapeutic protein. AAV can be manufactured at a large enough scale for use as a human therapeutic.

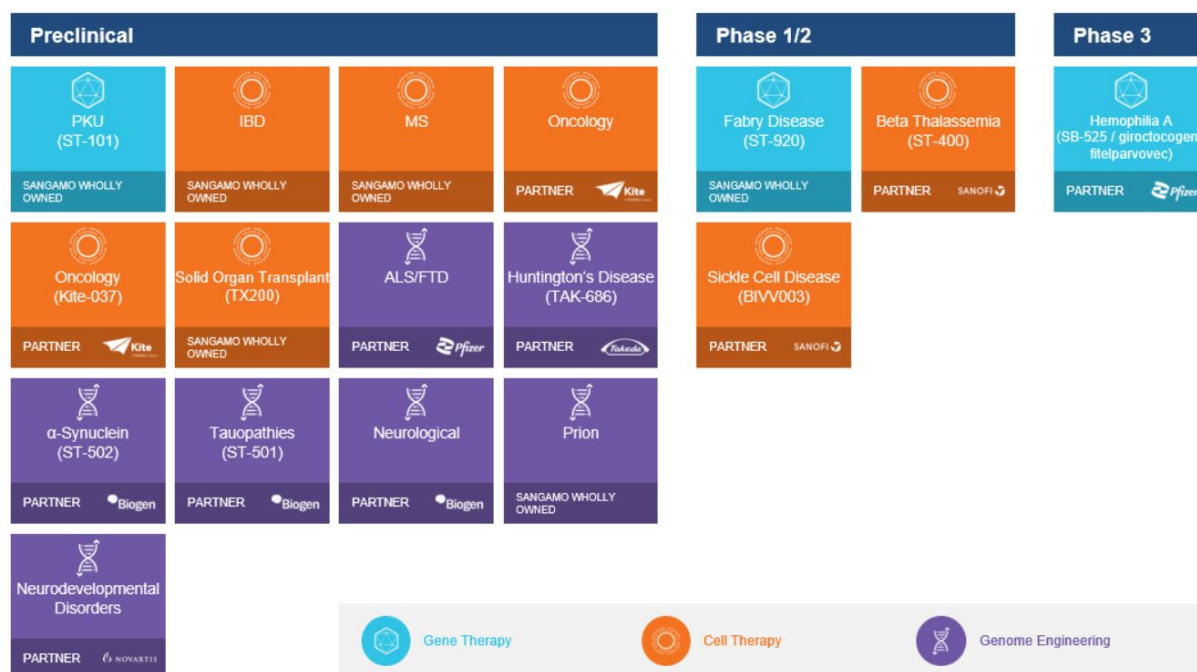


*Figure 6:*  
*Our gene therapy technology*

## **THERAPEUTIC PRODUCT DEVELOPMENT**

We are a clinical-stage genomic medicine company committed to translating ground-breaking science into medicines that transform the lives of patients with serious diseases. We plan to deliver on this mission through (i) development of our clinical and preclinical product candidates, (ii) our novel science and (iii) our in-house manufacturing capabilities.

## Our Product Candidates



## Proprietary Programs

### ST-920 - Fabry Disease

In August and September of 2020, we dosed the first two patients in our open label Phase 1/2 STAAR study evaluating our ST-920 gene therapy product candidate to treat Fabry disease, a rare inherited metabolic disease. These first two patients comprise the first of three dose-escalation cohorts in the study. In February 2021, we dosed the first patient in the second cohort. We are currently evaluating ST-920 in adult males with classic Fabry disease who are on an enzyme replacement therapy, or ERT, regimen; are ERT naïve; or are ERT pseudo naïve and have not received ERT treatment in the six months prior to the study. The goal of the study is to abrogate the need for ERT with a recombinant AAV2/6 vector encoding cDNA for human  $\alpha$ -Gal A, resulting in long-term expression of  $\alpha$ -Gal A. The primary endpoint of the trial is safety and tolerability. The secondary endpoints include pharmacodynamics of  $\alpha$  Gal A and the presence of its substrates in plasma over time; impact on ERT administration; impact on renal function; and vector DNA shedding over time. We will also be conducting a long-term follow-up study to monitor patients for an additional four years. We expect to present initial clinical data from the STAAR study by the end of 2021.

As a liver-directed gene therapy, ST-920 is designed to be delivered by a one-time IV infusion that does not require any preconditioning regimen for patients. We believe ST-920 has the potential to deliver efficacy with preserved renal function and reduced cardiac morbidity and neuropathy.

### TX200 - HLA-A2 Mismatched Kidney Transplant Rejection

TX200 is our autologous HLA-A2 specific CAR-Treg cell therapy product candidate that we have developed for the prevention of immune mediated rejection following HLA-A2 mismatched renal transplantation. We continue to seek the regulatory approvals necessary to initiate the proof-of-concept Phase 1/2 STEADFAST clinical study and we expect to initiate the study in Europe in the second half of 2021. The STEADFAST study will be critical for our understanding of CAR-Treg pharmacology and biology in patients as well as establishing process development and manufacturing know-how.

TX200 has been developed for patients with end-stage renal disease or ESRD, receiving a kidney transplant, where the recipient of the kidney is HLA-A2 negative and the donor is HLA-A2 positive. A kidney transplant is considered the best

treatment option for ESRD, the last stage of chronic kidney disease, when a person's kidneys are no longer working. HLA mismatch is the initial and most important barrier to successful transplantation after ABO blood types incompatibility, and approximately 20-25% of transplanted organs are HLA-A2 mismatched. In the case of an HLA-A2 positive kidney transplanted into an HLA-A2 negative patient, the recipient's immune system can recognize this mismatch and, without long-term immunosuppressive medication, will attack the new kidney carrying the HLA-A2 protein, leading to graft rejection. A lifetime of immunosuppressive therapy is associated with significant morbidity and mortality, including the development of systemic infection, malignancy and cardiovascular disease, the leading cause of death in this patient population. Therefore, the induction of immunological tolerance defined a stable and acceptable graft function without the need for immunosuppression remains a key priority in this field of medicine.

TX200 is composed of autologous Treg cells engineered to express an HLA-A2 CAR, allowing them to localize to the renal graft and activate upon recognition of the HLA-A2 antigen. We believe that TX200 has the potential to prevent kidney rejection by binding to the HLA-A2 positive kidney and inducing immune tolerance.

Similar to other genetically engineered cell therapy approaches, patients will undergo a leukapheresis procedure, from which their Treg cells will be isolated and engineered then cryopreserved. The HLA-A2 negative patient will subsequently undergo transplantation surgery to receive a kidney from an HLA-A2 positive living donor. Following a recovery period, the transplant recipient will receive their personalized TX200 drug candidate. As a result of this detailed process, we expect dosing of patients will occur several months after study initiation and patient enrollment.

Preclinical data supporting the STEADFAST study that we presented in October 2020 showed that the TX200 HLA-A2 CAR-Tregs efficiently prevented rejection in both graft versus host disease and skin transplantation models. They were also shown to be safe and well tolerated in our *in vivo* studies.

Our goal is that TX200 establishes the foundation for a portfolio of CAR-Tregs for major autoimmune indications. We believe that allogeneic therapies are the future of cell therapy and will overcome the challenges of autologous approaches such as scale and manufacturing. If we are able to demonstrate proof-of-concept of autologous TX200, we will continue to advance our kidney transplant allogeneic program which is currently in preclinical development.

#### *CAR-Treg Cell Therapy - IBD*

We continue to advance preclinical development of our wholly-owned CAR-Treg program to treat IBD. IBD covers debilitating disorders that involve chronic inflammation of the digestive tract, including ulcerative colitis and Crohn's disease. Our product candidate to treat IBD is composed of allogeneic Treg cells engineered to express a CAR designed to recognize an antigen relevant to IBD, so that it allows resulting CAR-Treg cells to localize and activate in the gut.

#### *CAR-Treg Cell Therapy - MS*

We continue to advance preclinical development of our wholly-owned CAR-Treg program to treat MS, an autoimmune disease of the CNS. Similarly to our IBD program, our product candidate to treat MS is composed of allogeneic Treg cells engineered to express a CAR designed to recognize an antigen relevant to MS, so that resulting CAR-Tregs can localize and activate in the CNS.

#### *ST-101 - PKU*

We continue to advance preclinical development of ST-101, our wholly-owned investigational gene therapy product candidate to treat phenylketonuria, or PKU, a rare inherited disorder that originates from a defect in the *PAH* gene and results in harmful accumulation of phenylalanine in cells throughout the body.

### **Partnered Programs**

#### *Giroctocogene Fitelparvovec - Hemophilia A*

We and Pfizer continue to develop giroctocogene fitelparvovec, which is currently being evaluated in our registrational Phase 3 AFFINE clinical trial as an investigational gene therapy for severe hemophilia A. Under our collaboration agreement with Pfizer, we conducted the Phase 1/2 clinical study and certain manufacturing activities, while Pfizer is responsible for subsequent worldwide development, manufacturing, marketing and commercialization.



In 2017, we initiated the Phase 1/2 Alta study to evaluate the safety and efficacy of giroctocogene fitelparvovec, also known as SB-525, in adults with severe hemophilia A. The Alta study is an open label, ascending dose Phase 1/2 clinical study in 11 male patients with severe hemophilia A. In December 2020, we and Pfizer jointly announced updated follow-up data from the study, which were presented at the 62<sup>nd</sup> American Society for Hematology Annual meeting. As of the August 31, 2020 cut-off date, all five patients in the high dose 3e13 vg/kg cohort had at least one year of follow-up, with 85 weeks of follow-up for the longest treated patient, and showed sustained factor VIII, or FVIII, activity levels, with a group median FVIII activity of 56.9% and a group geometric mean FVIII activity of 70.4% via chromogenic assay from week nine to 52. Steady-state FVIII activity was achieved for all patients in the high dose cohort within nine weeks of treatment with giroctocogene fitelparvovec, with no bleeding events and no FVIII infusions (beyond three weeks post-infusion) within the first year. As of the cut-off date, one patient had one target joint bleed requiring FVIII therapy, occurring after week 52. Giroctocogene fitelparvovec was generally well tolerated. As previously reported, one patient in the high dose cohort had a treatment-related serious adverse event of hypotension (grade 3) and fever (grade 2), with symptoms of headache and tachycardia, which occurred six hours post-infusion with giroctocogene fitelparvovec, and which fully resolved within 24 hours. No other treatment-related serious adverse events were reported as of the cutoff date. Among the five patients in the high dose cohort, four received corticosteroids for liver enzyme (alanine aminotransferase, or ALT) elevations. Three patients had subsequent ALT elevations that responded to corticosteroids. All episodes of ALT elevations fully resolved with oral corticosteroids, and as of the cutoff date no participants were on corticosteroids and no corticosteroid use has been initiated after week 52. Pfizer expects to present two-year follow-up data from the Alta study within the next year.

In October 2020, we and Pfizer jointly announced that we dosed the first participant in the registrational Phase 3 AFFINE clinical trial of giroctocogene fitelparvovec. AFFINE is a global Phase 3, open-label, multicenter, single arm trial evaluating the efficacy and safety of a single infusion of giroctocogene fitelparvovec in more than 60 adult (age 18-64) male patients with moderately severe to severe hemophilia A. The primary endpoint is impact on ABR through 12 months following treatment with giroctocogene fitelparvovec, compared to ABR on FVIII replacement therapy collected in the Phase 3 lead-in study period. Participants will be analyzed throughout the five-year study period following the single infusion to further assess the durability and efficacy. Pfizer expects a pivotal data readout from this trial in 2022.

Based on the results from the Alta study, the FDA granted regenerative medicine advanced therapy, or RMAT, designation to giroctocogene fitelparvovec. RMAT designation is granted to regenerative medicine therapies intended to treat, modify, reverse, or cure a serious condition, for which preliminary clinical evidence indicates that the medicine has the potential to address an unmet medical need. The RMAT designation includes all the benefits of the fast track and breakthrough therapy designation programs, including early interactions with the FDA. The FDA also granted giroctocogene fitelparvovec Orphan Drug and Fast Track designation, and the European Medicines Agency, or EMA, granted it Orphan Medicinal Product designation.

#### *BIVV003 – Sickle Cell Disease & ST-400 – Transfusion Dependent Beta Thalassemia*

We and Sanofi continue to develop therapeutics for hemoglobinopathies, focused on SCD and beta thalassemia. We and Sanofi are currently evaluating BIVV003, our cell therapy product candidate for SCD, in the Phase 1/2 PRECIZN-1 study and ST-400, our cell therapy product candidate for transfusion dependent beta thalassemia, in the Phase 1/2 Thales study. Sanofi is responsible for all subsequent development, manufacturing, marketing and commercialization.

BIVV003 and ST-400 are related product candidates using the same technology involving genome editing of a patient's own hematopoietic stem progenitor cells using non-viral delivery of our ZFN technology designed to induce the synthesis of fetal hemoglobin. This is achieved by gene-edited knock out of the erythroid specific enhancer of the *BCL11a* gene, which encodes a strong repressor of the gamma globin gene. In SCD, increased fetal hemoglobin synthesis may provide the patient with functional hemoglobin and help downregulate the abnormal sickle hemoglobin that results in painful sickle cell crises and other disease feature. In beta thalassemia, if fetal hemoglobin is expressed at high enough levels, it may substitute for a patient's absent or impaired levels of beta globin.

We are conducting the Phase 1/2 Thales study, an open-label, single arm clinical study to evaluate the safety and efficacy of ST-400 in up to six patients with beta thalassemia. We have dosed five patients into the Thales study, and we presented preliminary results in December 2019. Sanofi announced in early 2020 that it had dosed the first patient in the Phase 1/2 PRECIZN-1 study, and that it continues to enroll patients into the study.

We and Sanofi will present a new analysis of the studies' data when the two studies have accumulated a sufficient number of patients and follow-up, which we expect to do by the end of 2021. We will not dose any additional patients in the Thales study until the data from both studies has been collected and analyzed.

#### *KITE-037 - Cancer*

We and Kite continue to develop cell therapies to treat cancer using our research to design ZFNs and viral vectors to disrupt and insert select genes in T cells and natural killer cells, or NK-cells, including the insertion of genes that encode CARs, T cell receptors, or TCRs, and NK-cell receptors, or NKRs, directed to mutually agreed targets. Kite is responsible for all clinical development, manufacturing, marketing and commercialization. Kite anticipates submitting an investigational new drug, or IND, application for KITE-037, our allogeneic anti-CD19 CAR-T cell therapy product candidate, in the first half of 2021, and it expects to initiate a clinical study on KITE-037 by the end of 2021.

#### *ST-501 – Tauopathies & ST-502 - Synucleinopathies*

We and Biogen continue to develop preclinical genome engineering therapies, including our ST-501 product candidate to treat tauopathies including Alzheimer’s disease, ST-502 to treat synucleinopathies including Parkinson’s disease and an additional therapy to treat an undisclosed neuromuscular target. Under our collaboration agreement with Biogen, it has exclusive rights to nominate up to nine additional targets over a target selection period of five years. This collaboration leverages ZFP-TFs to aim to modulate the expression of key genes involved in neurological diseases. Our preclinical studies using AAV vectors to deliver tau-targeted (ST-501) and alpha synuclein-targeted (ST-502) ZFP-TFs have demonstrated highly specific, potent and tunable repression of tau and alpha synuclein.

#### *Genome Engineering – Autism Spectrum Disorder and Neurodevelopmental Disorders*

We and Novartis continue to develop preclinical genome engineering therapies for three neurodevelopmental targets, including genes linked to autism spectrum disorder and intellectual disability. The collaboration leverages our ZFP-TFs to aim to upregulate the expression of key genes involved in neurodevelopmental disorders.

#### *Genome Engineering – ALS & FTLN*

We and Pfizer continue to develop preclinical genome engineering product candidates that use ZFP-TFs to treat ALS and FTLN linked to mutations in the *C9ORF72* gene. In September 2020, we completed our research obligations associated with this collaboration, which required us to identify, characterize and preclinically develop ZFP-TFs satisfying pre-agreed criteria. Pfizer is now responsible for subsequent research and development activities as well as subsequent development, manufacturing, marketing and commercialization.

#### *TAK-686 – Huntington’s Disease*

We and Takeda continue to develop a preclinical genome engineering product candidate to treat Huntington’s Disease that uses a ZFP-TF designed to differentially down regulate the mutated disease-causing huntingtin gene, or *HTT* gene, while preserving the expression of the normal version of the gene. Takeda continues to advance preclinical IND enabling studies.

For more information on the collaborations underlying these partnered programs, see “—Collaborations” below.

### **Legacy Clinical Research Programs**

We have stopped development of the following clinical research programs. We continue to perform the appropriate long-term follow-up and closeout activities of the legacy studies in accordance with the study protocols.

#### *SB-728– Human Immunodeficiency Virus, or HIV*

We have recently stopped development in HIV.

SB-728 was one of the first clinical candidates to use an early generation of our ZFN-mediated genome editing technology. We conducted several clinical studies evaluating SB-728, demonstrating the safety of the platform and showing immune responses from a subset of patients, however the studies did not meet our clinical expectations.

#### *SB-913 - MPS II*

In January 2021, we announced that we have stopped development of SB-913, our ZFN genome editing product candidate for the treatment of Mucopolysaccharidosis Type II, or MPS II. While the Phase 1/2 CHAMPIONS study evaluating SB-913 demonstrated the first molecular evidence of genome editing, the study did not meet our clinical expectations. Research

is ongoing to improve the potency and delivery of our ZFNs for genome editing, which we believe will optimize the platform for therapeutic effect.

#### *SB-318 - MPS I & SB-FIX – Hemophilia B*

We have stopped development of SB-318 and SB-FIX, genome editing product candidates for the treatment of MPS I and hemophilia B, respectively.

### **COLLABORATIONS**

We have entered into strategic collaborations with larger biopharmaceutical companies for several of our therapeutic programs and other partnerships for several non-therapeutic applications of our technology. We will continue to pursue further collaborations when appropriate to fund internal research and development activities and to assist in product development, manufacturing, regulatory approval and commercialization. Decisions to collaborate or not will be based on review of our internal resources, institutional knowledge and commercial considerations.

#### ***Novartis***

In July 2020, we entered into a collaboration and license agreement with Novartis for the research, development and commercialization of gene regulation therapies to treat three neurodevelopmental disorders. Under the agreement, we granted to Novartis an exclusive, royalty bearing and worldwide license, under our relevant patents and know-how, to develop, manufacture and commercialize certain of our ZFP-TFs targeted to three undisclosed genes that are associated with neurodevelopmental disorders, including autism spectrum disorder and intellectual disability. We perform early research activities over the collaboration period for each gene target and manufacture the ZFP-TFs required for such research, costs of which are funded by Novartis. Novartis is responsible for additional research activities, IND-enabling studies, clinical development, regulatory approvals, manufacturing of preclinical, clinical and approved products, and global commercialization. Subject to certain exceptions set forth in the agreement, we are prohibited from developing, manufacturing or commercializing any therapeutic product targeting any of the three genes that are the subject of the collaboration. Novartis also has the option to license certain of our proprietary AAVs for the sole purpose of developing, manufacturing and commercializing licensed products arising from the collaboration.

Under the agreement, Novartis paid us a \$75.0 million upfront license fee payment in August 2020. In addition, we are eligible to earn from Novartis up to \$420.0 million in development milestones and up to \$300.0 million in commercial milestones. We are also eligible to earn from Novartis tiered high single-digit to sub-teen double-digit royalties on potential net commercial sales of licensed products arising from the collaboration. These royalty payments are subject to reduction due to patent expiration, loss of market exclusivity and payments made under certain licenses for third-party intellectual property. The agreement continues, on a product-by-product and country-by-country basis, until the expiration of the applicable royalty term. Novartis has the right to terminate the agreement, in its entirety or on a target-by-target basis, for any reason after a specified notice period. Each party has the right to terminate the agreement on account of the other party's bankruptcy or material, uncured breach.

#### ***Biogen***

In February 2020, we entered into a global licensing collaboration agreement with Biogen for the research, development and commercialization of gene regulation therapies for the treatment of neurological diseases which became effective in April 2020. Our collaboration aims to leverage our proprietary ZFP technology delivered via AAV to modulate expression of key genes involved in neurological diseases. Concurrently with the execution of the collaboration agreement, we also entered into a stock purchase agreement with Biogen MA, Inc., pursuant to which Biogen MA, Inc. purchased 24,420,157 shares of our common stock, or the Biogen Shares for an aggregate purchase price of \$225.0 million.

Under the collaboration agreement, Biogen paid us an upfront license fee payment of \$125.0 million. We are also eligible to earn research, development, regulatory and commercial milestone payments that could total up to approximately \$2.37 billion if Biogen selects all of the targets allowed under the agreement and all the specified milestones set forth in the agreement are achieved, which includes up to \$925.0 million in pre-approval milestone payments and up to \$1.45 billion in first commercial sale and other sales-based milestone payments. In addition, we are also eligible to receive tiered high single-digit to sub-teen royalties on potential net commercial sales of licensed products arising from the collaboration. These royalty payments are subject to reduction due to patent expiration, entry of biosimilar products to the market and payments made under certain licenses for third-party intellectual property.

Under the collaboration agreement, we granted to Biogen an exclusive, royalty bearing and worldwide license, under our relevant patents and know-how, to develop, manufacture and commercialize certain ZFP and/or AAV-based products directed to up to 12 neurological disease gene targets selected by Biogen. Biogen has already selected three of these: ST-501 for tauopathies including Alzheimer's disease, ST-502 for synucleinopathies including Parkinson's disease and a third undisclosed neuromuscular disease target. Biogen has exclusive rights to nominate up to nine additional targets over a target selection period of five years. For each gene target selected by Biogen, we perform early research activities, costs for which are shared by the companies, aimed at the development of the combination of proprietary CNS delivery vectors and ZFP-TFs targeting therapeutically relevant genes. Biogen then assumes responsibility and costs for the IND-enabling studies, clinical development, related regulatory interactions, and global commercialization. We are primarily responsible for GMP manufacturing activities for the initial clinical studies for the first three products of the collaboration and plan to leverage our in-house manufacturing capacity. Biogen is responsible for GMP manufacturing activities beyond the first clinical study for each of the first three products. Subject to certain exceptions set forth in the collaboration agreement, we are prohibited from developing, manufacturing or commercializing any therapeutic product directed to the targets selected by Biogen.

The collaboration agreement continues, on a product-by-product and country-by-country basis, until the expiration of the applicable royalty term. Biogen has the right to terminate the collaboration agreement, in its entirety or on target-by-target basis, for any reason after a specified notice period. Each party has the right to terminate this agreement on account of the other party's bankruptcy or material, uncured breach. In addition, we may terminate the collaboration agreement if Biogen challenges any patents licensed by us to Biogen.

Pursuant to the terms of the stock purchase agreement, Biogen has agreed not to, without our prior written and subject to specified conditions and exceptions, directly or indirectly acquire shares of our outstanding common stock, seek or propose a tender or exchange offer or merger between the parties, solicit proxies or consents with respect to any matter, or undertake other specified actions related to the potential acquisition of additional equity interests in us. Subject to customary exceptions, such standstill restrictions expire on the earlier of the three-year anniversary of the effectiveness of the Biogen collaboration agreement and the date that Biogen beneficially owns less than 5% of our common stock.

The stock purchase agreement also provides that until the first anniversary of the effectiveness of the Biogen collaboration agreement, Biogen must hold and not sell any of the Biogen Shares and from the first anniversary through the second anniversary, Biogen must hold and not sell at least 50% of the Biogen Shares, in addition to being subject to certain volume limitations. The stock purchase agreement further provides that, subject to certain limitations, upon Biogen's request, we must register for resale any of the Biogen Shares on a registration statement to be filed with the SEC, until such time as all remaining Biogen Shares may be sold pursuant to Rule 144 promulgated under the Securities Act during any 90-day period.

In addition, Biogen has agreed that, excluding specified extraordinary matters, it must vote the Biogen Shares in accordance with our recommendation and has granted us an irrevocable proxy with respect to the foregoing. Such voting provisions expire on the earlier of (i) the two-year anniversary of the effectiveness of the Biogen collaboration agreement, (ii) the date that Biogen beneficially owns less than 5% of our common stock and (iii) the date the Biogen collaboration agreement is terminated; provided, however, that in no event shall such expiration date be prior to the one-year anniversary of the effectiveness of the Biogen collaboration agreement.

#### **Kite**

In February 2018, we entered into a collaboration and license agreement with Kite, a wholly-owned subsidiary of Gilead, which became effective in April 2018 and was amended and restated in September 2019, for the research, development and commercialization of engineered cell therapies for cancer. Kite is responsible for all clinical development and commercialization of any resulting products. Kite anticipates an IND submission for KITE-037, our allogeneic anti-CD19 CAR-T cell therapy product candidate, in the first half of 2021, and it expects to initiate a clinical study on KITE-037 by the end of 2021.

Subject to the terms of this agreement, we granted Kite an exclusive, royalty-bearing, worldwide, sublicensable license, under our relevant patents and know-how, to develop, manufacture and commercialize, for the purpose of treating cancer, specific cell therapy products that may result from the research program and that are engineered *ex vivo* using selected ZFNs and AAVs developed under the research program, to express CARs, TCRs or NKRs directed to candidate targets.

During the research program term and subject to certain exceptions, except pursuant to this agreement, we are prohibited from researching, developing, manufacturing and commercializing, for the purpose of treating cancer, any cell therapy product that, as a result of *ex vivo* genome editing, expresses a CAR, TCR or NKR that is directed to a target expressed on or in a human cancer cell. After the research program term concludes and subject to certain exceptions, except pursuant to

this agreement, we are prohibited from developing, manufacturing and commercializing, for the purpose of treating cancer, any cell therapy product that, as a result of *ex vivo* genome editing, expresses a CAR, TCR or NKR that is directed to a candidate target.

We received a \$150.0 million upfront payment from Kite when the agreement became effective in April 2018. In addition, Kite reimburses our direct costs to conduct the joint research program, and Kite is responsible for all subsequent development, manufacturing and commercialization of any licensed products. We are also eligible to earn contingent development- and sales-based milestone payments that could total up to \$3.01 billion if all the specified milestones set forth in this agreement are achieved. Of this amount, approximately \$1.26 billion relates to the achievement of specified research, clinical development, regulatory and first commercial sale milestones, and approximately \$1.75 billion relates to the achievement of specified sales-based milestones if annual worldwide net sales of licensed products reach specified levels. Each development- and sales-based milestone payment is payable (i) only once for each licensed product, regardless of the number of times that the associated milestone event is achieved by such licensed product, and (ii) only for the first 10 times that the associated milestone event is achieved, regardless of the number of licensed products that may achieve such milestone event. In addition, we are entitled to receive escalating, tiered royalty payments with a percentage in the single digits based on potential future annual worldwide net sales of licensed products. These royalty payments are subject to reduction due to patent expiration, entry of biosimilar products to the market and payments made under certain licenses for third-party intellectual property.

Kite has the right to terminate this agreement, in its entirety or on a per licensed product or per candidate target basis, for any reason after a specified notice period. Each party has the right to terminate this agreement on account of the other party's bankruptcy or material, uncured breach.

## **Pfizer**

We have two separate collaboration agreements with Pfizer:

### *Giroctocogene Fitelparvovec Collaboration*

In May 2017, we entered into an exclusive, global collaboration and license agreement with Pfizer for the research, development and commercialization of giroctocogene fitelparvovec, also known as SB-525, our gene therapy product candidate for hemophilia A, and closely related products, which we amended in December 2019.

Under this agreement, we were responsible for conducting the Phase 1/2 clinical study and certain manufacturing activities for giroctocogene fitelparvovec, while Pfizer is responsible for subsequent worldwide development, manufacturing, marketing and commercialization of giroctocogene fitelparvovec. We may also collaborate in the research and development of additional AAV-based gene therapy products for hemophilia A.

We received an upfront license fee of \$70.0 million, achieved a \$25.0 million milestone in December 2019 upon completion of the transfer of the IND for giroctocogene fitelparvovec to Pfizer, and achieved a \$30.0 million in October 2020 upon the dosing of the first patient in our pivotal Phase III AFFINE trial. We are eligible to earn further development milestone payments on the achievement of specified clinical development, intellectual property, regulatory and first commercial sale milestones for giroctocogene fitelparvovec and potentially other products. The total amount of potential clinical development, intellectual property, regulatory, and first commercial sale milestone payments, assuming the achievement of all specified milestones in this agreement, is \$475.0 million, which includes up to \$300.0 million for giroctocogene fitelparvovec and up to \$175.0 million for other products that may be developed under the agreement, subject to reduction on account of payments made under certain licenses for third party intellectual property. In addition, Pfizer agreed to pay us royalties for each potential licensed product developed under the agreement that are an escalating tiered, double-digit percentage of the annual net sales of such product and are subject to reduction due to patent expiration, entry of biosimilar products to the market and payment made under certain licenses for third party intellectual property.

Subject to the terms of the agreement, we granted Pfizer an exclusive, worldwide, royalty-bearing license, with the right to grant sublicenses, to use certain technology controlled by us for the purpose of developing, manufacturing and commercializing giroctocogene fitelparvovec and related products. Pfizer granted us a non-exclusive, worldwide, royalty free, fully paid license, with the right to grant sublicenses, to use certain manufacturing technology developed under the agreement and controlled by Pfizer to manufacture our products that utilize the AAV delivery system. During a specified period, neither we nor Pfizer are permitted to clinically develop or commercialize, outside of the collaboration, certain AAV-based gene therapy products for hemophilia A.

Unless earlier terminated, the agreement has a term that continues, on a per product and per country basis, until the later of (i) the expiration of patent claims that cover the product in a country, (ii) the expiration of regulatory exclusivity for a product in a country, and (iii) 15 years after the first commercial sale of a product in a country. Pfizer has the right to terminate the agreement without cause in its entirety or on a per product or per country basis. The agreement may also be terminated by either party based on an uncured material breach by the other party or the bankruptcy of the other party. Upon termination for any reason, the license granted by us to Pfizer to develop, manufacture and commercialize giroctocogene fitelparvovec and related products automatically terminate. Upon termination by us for cause or by Pfizer in any country or countries, Pfizer is required to automatically grant us an exclusive, royalty-bearing license under certain technology controlled by Pfizer to develop, manufacture and commercialize giroctocogene fitelparvovec in the terminated country or countries.

#### *C9ORF72 Collaboration*

In December 2017, we entered into a separate exclusive, global collaboration and license agreement with Pfizer for the development and commercialization of potential gene therapy products that use ZFP-TFs to treat ALS and FTL D linked to mutations of the *C9ORF72* gene. Pursuant to this agreement, we agreed to work with Pfizer on a research program to identify, characterize and preclinically develop ZFP-TFs that bind to and specifically reduce expression of the mutant form of the *C9ORF72* gene.

We received a \$12.0 million upfront payment from Pfizer and achieved a \$5.0 million in September 2020 associated with the completion of all of our research activities for the *C9ORF72* collaboration. We are eligible to earn up to \$60.0 million in development milestone payments from Pfizer contingent on the achievement of specified preclinical development, clinical development and first commercial sale milestones, and up to \$90.0 million commercial milestone payments if annual worldwide net sales of the licensed products reach specified levels. In addition, Pfizer will pay us royalties based on an escalating tiered, mid- to high-single digit percentage of the annual worldwide net sales of the licensed products. These royalty payments are subject to reduction due to patent expiration, entry of biosimilar products to the market and payments made under certain licenses for third party intellectual property. Each party is responsible for the cost of its performance of the research program. Pfizer is operationally and financially responsible for subsequent development, manufacturing and commercialization of the licensed products.

Subject to the terms of the agreement, we granted Pfizer an exclusive, worldwide, royalty-bearing, license under our relevant patents and know-how to develop, manufacture and commercialize gene therapy products that use resulting ZFP-TFs that satisfy pre-agreed criteria. During a specified period, neither we nor Pfizer will be permitted to research, develop, manufacture or commercialize outside of the collaboration any ZFPs that specifically bind to the *C9ORF72* gene.

Unless earlier terminated, the agreement has a term that continues, on a per licensed product and per country basis, until the later of (i) the expiration of patent claims that cover the licensed product in a country, (ii) the expiration of regulatory exclusivity for a licensed product in a country, and (iii) 15 years after the first commercial sale of a licensed product in a major market country. Pfizer has the right to terminate the agreement without cause in its entirety or on a per product or per country basis. The agreement may also be terminated by either party based on an uncured material breach by the other party or the bankruptcy of the other party. The agreement will also terminate if we are unable to identify any lead candidates for development within a specified period of time or if Pfizer elects not to advance a lead candidate beyond a certain development milestone within a specified period of time. Upon termination for any reason, the license granted by us to Pfizer to develop, manufacture and commercialize licensed products under the agreement will automatically terminate. Upon termination by us for cause or by Pfizer without cause for any licensed product or licensed products in any country or countries, we will have the right to negotiate with Pfizer to obtain a non-exclusive, royalty-bearing license under certain technology controlled by Pfizer to develop, manufacture and commercialize the licensed product or licensed products in the terminated country or countries.

Following termination by us for Pfizer's material breach, either party will not be permitted to research, develop, manufacture or commercialize ZFPs that specifically bind to the *C9ORF72* gene for a period of time.

#### **Sanofi**

In January 2014, we entered into an exclusive worldwide collaboration and license agreement with Biogen MA, Inc., who subsequently assigned it to Bioverativ Inc., who was then subsequently acquired by Sanofi to develop therapeutics for hemoglobinopathies. Under the agreement, we are jointly conducting two research programs: the beta thalassemia program and the SCD program. In the beta thalassemia program, we are responsible for all discovery, research and development activities through the first human clinical study. In the SCD program, both parties are responsible for research and development activities through the submission of an IND application for ZFP therapeutics intended to treat SCD.

Under both programs, Sanofi is responsible for subsequent worldwide clinical development, manufacturing and commercialization of licensed products developed under the agreement. At the end of the specified research terms for each program or under certain specified circumstances, Sanofi has the right to step in and take over any of our remaining activities. Furthermore, we have an option to co-promote in the United States any licensed products to treat beta thalassemia and SCD developed under the agreement, and Sanofi will compensate us for such co-promotion activities. Subject to the terms of the agreement, we have granted Sanofi an exclusive, royalty-bearing license, with the right to grant sublicenses, to use certain ZFP and other technology controlled by us for the purpose of researching, developing, manufacturing and commercializing licensed products developed under the agreement. We have also granted Sanofi a non-exclusive, worldwide, royalty-free, fully paid license, with the right to grant sublicenses, under our interest in certain other intellectual property developed pursuant to the agreement. During the term of the agreement, we are not permitted to research, develop, manufacture or commercialize, outside of the agreement, certain gene therapy products that target genes relevant to the licensed products.

Under the agreement, we received an upfront license fee of \$20.0 million, achieved a \$6.0 million milestone in August 2019, upon dosing of the third subject in the ST-400 beta thalassemia Phase 1 clinical trial, achieved a \$7.5 million milestone in December 2019 upon dosing of the first subject in the SCD Phase 1 clinical study, and are eligible to earn additional development and sales milestone payments upon the achievement of specified regulatory, clinical development and sales milestones. The total amount of potential regulatory, clinical development, and sales milestone payments, assuming the achievement of all specified milestones in the agreement, is \$276.3 million. In addition, we will receive royalty payments for each licensed product that are a tiered double-digit percentage of annual net sales of each product.

The agreement may be terminated by (i) us or Sanofi for the uncured material breach of the other party, (ii) us or Sanofi for the bankruptcy or other insolvency proceeding of the other party; (iii) Sanofi, upon 180 days' advance written notice to us and (iv) Sanofi, for certain safety reasons upon written notice to, and after consultation with, us.

### **Takeda**

In January 2012, we entered into a collaboration and license agreement with Shire International GmbH, a wholly-owned subsidiary of Takeda, which we amended and restated in September 2015, to research, develop and commercialize human therapeutics and diagnostics for monogenic diseases based on our ZFP technology. We received an upfront license fee of \$13.0 million in 2012 and achieved a \$1.0 million milestone in 2014. Pursuant to the amended and restated agreement, Takeda has an exclusive, worldwide license to ZFP therapeutics for treating Huntington's disease.

Under the amended and restated agreement, Takeda has full control over, and full responsibility for the costs of, the Huntington's disease program, subject to certain obligations, including the obligation to retain us to perform ZFP design, optimization and assessment services and to reimburse us for the costs of such services. Takeda does not have any milestone payment obligations but is required to pay single digit percentage royalties to us, up to a specified maximum cap, on the commercial sales of ZFP therapeutic products for Huntington's disease. During the term of the amended and restated agreement, we are not permitted to research, develop or commercialize, outside of the agreement, certain products that target the *HTT* gene.

Under the amended and restated agreement, we have full control over, and full responsibility for the costs of, the hemophilia A and B programs returned to us by Takeda, subject to certain diligence obligations. We also granted Takeda a right of first negotiation to obtain a license to such programs under certain circumstances. We are required to pay single digit percentage royalties to Takeda, up to a specified maximum cap, on commercial sales of therapeutic products from the programs returned to us by Takeda.

The amended and restated agreement may be terminated by (i) us or Takeda, in whole or in part, for the uncured material breach of the other party, (ii) us or Takeda for the bankruptcy or other insolvency proceeding of the other party and (iii) Takeda, in its entirety, effective upon at least 90 days' advance written notice.

### **Other Partnerships**

In addition to our partnerships for the development of human therapeutic applications, we have also licensed our technology in several other areas, such as plant agriculture and research reagents, including the production of transgenic animals and cell-line engineering. These license partners include Dow AgroSciences LLC, Sigma-Aldrich Corporation, Genentech, Inc., Open Monoclonal Technology, Inc. and F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc.

## INTELLECTUAL PROPERTY

Patents, trade secrets, know-how and licenses are important to our business. Our strategy includes filing and licensing patents and patent applications to protect technology, inventions and improvements to inventions that we consider important for the development and commercialization of our genome editing and genome regulation technologies and our potential products. We have filed numerous patent applications with the U.S. Patent and Trademark Office, or U.S. PTO, and foreign jurisdictions. Our proprietary intellectual property includes methods relating to the design of zinc finger proteins, Transcription Activator-Like Effector, or TALE, proteins and Clustered Regularly Interspaced Short Palindromic Repeats, or CRISPR/Cas editing systems, therapeutic applications of genome editing technology, enabling technologies related to our platform and the use of genome editing across a variety of applications. We rely on a combination of patent, copyright, trademark, proprietary know-how, continuing technological innovations and trade secret laws, as well as confidentiality agreements, materials transfer agreements, research agreements and licensing agreements, to establish and protect our proprietary rights.

### In-Licensed Technology

We have exclusively licensed certain intellectual property directed to the design, selection, and use of ZFPs, ZFNs and ZFP-TFs for genome editing and genome regulation from numerous academic institutions. Although no individual in-license is material to our overall protection of our ZFP and ZFN platforms, we believe in combination our in-licenses, in connection with our own knowhow, patent applications and patents protects us from third parties who might try to copy our products.

In addition, with respect to our cell therapy products, our subsidiary, Sangamo France, has a license agreement with the University of British Columbia pursuant to which it exclusively licensed the right to the CAR for use in our TX200 product candidate. This license includes one patent family, which expires in September 2038, absent any patent term adjustment, patent term extension or terminal disclaimers.

### Our Intellectual Property

In addition to our in-licensed patent portfolio, we have numerous issued patents and pending patent filings directed to the design, composition and use of ZFPs, ZFNs, ZFP-TFs, TALE proteins and CRISPR/Cas editing systems and other technologies related to our program.

Given our two-decade history with zinc finger technology, some of the earliest zinc finger patents in our portfolio began expiring in 2015. However, we have continued to build on this patent portfolio and have been issued additional patents and have applications pending that provide protection for our ZFP technology. Additionally, patents that may be issued from our pending applications will extend the patent exclusivity of our patent estate.

We believe that our licensed patents and patent applications, as well as our issued patents and pending patent applications, in the aggregate, will provide us with a substantial intellectual property position in our commercial development of our gene therapy, cell therapy and genome engineering programs. In this regard, patents issued to us, applied for by us, or exclusively and non-exclusively licensed to us, cover the following types of inventions, processes and products:

- *ZFP and ZFN design, engineered nucleases (e.g., CAS), and compositions (four patents issued with expiration dates ranging from 2029 to 2036), absent any PTA, PTE or TD):* Includes DNA target site selection, zinc finger binding domain design, nuclease domain design, linker design, DNA nickases, ZFP libraries databases and methods of construction, as well as methods to increase zinc finger binding specificity (see, e.g., US9982245, US10066242, US10113207);
- *ZFP Therapeutics (three patents issued with expiration dates ranging from 2028 to 2031, absent any PTA, PTE or TD):* Methods relating to activation and inhibition of endogenous genes, identification of accessible regions within chromatin, including treatment of Huntington's disease, HIV, cancer therapeutics, modulation of cardiac contractility and methods to regulate the glucocorticoid receptor (see, e.g., US9943565);
- *Nuclease Therapeutics (12 patents issued with expiration dates ranging from 2031 to 2036, absent any PTA, PTE or TD):* Treatments for HIV, beta thalassemia and SCD, hemophilia inherited metabolic diseases, genome editing, Parkinson's Disease, regulation of the expression of PD1; Immunomodulatory therapeutics; Cystic Fibrosis; CNS disease; Severe combined immunodeficiency, Modified T cells, including HLA knock out and methods of editing stem cells (see, e.g., US9877988, US9963715, US10072066, US10081661, US10143760); and
- *Non-Therapeutic Applications of ZFPs and Nucleases (seven patents issued with expiration dates ranging from 2028 to 2035, absent any PTA, PTE or TD):* Identification of regulatory sequences, analysis of gene regulation, structure and biological function, methods of agricultural biotechnology, methods of altering cellular differentiation state,



development of cell lines for improved protein production, methods of transgenic animal development, engineering of stem cells, methods of genome editing (see, e.g., US9890395).

The patent positions of biopharmaceutical companies, including our patent position, are uncertain and involve complex legal and factual questions for which important legal tenets are largely unresolved and are subject to interpretation and refinement by the court system. Patent applications may not result in the issuance of patents and the coverage claimed in a patent application may be significantly reduced before a patent is issued. Although we have filed for patents on some aspects of our technology, we cannot provide assurances that patents will be issued as a result of these pending applications or that any patent that has been or may be issued will be upheld. The laws of some foreign countries may not protect our proprietary rights to the same extent as do the laws of the United States.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental 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 governmental fees on patents and/or applications will be due to be paid to the U.S. PTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our outside counsel to pay these fees due to non-U.S. patent agencies. The U.S. PTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ professionals to help us comply, and 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. However, there are situations 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, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business. We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting 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 can be less extensive than those in the United States. In addition, 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. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States.

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 protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

In the future, third parties may assert patent, copyright, trademark, and other intellectual property rights to technologies that are important to our business. The outcome following any potential legal assertions of invalidity and unenforceability is unpredictable. Any claims asserting that our products infringe or may infringe proprietary rights of third parties, if determined adversely to us, could significantly harm our business. See “Risk Factors—*Risks Relating to Our Intellectual Property*”.

## **COMPETITION**

We, and our biopharmaceutical collaborators, are leaders in the research and development of gene therapies, cell therapies and genome engineering therapies using ZFP DNA-binding proteins.

We are aware of several other companies focused on other methods for editing genes and regulating gene expression and a limited number of commercial and academic groups pursuing the development of ZFP genome engineering technologies. The fields of gene therapy, cell therapy and genome engineering are highly competitive, and we expect competition to persist and intensify in the future from a number of different sources, including other biopharmaceutical companies; academic and research institutions; and government agencies that will seek to develop ZFPs as well as technologies that will compete with our ZFP technology platform, such as TALE proteins and the CRISPR-Cas editing system.

Accordingly, our competitors may succeed in obtaining patent protection, receiving FDA approval or commercializing competitive products before us. If we commence commercial product sales, we may be competing against companies with greater marketing, sales, distribution and manufacturing capabilities, areas in which we have limited or no experience. In addition, any product candidate that we successfully develop may compete with existing products that have long histories of safe and effective use.

Although we are in the clinical development phase of operations and have no current therapeutic product sales, we believe the following companies, products and/or technologies may potentially be competitive with our technology or our product candidates under development:

- Protein pharmaceuticals under development at pharmaceutical and biotechnology companies such as Pfizer, Bayer AG, Novo Nordisk A/S, Sanofi, Takeda, BioMarin Pharmaceutical Inc., or BioMarin, Biogen, Acceleron Pharma Inc., ArmaGen, Inc., Amicus Therapeutics, Inc., Protalix Biotherapeutics, Inc., F. Hoffman-LaRoche Ltd., Novartis AG, and numerous other biopharmaceutical firms.
- Gene therapy companies developing gene-based products in clinical trials. Orchard Therapeutics plc's Strimvelis™ (acquired from GlaxoSmithKline plc, or GSK) is approved in Europe, Spark Therapeutics, Inc.'s LUXTURN™ is approved in the United States and Europe, Novartis Gene Therapies' Zolgensma™ is approved in the United States, Europe and additional territories, and bluebird bio's ZYNTEGLO™ is approved in Europe. Other competitors in this category may include, but not be limited to, uniQure N.V., BioMarin, REGENXBIO Inc., Ultragenyx Pharmaceutical Inc., Voyager Therapeutics, Inc., Takeda, Pfizer, Freeline Therapeutics, Amicus Therapeutics, Inc., and Novartis AG.
- Cell therapy companies developing cell-based products. Novartis AG's Kymriah™ and Gilead's Yescarta™, gene-modified cell-based therapies, are approved in both the United States and Europe, and Bristol Myers Squibb's Breyanzi™ is approved in the United States. Other competitors in this category may include, but not be limited to, Adaptimmune Therapeutics PLC, bluebird bio, Inc., Collectis S.A., Sana, Lyell, Inc., Kite/Gilead, AvroBio, Inc., Medeor Therapeutics, Inc., CRISPR Therapeutics AG, Intellia Therapeutics, Inc., Casebia Therapeutics, Targazyme, Inc., ZIOPHARM Oncology, Inc., Tmunity Therapeutics, Inc., Caladrius Biosciences, Inc., TRACT Therapeutics, Inc., Cellenkos™, Inc., Regcell Co., Ltd., Allogene, Fate Therapeutics, NKarta Therapeutics and Bristol Myers Squibb.
- Nuclease and base editing technologies under development for therapeutic applications of genome modification including companies such as Editas Medicine, Inc., CRISPR Therapeutics AG, Caribou Biosciences, Inc., Intellia Therapeutics, Inc. and Beam Therapeutics developing the CRISPR/Cas9 editing system, Collectis S.A. developing TALE nucleases and meganucleases, bluebird bio, Inc. developing Homing Endonucleases and MegaTALs and Precision BioSciences, Inc. developing meganucleases.
- Antisense therapeutics and RNA interference technology, including RNAi and microRNA, which are technologies that may compete with us in the development of novel therapeutic products acting through the regulation of gene expression. These technologies are being developed by several companies including Alnylam Pharmaceuticals, Inc., Ionis Pharmaceuticals, Inc., Moderna, Inc., Sanofi and Regulus Therapeutics Inc.
- Small molecules in development from both in-house drug discovery programs of pharmaceutical companies such as Pfizer, GSK, Novartis AG and Merck & Co., Inc., as well as from biotechnology companies with expertise and capabilities in small molecule discovery and development such as Gilead, Sanofi, Bristol Myers Squibb, and Global Blood Therapeutics, Inc., which has an approved small molecule product for SCD.
- Monoclonal antibody companies and product candidates from certain biotechnology firms such as Genentech, Inc. and Amgen Inc.

We expect to face intense competition from other companies for collaborative arrangements with biopharmaceutical companies for establishing relationships with academic and research institutions, for licenses to proprietary technology and for subjects in our clinical trials of treatments for rare diseases. These competitors, either alone or with their collaborative partners, may succeed in developing technologies or products that are more effective or less costly than ours.

Our ability to compete successfully will depend, in part, on our ability to:

- develop safe, efficacious and commercially attractive proprietary products;
- obtain access to gene transfer technology on commercially reasonable terms;
- obtain required regulatory approvals;
- obtain reimbursement for our products in approved indications;
- attract and retain qualified scientific and product development personnel;
- enter into collaborative and strategic partnerships with others, including our competitors, to develop our technology and product candidates;
- obtain and enforce patents, licenses or other proprietary protection for our products and technologies;

- formulate, manufacture, market and sell any product that we develop;
- develop and maintain products that reach the market first and are technologically superior to or are of lower cost than other products in the market; and
- recruit subjects into our clinical trials in a timely fashion.

## **MANUFACTURING**

We currently rely heavily on CMOs to produce our preclinical and clinical product candidates in accordance with FDA and EMA mandated regulations, also known as current good manufacturing practices, or cGMPs. We employ a technical operations staff in the areas of process development, analytical development, quality control, quality assurance, project management, and manufacturing to facilitate appropriate oversight of our CMOs, support of our regulatory filings and execution of clinical trials.

We believe that in-house manufacturing capability can provide a competitive advantage. To this end, we have recently completed and brought online an AAV cGMP manufacturing facility in our headquarters building in Brisbane, California designed to manufacture Phase 1/2 clinical study supplies for our gene therapy pipeline. We are also building cell therapy manufacturing facilities in Brisbane, California and in Valbonne, France that we expect to become operational by the end of 2021.

Even after these in-house facilities are operational, we intend to continue to rely on CMOs for the manufacture of our product candidates for any Phase 3 clinical trials, and if approved, for commercial supply. We believe this balanced approach to manufacturing, investing in internal capacity and capabilities while strengthening our commitment to external capacity, will enable us to meet our anticipated pipeline needs. Additionally, in 2019 we signed an expanded services agreement with Brammer Bio MA, a Thermo Fisher Scientific, Inc. subsidiary, or Brammer, which provided us with access to dedicated AAV manufacturing capacity up to 2000-L bioreactor scale capable of handling large-scale, commercial-grade runs for products such as ST-920, our gene therapy product candidate for Fabry disease. The agreement also allows us to leverage Brammer's viral vector manufacturing know-how in our Brisbane manufacturing facility that we believe will provide a seamless transition from early development to late-stage clinical trials and commercial-scale manufacturing.

We currently leverage three distinct manufacturing platforms: AAV vector production for our genome engineering and gene therapy product candidates, HSPC modification for some of our cell therapy product candidates and engineered T cell therapies. We use a commercial scale baculovirus manufacturing platform to manufacture AAV vectors for genome editing and gene therapy, with each AAV vector packaging a different transgene specific to the target indication or ZFN. The manufacturing process for our HSPC cell therapy product candidates utilizes the patient's own HSPCs. These HSPCs are transfected using mRNA to produce ZFNs that target specific DNA sites, resulting in modified HSPCs. The third platform utilizes our ZFN technology to transform CAR-Tregs for autologous and allogeneic cell therapies. With the acquisition of Sangamo France, we also added capabilities to manufacture regulatory T cells in therapeutic quantities to be used to treat inflammatory and autoimmune disorders.

## **GOVERNMENT REGULATION**

We operate within the heavily regulated biopharmaceutical industry and much of our operations, including nonclinical and clinical trials, development, manufacturing, commercialization, marketing and reimbursement are subject to regulatory approvals. Relevant regulatory authorities include, but are not limited to, the FDA, the EMA, Commission of the European Union, or EU, Member State agencies, including the UK Medicines and Healthcare Products Regulatory Agency, or MHRA.

### **Product Regulation**

In the United States, the FDA regulates biologic products including gene therapy and human cellular therapy products under the Federal Food, Drug, and Cosmetic Act, or the FDCA, the Public Health Service Act, or the PHSA, and regulations and guidance implementing these laws. The FDCA, PHSA and their corresponding 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. Applications to the FDA are required before conducting human clinical testing of biologic products and in the EU, approval must be obtained from the EMA. FDA approval also must be obtained before marketing of biologic products. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals.

## ***Accelerated Assessment***

A number of agencies, including the FDA and the EMA, have expedited development programs, including for innovative products and in areas of high unmet medical need, such as PRIME in the EU. These programs require a certain level of evidence demonstrating safety and efficacy in patients from early-stage clinical trials. Entry into one of these expedited programs may result in assistance with the scientific opinion and faster approval timelines. Some of these programs may offer joint approval and reimbursement advice. It is noted that even applications in an expedited development program may be assessed under standard timelines, where the regulatory authority deems that the program may no longer meet the requirements for priority review.

## ***U.S. Biologic Products Development Process***

Our product candidates must be approved by the FDA before they may be legally marketed in the United States. The process required by the FDA before a biologic product candidate may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests and *in vivo* studies in accordance with the FDA's current Good Laboratory Practice, or GLP, regulations and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an IND application, which allows human clinical trials to begin unless FDA objects within 30 days;
- approval by an independent institutional review board, or IRB, reviewing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials according to the FDA's Good Clinical Practice, or GCP, regulations, and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biologic product candidate for its intended use;
- preparation and submission to the FDA of a biologics license application, or BLA, for marketing approval that includes substantial evidence of safety and efficacy from results of nonclinical testing and clinical trials;
- review of the product 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, potency and purity;
- potential FDA inspection of the nonclinical and clinical trial sites that generated the data in support of the BLA; and
- payment of user fees, if applicable, and FDA review and approval, or licensure, of the BLA.

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. The conduct of the preclinical tests must comply with federal regulations and requirements including GLPs.

Concurrent with clinical trials, companies usually must complete additional preclinical testing, that may include animal tests of reproductive adverse events and carcinogenicity, and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the drug in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and, among other things, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drug product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the drug candidate does not undergo unacceptable deterioration over its shelf life.

Human gene transfer protocols are subject to the FDA's oversight and other clinical trial regulations, and oversight at the local level as set forth in National Institutes of Health, or NIH, Guidelines. Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. 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 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. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA also may impose clinical holds on a biologic product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical studies to begin, or that, once begun, issues will not arise that suspend or terminate such studies.

### ***EU Drug Development Process***

Similar to the United States, the EU regulatory framework sets both EU-wide and national, Member State-specific requirements for the development and approval of medicinal products. Article 8(3) of Directive 2001/83/EC sets out the contents of a marketing authorization, or MA, application and all the information that must be submitted for the evaluation of a medicinal product. Certain preclinical (also termed “non-clinical”) data is required in order to enable clinical trials and later be used in dossier for a marketing authorization application. All studies should take place in accordance with GLP and all applicable EMA, Commission and European Pharmacopoeia guidelines on preclinical studies, including guidance on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells.

The requisite amount of preclinical data enables the design of a clinical trial, from Phase 1 (first-in-human clinical trials) through to Phases 2 and 3, which are safety and efficacy studies. Similar restrictions and requirements apply as in the United States regarding preclinical data to support trials using viral vectors. The preclinical tests should establish parameters such as toxicity, pharmacodynamics and pharmacokinetic properties, as well as the quality of the gene therapy medicinal products. Due to the particular nature of gene therapy medicinal products, it is recognized that it may not always be possible for the non-clinical safety studies to be in conformity with the principles of GLP and a proper justification should be submitted where a pivotal non-clinical safety study has not been conducted under GLP rules.

Clinical studies are crucial to obtaining the required data and the requirements governing the conduct of clinical trials are further analyzed below.

All medicinal products and advanced therapy medicinal products, or ATMPs, must be manufactured in accordance with the guidelines on Good Manufacturing Practice, or GMP, and in a GMP licensed facility, which can be subject to GMP inspections.

### ***Human Clinical Trials Under an IND***

Clinical trials involve the administration of the biologic product candidate to patients under the supervision of qualified investigators which generally are 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 items such 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 their legal representative, reviews and approves the study protocol, and must monitor the clinical trial until completed.

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

- *Phase 1.* The biologic product candidate initially is introduced into a small number of human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early understanding of its effectiveness. Phase 1 clinical trials of gene and cell 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 candidate 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 which 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 studies, the biologic product candidate 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 efficacy 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 referred to as Phase 4 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. Sometimes approval for a product is conditional upon the completion of post-marketing clinical studies.

During all phases of clinical development, regulatory agencies (such as the FDA, the EMA and other comparable regulatory agencies) require extensive monitoring and auditing of all clinical activities, clinical data and clinical trial investigators. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA.

Written IND safety reports must be promptly submitted to the FDA 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.

The FDA or the sponsor or its data safety monitoring board 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 safety 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.

The FDA usually recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for up to a 15-year period.

In the EU, clinical trials almost always require approval from a national competent authority of the relevant Member State and an approval from an Ethics Committee. If the medicinal product is considered to be a genetically modified organism, or GMO, then GMO approval must also be obtained. There is no harmonization between Member States regarding the approach to and timelines of GMO approval, which may result in the submission of additional information, which may impact study initiation in a given country.

The conduct of clinical trials should follow the approved clinical trial protocol and be in accordance with the principles of GCP. Gene therapy medicinal products are in addition subject to the rules of GCP for ATMPs, which outline specific additional safeguards and requirements. Record retention requirements are increased for ATMPs as there are relevant long-term follow-up and human safety and traceability requirements.

### ***Compliance with cGMP Requirements***

Manufacturers of biologics must comply with applicable current Good Manufacturing Practices, or 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. Any material changes to the manufacturing equipment, process or location of the approved manufacturing site must be reported to the relevant agency/authority. Establishments may be subject to periodic, unannounced inspections by government authorities (including regulatory agencies) 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, issue warning or similar letters or seeking civil, criminal or administrative sanctions against the company. The

FDA will not approve a BLA 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 specification.

Concurrent with clinical trials, companies develop additional information about the physical and biological characteristics of the 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 PHSa 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.

For a product candidate that is also a human cellular or tissue product, the FDA also requires compliance with current Good Tissue Practices, or cGTPs. These are FDA regulations 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 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.

### ***U.S. Review and Approval Processes***

The results of the preclinical tests and clinical trials, together with detailed information relating to the product's Chemistry, Manufacturing and Controls, or CMC, and proposed labeling, among other things, are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a significant user fee. The FDA adjusts the PDUFA user fees on an annual basis. The PDUFA also imposes an annual program fee for approved biologics. 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 or for a product indication for orphan diseases.

The FDA reviews a BLA within 60 days of submission to determine if it is substantially complete before the agency accepts it for filing. 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 that event, the BLA must 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.

The FDA reviews the BLA to determine, among other things, whether the proposed product candidate is safe and effective, for its intended use and whether the product candidate is being manufactured in accordance with cGMP to assure and preserve the product candidate'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 product approval process, the FDA also will determine whether a risk evaluation and mitigation strategy, or REMS, is necessary to assure the safe use of the product candidate. 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.

Before approving 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 candidate within required specifications. Additionally, before approving a BLA, the FDA typically will inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND trial requirements and GCP requirements.

On the basis of the BLA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the biologic product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the BLA, the FDA will issue an approval letter.

If a product candidate receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited. 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, sometimes referred to as Phase 4 clinical trials, designed to further assess a biologic product's safety and effectiveness, 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 10 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.

### ***EU Review and Approval Process***

Before a medicinal product can be placed on the market in the EU, it must have received an MA. This could either be at national or EU level under a mutual recognition, decentralized or centralized procedure. Our product candidates are innovative treatments, which will bear the classification of ATMP. As such, the appropriate authorization procedure is the centralized procedure, which involves an MA being granted by the European Commission following a positive opinion by the EMA. A centralized MA is simultaneously valid in all EU Member States and the European Economic Area, or EEA, (Iceland, Liechtenstein and Norway). A centralized MA also results in a single set of product information (patient information leaflet, labelling and summary of product characteristics) for all EU Member States.

The timeline for the grant of a centralized MA since the time of the application is 210 days for the assessment of the application (including "clock stops" for the applicant to prepare answers to the questions from the EMA). The Committee for Medicinal Products for Human Use, or the CHMP, may either provide a positive or negative opinion. Following a positive opinion, the European Commission will usually issue its legally binding MA after 67 days. A negative opinion may be appealed by the applicant who must submit a request for re-examination within 60 days. There is the possibility for accelerated timelines of drug applications for eligible applicants, which can reduce the timeline to 150 days, if the applicant can produce sufficient justification.

If the MA application contains less comprehensive than the required standard as at the time of the application, when there are public health grounds and often in the case of orphan medicinal products, the EMA may recommend to the European Commission that it issues a different type of an MA, as follows: (a) a Conditional MA (valid for one year and renewable), when the medicinal product shows a positive benefit-risk balance and targets an unmet medical need and it is expected that the applicant will be able to provide comprehensive data in due course; or (b) an MA under 'exceptional circumstances', when it is not expected that the applicant will be able to provide comprehensive efficacy and safety data (often for very rare indications).

### ***Manufacturing Regulation in Europe***

Various requirements apply to the manufacturing and placing on the EU market of medicinal products. The manufacturing of medicinal products in the EU requires a manufacturing authorization, and the manufacturing authorization holder must comply with various requirements set out in the applicable EU laws, regulations and guidance, including EU cGMP standards. Similarly, the distribution of medicinal products into and within the EU is subject to compliance with the applicable EU laws, regulations and guidelines, including the requirement to hold appropriate authorizations for distribution granted by the competent authorities of the EU member states. Marketing authorization holders may be subject to civil, criminal or administrative sanctions, including suspension of manufacturing authorization, in case of non-compliance with the EU or EU member states' requirements applicable to the manufacturing of medicinal products.



### ***Post-approval Requirements***

Rigorous and extensive FDA regulation of biologic products continues after approval, particularly with respect to cGMP requirements. Manufacturers are required to comply with applicable requirements in the cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Other post-approval requirements applicable to biologic products include reporting of cGMP deviations that may affect the identity, potency, purity and overall safety of a distributed product, record-keeping requirements, reporting of adverse effects, reporting updated safety and efficacy information and complying with electronic record and signature requirements. After a BLA is approved, the product also may be subject to official lot release. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA, together with a release protocol, showing a summary of the history of manufacture of the lot and the results of all tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products before releasing the lots for distribution. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency and effectiveness of biologic products. Failure to comply with the FDA's post-approval regulations can result in withdrawal of product approval and licensure.

A sponsor also must comply with the FDA's or appropriate national authority's advertising and promotion requirements, such as the prohibition on promoting products for uses or in patient populations that are not described in the product's approved labeling (known as "off-label use"). Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. In addition, changes to the manufacturing process or facility generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

### ***Orphan and RMAT designation***

Products that are intended for treating rare conditions that affect fewer than 200,000 people in the U.S., or that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a treatment drug, may qualify for orphan designation. In the EU, these rare conditions are defined as having a prevalence of no more than five in every 10,000 people in the EU. Once a medicinal product with orphan designation obtains a marketing approval, it can benefit from a marketing exclusivity period in respect of the specific orphan indication for which the drug has been approved for a period of seven years in the U.S. and for up to 10 years in the EU. If the manufacturer is no longer able to assert that the product meets the orphan designation criteria or is not able to provide sufficient quantities, it may lose the orphan market exclusivity.

Regenerative medicine advanced therapy, or RMAT, designation is intended to expedite review of a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition and for which preliminary clinical evidence indicates the potential to address unmet medical needs for such a disease or condition.

RMAT designation provides potential benefits that include more frequent meetings with the FDA to discuss the development plan for the product candidate, and eligibility for rolling review and priority review of the related BLA. Products granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites, including through expansion to additional sites. However, RMAT designation does not change the FDA's standards for product approval. Additionally, RMAT designation can be revoked if the criteria for eligibility cease to be met as clinical data emerges.

### ***Clinical Trial Data Disclosure***

Many jurisdictions have mandatory clinical trial information obligations on sponsors. In the EU this is under the Transparency Regulation No. 1049/ 2001, EMA Policy 0043, EMA Policy 0070, as well as the new Clinical Trials Regulation No. 536/2014, all of which impose on sponsors the obligation to make publicly available certain information stemming from clinical studies. In the EU, the transparency framework provides for a wide right for (EU-based at the moment) interested parties to submit an access to documents request to the EMA for information included in the marketing authorization application dossier for approved medicinal products. Only very limited information is exempted from disclosure (i.e., commercially confidential information, which is construed increasingly narrowly and protected personal data). It is possible for competitors to access and use this data in their own research and development programs anywhere in the world, once these data are in the public domain.

## Regulation of Our Operations

Although we currently do not have any products on the market, we may be subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which we conduct our business. Such laws include, without limitation:

- the federal healthcare Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;
- federal civil and criminal false claims laws, including the federal False Claims Act, and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment or approval that are false or fraudulent or making a false 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 imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also created federal criminal laws that prohibit, among other things, knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statements in connection with the delivery of or payment for healthcare benefits, items or services;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, which impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information held by certain healthcare providers, health plans and healthcare clearinghouses, known as covered entities, and individuals and entities that perform services for them that involve individually identifiable health information, known as business associates as well as covered subcontractors;
- the federal Physician Payments Sunshine Act created under the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the ACA, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to the Centers for Medicare and Medicaid Services, or CMS, information related to payments and other transfers of value to physicians, currently defined to include doctors, dentists, optometrists, podiatrists and chiropractors, and teaching hospitals, and ownership and investment interests held by physicians and their immediate family members. Effective January 1, 2022, these reporting obligations will extend to include transfers of value made to physician assistants, nurse practitioners, clinical nurse specialists, anesthesiologist assistants, certified registered nurse anesthetists and certified nurse midwives during the previous year;
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services 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, require drug manufacturers to report information related to payments and other transfers of value to other healthcare providers and healthcare entities, marketing expenditures; or drug pricing; and/or ensure the registration of sales personnel; and
- state and foreign laws govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

If our operations are found to be in violation of any of such laws or any other governmental regulations that apply to us, we may be subject to significant penalties, including, without limitation, administrative, civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, exclusion from participation in federal and state healthcare programs, imprisonment, suspension or withdrawal of our marketing and commercialization in respect of our commercially approved products, and additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, any of which could adversely affect our ability to operate our business and our financial results. Responding to investigations can be time-and resource-consuming and can divert management's attention from the business. Any such investigation or settlement could increase our costs or otherwise have an adverse effect on our business. See "Risk Factors—Our current and future relationships with healthcare providers, customers and third-party payors subject us to applicable anti-kickback, fraud and abuse, privacy, data security and other healthcare laws and regulations. If we fail to comply with such regulations, we could face substantial penalties and our business, results of operations, financial condition and prospects could be adversely affected."

## Healthcare Reform

The U.S. and some foreign jurisdictions are considering enacting or have enacted a number of additional legislative and regulatory proposals to change the healthcare system in ways that could affect our ability to sell our product candidates profitably, if approved. Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts, which include major legislative initiatives, such as the ACA, to reduce the cost of care through changes in the healthcare system, including limits on the pricing, coverage, and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded health care programs, and increased governmental control of drug pricing. The ACA and its implementing regulations, among other things, addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for certain drugs and biologics, including products similar to our product candidates, that are inhaled, infused, instilled, implanted or injected, increased the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program, extended the Medicaid Drug Rebate Program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations, subjected manufacturers to new annual fees and taxes for certain branded prescription drugs, created a new Patient Centered Outcomes Research Institute, which provides incentives to programs that increase the federal government's comparative effectiveness research, established a new Medicare Part D coverage gap discount program, in which manufacturers must now agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D, and created a licensure framework for follow-on biologic products.

There remain legal and political challenges to certain aspects of the ACA, as well as efforts to repeal or replace certain aspects of the ACA. Several bills affecting the implementation of certain taxes under the ACA have been signed into law. The Tax Cuts and Jobs Act of 2017, or Tax Act, included a provision that repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". In addition, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the ACA-mandated "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminates the health insurer tax. In addition, the Bipartisan Budget Act of 2018, or the BBA, among other things, amended the ACA, effective January 1, 2019, to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole."

On December 14, 2018, a Texas U.S. District Court Judge ruled that the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act. Additionally, on December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. The United States Supreme Court is currently reviewing this case, although it is unclear when a decision will be made.

Other legislative changes have been proposed and adopted in the United States since the ACA 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. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect in April 2013, and, due to subsequent legislative amendments to the statute, including the Bipartisan Budget Act of 2018, will remain in effect through 2029 unless additional Congressional action is taken. However, pursuant to COVID-19 pandemic relief legislation, these Medicare sequester reductions are suspended from May 1, 2020 through March 31, 2021 due to the COVID-19 pandemic. In January 2013, the American Taxpayer Relief Act of 2012, or the ATRA, was signed into law, which, among other things, further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Also, there has been heightened governmental scrutiny recently over pharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for pharmaceutical products. At the federal level, the Trump administration's budget proposal for fiscal year 2021 included a \$135 billion allowance to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients, and increase patient access to lower-cost generic and biosimilar drugs. On March 10, 2020, the Trump administration sent "principles" for drug pricing to Congress, calling for legislation that would, among other things, cap Medicare Part D beneficiary out-of-pocket pharmacy expenses, provide an option to cap Medicare Part D

beneficiary monthly out-of-pocket expenses, and place limits on pharmaceutical price increases. Further, the Trump administration released a “Blueprint” to lower drug prices and reduce out-of-pocket costs of drugs that contained proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out-of-pocket costs of drug products paid by consumers. On July 24, 2020 and September 13, 2020, the Trump administration announced several executive orders related to prescription drug pricing that seek to implement several of the administration’s proposals. As a result, the FDA released a final rule on September 24, 2020, effective November 30, 2020, providing guidance for states to build and submit importation plans for drugs from Canada. Further, on November 20, 2020, the U.S. Department of Health and Human Services, or 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 rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers. On November 20, 2020, CMS issued an interim final rule implementing President Trump’s Most Favored Nation executive order, which would tie Medicare Part B payments for certain physician-administered drugs to the lowest price paid in other economically advanced countries, effective January 1, 2021. On December 28, 2020, the United States District Court in Northern California issued a nationwide preliminary injunction against implementation of the interim final rule. It is unclear whether the Biden administration will work to reverse these measures or pursue similar policy initiatives. At the state level, legislatures have increasingly passed legislation and implemented 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 the United States, the EU and other potentially significant markets for our product candidates, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. Furthermore, the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in the EU will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our future product sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general.

See “Risk Factors—*Recently enacted and future legislation, including potentially unfavorable pricing regulations or other healthcare reform initiatives, may increase the difficulty and cost for us to obtain regulatory approval of and commercialize our product candidates and affect the prices we may obtain.*”

### **Pricing, Coverage and Reimbursement**

Pricing and reimbursement of a therapeutic product will largely determine the affordability of the product, and whether the product is prescribed and supplied to patients and private insurance companies may take into account government reimbursement methodologies. Due to these proposed and enacted laws, as well as other actions, significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we obtain regulatory approval, particularly for novel products. In both domestic and foreign markets, sales and reimbursement of any approved products will depend, in part, on the extent to which third-party payors, such as government health programs, commercial insurance and managed healthcare organizations provide coverage, and establish adequate reimbursement levels, for such products. Third-party payors are increasingly challenging the prices charged for 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 FDA-approved products for a particular indication. Additionally, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost-effectiveness of our products. If third-party payors do not consider our products to be cost-effective compared to other therapies, these payors may not cover our products after approved as a benefit under their plans or, if they do, the level of reimbursement may not be sufficient to allow us to sell our products on a profitable basis. See “Risk Factors—*Even if we are able to commercialize our product candidates, the products may not receive coverage and adequate reimbursement from third-party payors in the United States and in other countries in which we seek to commercialize our products, which could harm our business.*”

In the EU, pricing and reimbursement are the prerogative of Member States. Therefore, the requirements around reimbursement of medicinal products can vary widely. Each Member State can follow its own approach, subject to common rules of transparency, competition, and freedom of trade and movement in the EU. Many Member States, including France, Germany and the United Kingdom, follow a health technology assessment, or HTA, procedure for medicinal products in order to assess the cost-effectiveness of a product which could then be recommended for reimbursement under the national health services. There is increasingly exchange of information concerning HTAs on a voluntary basis among EU Member States. In

the United Kingdom, the National Institute for Health and Care Excellence is the body which conducts HTAs and issues guidance to be followed by the regional health bodies called clinical commissioning groups.

## **Environmental Regulation**

U.S. federal and state laws regarding safe working conditions, environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. We may incur significant costs to comply with such laws and regulations now or in the future. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. We believe that we are in material compliance with applicable environmental laws and regulations that continued compliance therewith will not have a material effect on our business. We cannot predict, however, how changes in these laws and regulations may affect our future operations.

## **Privacy Regulation**

We are required to comply with privacy and data security laws in the United States and in other foreign jurisdiction in which we operate, such as the EU General Data Protection Regulation, or GDPR, and the California Consumer Privacy Act of 2018, or CCPA, which apply to the collection, use, disclosure, transfer, or other processing of personal data.

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, which is wide-ranging in scope, imposes several requirements on us relating to, among other things, the control over personal data by individuals to whom the personal data relates; notice we must provide to individuals regarding our processing of their personal data; the documentation we must maintain; the security and confidentiality of the personal data; data breach notification; and the use of third-party processors in connection with the processing of personal data. The GDPR also imposes strict rules on the transfer of personal data out of the EU. The post-Brexit trade and cooperation agreement between the United Kingdom and the EU provides that transfers of personal data from the EU to the United Kingdom will not be treated as restricted transfers to a non-EU country for a period of up to six months from January 1, 2021. However, unless the EU Commission makes an “adequacy finding” with respect to the United Kingdom before the end of that transition period, from that date the United Kingdom will be a “third country” under the GDPR, and transfers of personal data from the European Economic Area to the United Kingdom will require an “adequacy mechanism,” such as the Standard Contractual Clauses. The GDPR authorizes the imposition of large penalties for noncompliance, including the potential for fines of up to €20 million or 4% of the annual global revenue of the noncompliant company, whichever is greater. The GDPR requirements related to international data transfers apply not only to third-party transactions, but also to transfers of information between us and our subsidiaries such as Sangamo France, including employee information. The GDPR has increased our responsibility and potential liability in relation to personal data that we process compared to prior EU law, particularly in light of our acquisition of Sangamo France, and we may be required to put in place additional mechanisms to ensure compliance with the GDPR, which could divert management’s attention and increase our cost of doing business.

Despite our ongoing efforts to bring our practices into compliance with the GDPR, we may not be successful either due to various factors within our control or other factors outside our control. It is possible that local data protection authorities may interpret the GDPR in different ways, leading to potential inconsistencies in the requirements we must meet in different EU member states. Any failure or alleged failure (including as a result of deficiencies in our policies, procedures or measures relating to privacy, data security, marketing or communications) by us to comply with laws, regulations, policies, legal or contractual obligations, industry standards or regulatory guidance relating to privacy or data security may result in governmental investigations and enforcement actions, litigation, fines and penalties or adverse publicity.

In the United States, California adopted the CCPA, which became effective in January 2020. The CCPA establishes a privacy framework for covered businesses, including an expansive definition of personal information and data privacy rights for California residents. The CCPA includes a framework with potentially severe statutory damages and private rights of action. The CCPA requires covered businesses to provide new disclosures to California consumers (as that word is broadly defined in the CCPA), provide such consumers new ways to opt-out of certain sales of their personal information and allow for a new cause of action for data breaches. It remains unclear how the CCPA will be interpreted, but as currently written, it will likely impact our business activities and exemplifies the vulnerability of our business not only to cyber threats but also to the evolving regulatory environment related to personal data. As we expand our operations, the CCPA may increase our compliance costs and potential liability.

The CCPA itself will be expanded substantially effective January 1, 2023, as a result of California voters approving the California Privacy Rights Act of 2020, or CPRA, on the November 2020 ballot, which will, among other things, establish the California Privacy Protection Agency to implement and enforce the new law, as well as impose administrative fines. Some observers have noted that the CCPA and CPRA could mark the beginning of a trend toward more stringent privacy legislation in the United States. Other states are beginning to pass similar laws.

Compliance with these and any other applicable privacy and data security laws and regulations is a rigorous and time-intensive process, and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. If we fail to comply with any such laws or regulations, we may face significant fines and penalties that could adversely affect our business, financial condition and results of operations. Furthermore, the laws are not consistent, and compliance in the event of a widespread data breach is costly. See “Risk Factors—*Our current and future relationships with healthcare providers, customers and third-party payors subject us to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations. If we fail to comply with federal, state and foreign laws and regulations, including healthcare, privacy and data security laws and regulations, we could face substantial penalties and our business, results of operations, financial condition and prospects could be adversely affected.*”

## HUMAN CAPITAL MANAGEMENT

### Our Mission & Our Employees

At Sangamo, we are committed to translating ground-breaking science into genomic medicines that transform patients’ lives. We are a passionate group of biotechnology professionals based in the United States, France and the United Kingdom with years of experience and technical expertise, committed to developing best-in-class genomic medicines. We embrace collaboration, discipline and efficiency while welcoming fresh ideas and stimulating personal development. We encourage and embrace diversity and believe it enhances our work towards one common goal: to transform the lives of the patients we aim to serve.

We view our employees as one of our most valuable assets in serving our mission. We compete in the highly competitive biotechnology industry, and attracting, retaining and developing a diverse group of talented employees is crucial to our strategy and our ability to compete effectively. We need to grow the size of our organization in order to support our current research, product development, manufacturing and regulatory efforts and our plans for commercializing our wholly-owned product candidates when approved. This growth is critical to our success. There currently is a shortage of skilled individuals with substantial experience discovering, developing and manufacturing genomic medicines, which is likely to continue. As a result, competition for these individuals is intense and the turnover rate can be high. We face substantial competition among numerous biopharmaceutical companies and academic institutions for individuals with these skills.

### Our Values

We believe success comes when we and our employees align our core values with our mission to translate our ground-breaking science into genomic medicines that transform patients’ lives. Our core values are:

- **Patient Centric**: Patient welfare is the rule against which we measure our actions and decisions. Before making decisions affecting patients, we work to understand their needs and experiences. Our focus on patients is a source of competitive advantage: it sharpens our research and development efforts and guides us to develop important medicines.
- **Teamwork & Accountability**: We seek appropriate input across our company to make better decisions and achieve our goals. We treat one another with respect and expect a great deal of each other. We honor our commitments.
- **Innovation & Excellence**: The bold pursuit of technological advancement and scientific breakthrough is our foundation. We are also a company where individuals can flourish, grow and develop their expertise. We celebrate our successes and the personal and professional accomplishments of our colleagues.
- **Urgency & Efficiency**: We make decisions quickly and thoughtfully; we decentralize decision making authority appropriately to foster ownership and action. We define our priorities clearly, communicate them and move forward decisively. We commit to achieving or surpassing performance objectives, and we celebrate exceeding the expectations of our colleagues and stakeholders.

## **Our Management of Human Capital**

We recently hired our first Chief People Officer in September 2020 to lead our human resources function and to expand our programs to recruit, retain and develop our employees. As of December 31, 2020, our global human resources function was comprised of nine full time human resources professionals.

To manage our human resources, we track and report internally on key talent metrics including headcount by business unit and country, historical headcount growth, turnover, new hires and terminations, open roles and employee demographics including gender, race and ethnicity. Our senior executives use these metrics to assist with resource planning, recruitment and retention initiatives and design of compensation and benefits programs. We share these metrics quarterly with the Compensation Committee of our Board of Directors to assist it in fulfilling its duties to (a) establish our enterprise compensation philosophy, (b) administer our compensation and benefit plans, (c) evaluate the performance of our executive officers and key employees and (d) review and monitor management development and succession plans.

As of December 31, 2020, we had 413 full time employees located in the United States, France and the United Kingdom. Of these employees, 349 are located in the United States, primarily in the San Francisco Bay Area, 57 are located in Valbonne, France and the remaining 7 are located near London, United Kingdom. Of these employees, 185 were primarily engaged in research and development activities, 140 were primarily engaged in technical operations and manufacturing and 88 were primarily engaged in general and administrative activities. We also engage the services of independent contractors and consultants as needed for special or temporary projects or specific expertise.

## **Our Commitment to Diversity, Equity & Inclusion**

We strongly believe in a diverse workplace where all Sangamo employees can thrive in an inclusive environment free from discrimination, harassment, bias and prejudice. We aim to treat all individuals with respect and dignity and to provide all Sangamo employees with equal opportunity and fair treatment based on merit. By embracing diversity and inclusion, we create an organization committed to working together to develop innovative solutions in support of the Sangamo mission consistent with our values. At Sangamo, we cultivate a culture and environment where different backgrounds and perspectives are not only respected and heard, but embraced and celebrated. Not only is a diverse, equitable and inclusive mindset and culture critical to an engaged and committed workplace, but it is also imperative to understanding and meeting the needs of the patients we seek to help with our medicines.

In 2020, with the support of our Board of Directors and Chief Executive Officer, we convened a Diversity, Equity and Inclusion, or DEI, working group comprised of a diverse group of employees tasked with designing and implementing specific initiatives to promote greater diversity, equity and inclusion at Sangamo worldwide, including potential amendments to our company values to reflect our commitment to these ideals and the launch of a corporate social responsibility scorecard tracking our DEI initiatives. We also have engaged an experienced DEI consultant to help survey our employee population and advise us on the design and planning of new DEI initiatives based on this input and external benchmarking. In 2021, we expect to implement these measures, institute new DEI tracking and benchmarking and increase our focus on DEI in recruitment and retention initiatives.

As of December 31, 2020, women accounted for the following approximate percentages of our full-time employees: 51% globally; 48% in the United States; 65% in France and 71% in the United Kingdom. As of December 31, 2020, our employee records indicate that approximately 57% of our full-time U.S. employees identify as non-white.

## **Our Compensation & Benefits**

Given the highly competitive nature of our industry and the importance of recruitment and retention to our success, we strive to furnish our employees with what we believe is a very competitive and comprehensive total rewards package of compensation, benefits and services. This package includes at or above-market pay; healthcare benefits for employees and family members; a health savings account for eligible U.S. employees with above market employer contributions; generous paid time off benefits; family leave; flexible work schedules; contributions to retirement and/or pension plans; mental health benefits and onsite services. In addition, we offer every full-time employee globally the benefit of equity ownership in the company through stock option grants and/or restricted stock units. Our U.S. employees are also eligible to participate in an employee stock purchase plan, which offers the opportunity to purchase our common stock at a discount of at least 15%.

## **Our Efforts to Address the COVID-19 Pandemic**

Employee safety and wellbeing is of paramount importance to us in any year and was of particular focus in 2020 in light of the evolving COVID-19 pandemic. In response to the pandemic, we have supported our employees and government efforts to curb the COVID-19 pandemic through safety and communication efforts and investments, which include:

- Creating a COVID-19 task force responsible for establishing COVID-19, safety protocols and regularly communicating updates to all employees;
- A strict working from home policy in the U.S., in which all work that can be done from home, must be done from home;
- Decreasing density and increasing physical distancing in our facilities for employees working onsite using scheduling adjustments and flexibility;
- Mandatory weekly COVID-19 testing for all onsite employees in our U.S. facilities;
- Robust cleaning protocols across all locations;
- Provision of masks to all onsite employees and strict masking requirements;
- Rigorous procedures to address actual and suspected COVID-19 cases and potential exposure; and
- Prohibition of all domestic and international non-essential travel for all employees.

Additionally, from time to time we have instituted additional programs during the pandemic to support our employees, including monthly subsidies to all employees for home office expenses and upgrades, mental well-being private coaching and therapy services.

## **Trademarks & Tradenames**

SANGAMO®, Better Therapeutics By Design®, ZFP Therapeutic® and Engineering Genetic Cures® are our registered trademarks in the United States and Sangamo Therapeutics™ and Pioneering Genetic Cures™ are our trademarks. All other trademarks or trade names referred to in this Annual Report on Form 10-K are the property of their respective owners.

## **Available Information**

Our website is located at [www.sangamo.com](http://www.sangamo.com). This Annual Report on Form 10-K, our Quarterly Reports on Form 10-Q and our Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act are available free of charge on our website as soon as reasonably practicable after we electronically file this material with, or furnish it to, the Securities and Exchange Commission, or SEC. Information found on, or accessible through, our website is not a part of, and is not incorporated into, this Annual Report on Form 10-K. In addition, the SEC maintains a website at [www.sec.gov](http://www.sec.gov) that contains reports, proxy and information statements and other information regarding issuers that file electronically with the SEC.

## **ITEM 1A – RISK FACTORS**

*Our business involves significant risks, some of which are described below. Before making investment decisions regarding our common stock, you should carefully consider these risks, as well as the other information in this Annual Report on Form 10-K, including our financial statements and the related notes and “Management’s Discussion and Analysis of Financial Condition and Results of Operations.” The occurrence of any of the events or developments described below could have a material adverse effect on our business, results of operations, financial condition, prospects and stock price. In such event, the market price of our common stock could decline, and you could lose all or part of your investment. In addition, there are also additional risks not described below that are either not presently known to us or that we currently deem immaterial, and these additional risks could also materially impair our business, operations or market price of our common stock.*

### **Risks Relating to Research, Development, Regulatory Approval and Commercialization of Our Product Candidates and Technologies**

*Our success depends substantially on clinical trial results demonstrating safety and efficacy of our product candidates to the satisfaction of regulatory authorities. We may be unable to obtain positive clinical trial results and regulatory approvals for any of our product candidates.*

We are a clinical-stage biotechnology company with no approved products and no product revenues. We have ongoing clinical trials evaluating product candidates that use our platform technologies in gene therapy and cell therapy and we



anticipate initiating additional clinical trials in the future on other product candidates. We are substantially dependent on the results of these clinical trials, and there is no guarantee that final results of clinical trials conducted on our product candidates now or in the future will demonstrate the safety and efficacy of any of our product candidates. In addition, none of our product candidates have obtained regulatory approval. Obtaining positive clinical trial results and regulatory approvals is expensive, lengthy, challenging and unpredictable and may never occur for any of our product candidates. If we fail to obtain positive clinical trial results and regulatory approvals for our product candidates, our anticipated revenues from our product candidates and our prospects for profitability would be adversely affected, which would likely cause the market price of our common stock to significantly decline.

***Conducting clinical trials and obtaining regulatory approvals is complex and exposes our business to numerous risks, including potential unexpected costs and delays.***

We must conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates to the satisfaction of regulatory authorities in order to obtain regulatory approvals necessary for commercialization. We have limited experience in conducting later stage clinical trials and may not possess the necessary resources and expertise to complete such trials. Clinical trials are expensive, lengthy and unpredictable. 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. Events that may prevent successful or timely completion of clinical development and regulatory approval include, among others:

- delays in reaching a consensus with regulatory authorities on clinical trial design;
- delays in reaching agreement on acceptable terms with prospective clinical research organizations, or CROs, and clinical trial sites;
- delays in opening clinical trial sites or obtaining required institutional review board, or IRB, or independent ethics committee approval at each clinical trial site;
- delays in recruiting and enrolling suitable patients to participate in our clinical trials;
- delays in clinical trial activities due to the evolving COVID-19 global pandemic and the diversion of healthcare resources to fight the pandemic, such as the delays that have previously impacted clinical trial timelines for our Fabry and TX200 programs;
- imposition of clinical holds by regulatory authorities as a result of serious adverse events or after an inspection of clinical trial operations or trial sites;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with the Good Clinical Practice regulations of the U.S. FDA, or applicable regulatory guidelines in the EU and other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions, or as a result of manufacturing or formulation changes to our product candidates;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial;
- selections of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrences of serious adverse events or other safety concerns associated with product candidates that are viewed to outweigh their potential benefits, result in approval delays or other regulatory restrictions, or harm our reputation;
- occurrences of serious adverse events or other safety concerns in clinical trials of the same class of agents conducted by other sponsors;
- failures to demonstrate that product candidates are safe and effective for their proposed indication;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- unexpected costs and expenses and lack of sufficient funding to develop our product candidates; and
- losses of licenses to critical intellectual properties.

We have not yet reached agreement with regulatory authorities on the complete development pathway for certain product candidates, and such authorities have the ability to change decisions or guidance with respect to approvable endpoints, particularly as the technology continues to develop in these areas. For example, we are aware of another company developing a gene therapy to treat hemophilia A that the FDA recommended complete its Phase 3 study and submit two-year follow-up safety and efficacy data on all study participants notwithstanding the company's contention that it and the FDA had previously agreed on the extent of data necessary to support a BLA.

Due to the novelty of certain product candidates and their technologies, the endpoints needed to support regulatory approvals will likely be different from those originally anticipated. Any inability to successfully complete preclinical and clinical development of our product candidates, or complete such trials in the time frames anticipated, could result in additional costs to us or impair our ability to generate revenues from product sales or achieve regulatory and commercialization milestones and royalties, or shorten any periods during which we may have exclusivity.

Even if a product candidate successfully obtains approval from the FDA and comparable foreign regulatory authorities, any approval might contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. Also, any regulatory approval of our product candidates, once obtained, may be withdrawn. If we are unable to obtain and maintain regulatory approvals for our product candidates in one or more jurisdictions, or if any approval contains significant limitations, we would not be able to generate anticipated revenues and may struggle to become profitable, which would have an adverse effect on our business operations and financial conditions.

***Success in research and preclinical studies or early clinical trial results may not be indicative of results obtained in later trials. Likewise, preliminary, initial or interim data from clinical trials may be materially different from final data.***

Results from research and preclinical studies or early clinical trials are not necessarily predictive of future clinical trial results, and interim results of a clinical trial are not necessarily indicative of final results. Our product candidates may fail to show the desired safety and efficacy in clinical trials despite demonstrating positive results in preclinical studies or having successfully advanced through initial clinical trials or preliminary stages of clinical trials. From time to time, we have and may in the future publish or report preliminary, initial or interim data. Preliminary, initial or interim data from our clinical trials and those of our collaborators may not be indicative of the final results of the trial and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and/or more patient data become available. In this regard, such data may show initial evidence of clinical benefit, but as patients continue to be followed and more patient data becomes available, there is a risk that any therapeutic effects will not be durable in patients and/or will decrease over time, or cease entirely. Preliminary, initial or interim data also remain subject to audit and verification procedures that may result in the final data being materially different from such preliminary, initial or interim data. As a result, preliminary, initial or interim data should be considered carefully and with caution until the final data are available. For example, there can be no assurance that the steady-state FVIII levels shown in the preliminary follow-up data presented in December 2020 by Pfizer and us from the Phase 1/2 Alta study of giroctocogene fitelparvovec will persist in future follow-up or any other data from the Alta study, or that giroctocogene fitelparvovec will ultimately demonstrate a clinical benefit in the final results of the Alta study or in the Phase 3 AFFINE clinical trial of giroctocogene fitelparvovec.

There is no guarantee that any of our pending clinical trials will be successful. Many of our product candidates currently use our ZFP technology platform, including ZFN and ZPT-TF technologies, which has not yet yielded any approved therapeutic products. Moreover, many of our product candidates are preclinical and have never demonstrated any clinical benefit. In addition, our viral delivery systems continue to evolve and have not been used in any approved products. If our product candidates using our ZFP technology platform and viral delivery systems are not able to demonstrate the safe, effective and durable results we are hoping to see in clinical trials, we may be forced to suspend or terminate development of some or all of our product candidates or seek alternative technologies to develop or deliver product candidates.

In addition, there is a high failure rate for product candidates proceeding through clinical trials. Many companies in the biopharmaceutical 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. Any such setbacks could adversely affect our business, financial condition, results of operations and prospects.

***Our product candidates are subject to a lengthy and unpredictable regulatory approval process in each jurisdiction where approval is sought.***

A regulatory authority such as the FDA or the EMA must approve any human therapeutic product before it can be marketed in the jurisdiction it governs. The process for receiving regulatory approval is lengthy and unpredictable, and a product candidate may not withstand the rigors of testing under the process. Before commencing clinical trials in humans in the United States, we must submit an IND, to the FDA. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials. In the EU, for example, a clinical trial authorization, or CTA, must be submitted for each clinical protocol to each country's national health authority and an independent ethics committee. Only after an IND becomes effective and/or the applicable CTA has been accepted may clinical trials begin. See "Business—Government Regulation" for details regarding the regulatory approval processes applicable to our product candidates. While there is some overlap, the regulatory requirements to conduct clinical trials and seek marketing approval vary by jurisdiction. There is no guarantee that the safety studies and other data generated will be sufficient to permit us to conduct clinical trials in all jurisdictions where planned, or once generated, that such clinical trial data will be sufficient to obtain marketing approval in all jurisdictions in which we intend to seek such approval. If we are not able to obtain the necessary regulatory approvals to conduct our clinical trials and commercialize our product

candidates, or if such approvals are delayed or suspended, our business, prospects and market price of our common stock would be adversely affected.

***We may not be able to identify, qualify and enroll sufficient patients for our clinical trials or complete our clinical trials in a timely manner, which could delay or prevent us from proceeding with the development of our product candidates.***

Identifying, qualifying and enrolling patients in clinical trials of our product candidates, and completing these clinical trials, is critical to our success. Patient enrollment and trial completion is affected by factors including:

- size of the patient population and process for identifying patients;
- design of the trial protocol;
- eligibility and exclusion criteria;
- perceived risks and benefits of the product candidate under study;
- perceived risks and benefits of genomic approaches to treatment of diseases;
- availability of competing therapies and clinical trials;
- potential delays related to the evolving COVID-19 global pandemic and the diversion of healthcare resources to fight the pandemic;
- severity of the disease under investigation;
- availability of genetic testing for potential patients;
- proximity and availability of clinical trial sites for prospective patients;
- required and desired characteristics of patients;
- ability to obtain and maintain patient consent;
- risk that enrolled patients will drop out before completion of the trial;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

The timing of our clinical trials depends on our ability to recruit patients to participate as well as completion of required follow-up periods. There are also a number of other product candidates in development by our competitors, who compete for the same limited patient populations. If we are not able to enroll the necessary number of patients in a timely manner, we may not be able to complete our clinical trials on our desired timelines or at all. If fewer patients are willing to participate in our clinical trials because of negative publicity from adverse events related to genomic medicines, competitive clinical trials for similar patient populations or for other reasons, the timelines for conducting clinical trials of our product candidates may be delayed. These delays could result in increased costs, limitation or termination of clinical trials, and delays in product development timelines. If we are forced to expand to additional jurisdictions to address these challenges, it could impose additional costs, delays and risks. If we are not successful in conducting our clinical trials as planned, it would have an adverse effect on our business, financial condition, results of operations, prospects and market price of our common stock.

***We may encounter difficulties in advancing product candidates from research programs to preclinical and clinical development.***

We intend to advance our product candidates from research programs through preclinical development and to submit new INDs, CTAs and equivalent filings in other jurisdictions necessary to conduct human clinical trials evaluating our product candidates. The preparation and submission of applications to conduct clinical trials requires us to conduct rigorous and time-consuming preclinical testing and studies and prepare documentation relating to, among other things, the toxicity, safety, manufacturing, chemistry and clinical protocols of our product candidates. We may experience unforeseen difficulties that could delay or otherwise prevent us from executing this strategy successfully. For example, we may encounter problems in the manufacturing of a product candidates and may fail to demonstrate consistency in the formulation of a product candidate. Our preclinical tests may produce negative or inconclusive results, which may lead us to decide, or which may lead regulators to require us, to conduct additional preclinical testing. If we cannot obtain positive results in preclinical testing, we may decide to abandon a product candidate altogether. In addition, our ability to complete and submit such applications to conduct clinical trials may depend on the support of our collaborators and the timely performance of their obligations under relevant collaboration agreements. If our collaborators are not able to perform such obligations or if they choose to slow down or delay the development of a product candidate, we may not be able to submit the clinical trial applications on a timely basis or at all. Furthermore, the submission of applications to conduct clinical trials involves significant cost and labor, and we may not have sufficient resources and personnel to complete the filing of all intended applications, which may force us to scale back the number of applications or forego potential applications that we believe are promising. Any delay, suspension or reduction of our efforts to pursue our preclinical and clinical development strategy could have an adverse effect on our business and cause the market price of our common stock to decline.

***Special regulatory designations, such as RMAT, or orphan drug designations, may not be available for our product candidates or may not lead to a faster development or regulatory review or approval process.***

We have received RMAT, designation for our product candidate to treat severe hemophilia A. Additionally, some of our product candidates, including our product candidate to treat Fabry disease, have also been granted Orphan Drug Designation by the FDA, and some have also been designated Orphan Medicinal Products by the EMA. Regulatory authorities in some jurisdictions, including the United States and the EU, may designate drugs for relatively small patient populations as orphan drugs. For additional information regarding these special regulatory designations, see “Business—Government Regulation.”

If we request such designations for our other current or future product candidates, there can be no assurances that the FDA or the EMA will grant any of our product candidates such designations. Additionally, such designations do not guarantee that any regulatory agency will accelerate regulatory review of, or ultimately approve, those product candidates, nor does it limit the ability of any regulatory agency to grant such designations to product candidates of other companies that treat the same indications as our product candidates prior to our product candidates receiving exclusive marketing approval. Such designations can also be revoked. RMAT designation can be revoked if the criteria for eligibility cease to be met as clinical data emerges. Orphan drug exclusivity may be revoked if any regulatory agency 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 patients with the rare disease or condition.

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

During the conduct of clinical trials, patients report changes in their health, including illnesses, injuries and discomforts, to their study doctor. Often, it is not possible to determine whether or not the product candidate being studied caused these conditions, particularly as many of the diseases we are studying have complex comorbidities. If clinical experience indicates that a product candidate has side effects or cause serious or life-threatening side effects, the development of the product candidate may fail or be delayed, or, if the product candidate has received regulatory approval, such approval may be revoked, which would severely harm our business, prospects, operating results and financial condition.

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 trials using other genomic therapies. Gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of significantly 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 could occur with treatment with gene therapy products include an immunologic reaction early after administration that, while not necessarily adverse to the patient’s health, could substantially limit the effectiveness of the treatment.

***Even if our product development efforts are successful and even if the requisite regulatory approvals are obtained, our products may not gain market acceptance among physicians, patients, healthcare payors and the medical community.***

Even if we obtain regulatory approval for any of our product candidates that we may develop or acquire in the future, the approved product may not gain market acceptance among physicians, healthcare payors, patients or the medical community. Market acceptance of approved products depends on a number of factors, including:

- the efficacy and safety of the product as demonstrated in clinical trials;
- the clinical indications and patient populations for which the product is approved;
- acceptance by physicians, treatment centers and patients of the product as a safe and effective treatment;
- the adoption of novel genomic therapies by physicians, hospitals and third-party payors;
- the potential and perceived advantages of the product over alternative treatments;
- the safety of the product seen in a broader patient group, including its use outside the approved indications;
- any restrictions on product use together with other medications;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other regulatory authorities;
- the timing of market introduction of the product as well as competitive products;
- the development of manufacturing and distribution processes for the product;
- the cost of treatment in relation to alternative treatments;
- the availability of coverage and adequate reimbursement and the willingness of patients to pay out-of-pocket in the absence of coverage or inadequacy of reimbursement by third-party payors and government authorities;
- relative convenience and ease of administration; and

- the effectiveness of our sales and marketing efforts and those of our collaborators.

If any of our product candidates are approved but fail to achieve market acceptance among physicians, patients, healthcare payors or treatment centers, we will not be able to generate significant revenues from the approved product, which would compromise our ability to become profitable.

***Even if we are able to commercialize any approved products, such products may not receive coverage and adequate reimbursement from third-party payors in the United States and in other countries in which we seek to commercialize them, which could harm our business.***

Our ability to commercialize any product successfully will depend, in part, on the extent to which coverage and adequate reimbursement for these products and related treatments will be available. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, determine which medications they will cover and establish reimbursement levels, which can affect demand for, or the price of, any approved product. Given the nature of the product candidates that we are developing, some patients may require treatment only one-time (*e.g.*, single dose administration), and there is substantial uncertainty about the pricing structure for such products, and the level of coverage and reimbursement that will be available for a shift to single-dose treatment as compared to chronic therapy over a patient's lifetime. If other companies establish a new pricing structure or business model, including payment based on demonstration of long-term efficacy, our ability to price or obtain reimbursement for our products may be adversely affected. If such pricing structure or business model do not adequately fund the costs of our research and development, manufacturing and commercialization efforts, our business may be adversely affected.

In addition to uncertainty about the potential pricing structure for certain of our product candidates, cost containment is a recurrent trend in the healthcare industry. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for certain medications. We cannot be sure that coverage and adequate reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be. If reimbursement is not available or is available only at limited levels, we may be unable to successfully commercialize any product candidate for which we obtain regulatory approval. Our inability to promptly obtain coverage and profitable reimbursement rates from both government-funded and private payors for any approved products that we develop could have an adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

***Recently enacted and future legislation, including potentially unfavorable pricing regulations or other healthcare reform initiatives, may increase the difficulty and cost for us to obtain regulatory approval of and commercialize our product candidates and affect the prices we may obtain.***

The regulations that govern, among other things, regulatory approvals, coverage, pricing and reimbursement for new drug products vary widely from country to country. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay regulatory approval of our product candidates, restrict or regulate post-approval activities and affect our ability to successfully sell any product candidates for which we obtain regulatory approval. Also, there has been heightened governmental scrutiny recently over biopharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for biopharmaceutical products. At the state level, legislatures have increasingly passed legislation and implemented 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, have been designed to encourage importation from other countries and bulk purchasing. For a discussion of health reform activity and the current pricing framework, see "Business—Government Regulation—Healthcare Reform" and "—Pricing, Coverage and Reimbursement."

The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

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

Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability.

***Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny.***

Even if we obtain regulatory approval in a jurisdiction, the regulatory authority may still impose significant restrictions on the indicated uses or marketing of our product candidates or impose ongoing requirements for potentially costly post-approval studies, post-market surveillance or patient or drug restrictions. For example, the FDA typically advises that patients treated with gene therapy undergo follow-up observations for potential adverse events for a 15-year period. Additionally, the holder of an approved BLA is required to comply with FDA rules and is subject to FDA review and periodic inspections, in addition to other potentially applicable federal and state laws, to ensure compliance with cGMP and adherence to commitments made in the BLA.

If we or a regulatory agency discovers 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 agency may impose restrictions relative to that product or the manufacturing facility, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. Moreover, product labeling, advertising and promotion for any approved product will be subject to regulatory requirements and continuing regulatory review. Failure to comply with such requirements, when and if applicable, could subject us to a number of actions ranging from warning letters to product seizures or significant fines, among other actions. See “Business—Government Regulation—U.S. Review and Approval Processes” for more information.

Any government investigation of alleged violations of laws or regulations could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and generate revenues.

***Our employees or contractors may engage in misconduct or other improper activities, including noncompliance with research, development, manufacturing or regulatory standards and requirements, which could cause significant liability for us and harm our reputation.***

We are exposed to the risk of fraud or other misconduct by our employees and contractors, 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 we have established, 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. Misconduct by our employees and contractors 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. It is not always possible to identify and deter such 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 or regulations. 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 civil, criminal and administrative penalties, damages, fines, disgorgement, personal imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations.

***We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on other programs or product candidates that may be more profitable or for which there is a greater likelihood of success.***

We have limited resources and may forego or delay pursuit of certain research programs or product candidates that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities or pursue collaborations rather than retain sole responsibility for development. Our current and future research and development programs for product candidates may not yield any commercially viable products. The evaluation of the commercial potential or target market for a particular product candidate is forward-looking and based upon assumptions involving, for example and not limited to, market evolution, advances in disease

standard of care, competition and reimbursement. This reliance on assumptions means that, if our assumptions prove to be inaccurate or incomplete, we may pursue opportunities that end up having a number of competitors that are more advanced than our product candidates, or we may relinquish valuable rights to a product candidate through strategic collaboration, licensing or other royalty arrangements in cases where it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. We may also allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a collaboration or that does not prove to have viable commercial opportunities. Any failure to use our financial and human resources efficiently could harm our business and operations.

***ZFP technology is novel and has never been used to develop any approved, commercially viable therapeutic products.***

Our ZFP technology is a novel technology which to date has not yielded any approved commercially viable therapeutic products, and there can be no guarantee that our product development efforts using ZFP technology will be fruitful. We have invested heavily in development of this technology, and our failure to develop approved, commercially viable products using ZFP technology would significantly limit our business and prospects and would adversely impact the market value of our common stock.

## **Risks Relating to Manufacturing**

***We are building manufacturing facilities for clinical trial supplies. We have limited experience manufacturing biopharmaceutical products, and there can be no assurance that we will be able to maintain compliant manufacturing facilities, build additional facilities and manufacture our product candidates as intended.***

We expect to use both contract manufacturing organizations, or CMOs, and our own facilities to meet our projected needs for clinical trial supply. In 2020, we completed the construction of an AAV manufacturing facility in our Brisbane, California headquarters building to manufacture Phase 1/2 clinical study supplies for our gene therapy product candidates and we are building additional facilities in our headquarters building and in Valbonne, France to manufacture supplies for our cell therapy product candidates. We expect the cell therapy facilities to be operational by the end of 2021. Building these facilities requires us to transition manufacturing processes and know-how of our product candidates from our CMOs to our own facilities. Transferring manufacturing processes and know-how is complex and involves review and incorporation of both documented and undocumented processes that may have evolved over time. In addition, transferring production to different facilities may require utilization of new or different processes to meet the specific requirements of a given facility. Additional studies may also need to be conducted to support the transfer of certain manufacturing processes and process improvements. We cannot be certain that all relevant know-how and data has been adequately incorporated into the manufacturing process until the completion of studies and evaluations intended to demonstrate the comparability of material previously produced by CMOs with that generated by our facilities. Although some of our employees have experience in the manufacturing of biopharmaceutical products from prior employment at other companies, we, as a company, have no prior experience in biopharmaceutical product manufacturing, and operating these facilities will require us to comply with complex regulations and to continue to hire and retain experienced scientific, quality control, quality assurance and manufacturing personnel. Designing and building manufacturing facilities has been and will continue to be time-consuming and expensive, and we may experience delays or cost overruns. In addition, government approvals are required for us to operate manufacturing facilities and are time-consuming to obtain and maintain. As a manufacturer of biopharmaceutical products, we also will be required to demonstrate and maintain cGMP compliance. These requirements include, among other things, quality control, quality assurance and the maintenance of records and documentation. Furthermore, establishing manufacturing operations will require a reallocation of other resources, particularly the time and attention of our senior management. Even if we are able to establish our own manufacturing capabilities, we could encounter challenges in operating the manufacturing facilities in compliance with cGMP, regulatory or other applicable requirements, resulting in potential negative consequences, including regulatory actions, which could undermine our ability to use these facilities for our own manufacturing needs. Any failure or delay in the development of our manufacturing capabilities could adversely impact the development of our product candidates.

***The manufacture, storage and transport of our product candidates is complex, expensive, highly regulated and risky, which could hamper their commercial viability.***

There are significant risks associated with manufacturing, storing and transporting our product candidates including, among others, cGMP compliance, cost overruns, technical problems with process scale-up, specialized facilities, process reproducibility, stability issues, lot consistency, yields and timely availability of highly specific raw materials. Even though product batches released for use in clinical trials undergo sample testing, some defects may only be identified following release. In addition, process deviations or unanticipated effects of approved process changes may result in these intermediate products

not complying with stability requirements or specifications. Also, our product candidates must be stored and transported at temperatures within a certain range. If these environmental conditions deviate, our product candidates' remaining shelf-lives could be impaired or their efficacy and safety could be adversely affected, making them no longer suitable for use. Moreover, product candidates that are biologics involving complex processes, including the development of cell lines or cell systems to produce the biologic, with the challenge of significant variability. There are difficulties in growing large quantities of such cells, consistently and sufficiently isolating certain types of cells and harvesting and purifying the biologic produced by them. The cost to manufacture biologics is generally far higher than traditional small molecule chemical compounds, and the manufacturing process can be difficult to reproduce.

Moreover, manufacturing, storing and transporting our product candidates is subject to strict regulatory standards, which adds additional production risk. Even if efficacy and safety data from our clinical trials would otherwise support regulatory approval of a product candidate, there is no assurance that we or our CMOs will be able to manufacture our product candidates to specifications at levels necessary to support or maintain regulatory approval by the FDA or other regulatory authorities.

Thus, there is no guarantee we will be successful in establishing a larger-scale commercial manufacturing process for our product candidates or obtaining the needed manufacturing capacity. Due to these manufacturing challenges, there is risk that some of our product candidates could be subject to inventory outages, reputational damage and product liability risks, and result in additional expense and delays to clinical trials and commercialization. Supply interruptions or shortages could result in potential negative impacts to our business, prospects and market price of our common stock.

***If we use chemical, biological or hazardous materials in a manner that causes injury or violates laws, we may be liable for damages.***

Our research and development activities involve the controlled use of potentially harmful biological materials as well as hazardous materials, chemicals, and various radioactive compounds typically employed in the study of molecular and cellular biology. We routinely use cells in culture and gene delivery vectors, and we employ small amounts of radioisotopes in trace experiments. Although we maintain up-to-date licensing and training programs, we cannot completely eliminate the risk of accidental contamination or injury from the use, storage, handling, or disposal of these materials. In the event of contamination or injury, we could be held liable for damages that result, and any liability could exceed our resources. We currently carry insurance covering certain claims arising from our use of these materials. However, if we are unable to maintain our insurance coverage at a reasonable cost and with adequate coverage, our insurance may not cover any liability that may arise. We are subject to federal, state, and local laws and regulations governing the use, storage, handling, and disposal of these materials and specified waste products. Failure to comply with these laws and regulations could result in fines, penalties and additional liabilities and restrictions on our operations.

***We currently rely on third parties to conduct some or all aspects of manufacturing of our product candidates for preclinical and clinical development. If one of our third-party manufacturers fails to perform adequately or fulfill our needs, we may be required to incur significant costs and devote significant efforts, to find new suppliers or manufacturers.***

We currently have limited experience in clinical-scale manufacturing of our product candidates, and we rely in large part upon third-party CMOs to manufacture and supply drug product for our preclinical studies and clinical trials. Although we are in the process of establishing manufacturing facilities in Brisbane, California and Valbonne, France, these facilities will only manufacture limited quantities of our product candidates for our early-stage clinical trials. We intend to continue to rely on third parties for the manufacture of product candidates for later stage clinical trials, and for commercial-scale manufacturing for any approved product. The manufacture of biopharmaceutical products in compliance with the FDA's cGMP requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of biopharmaceutical products often encounter difficulties in production, including difficulties with production costs and yields, quality control, including stability of the product candidate and quality assurance testing, shortages of qualified personnel, as well as compliance with strictly enforced cGMP requirements, other federal and state regulatory requirements and foreign regulations. If our manufacturers were to encounter any of these difficulties or otherwise fail to comply with their obligations to us or under applicable regulations, our ability to conduct later-stage clinical trials could be jeopardized. Any delay or interruption in the supply of clinical trial materials could delay the completion of our clinical trials, increase the costs associated with developing our product candidates and, depending upon the period of delay, require us to commence new clinical trials at significant additional expense or terminate the clinical trials completely.

We and our CMOs must comply with cGMP requirements enforced by the FDA through its facilities inspection program. These requirements include, among other things, quality control, quality assurance and the maintenance of records and documentation. We and our CMOs may be unable to comply with these cGMP requirements and with other FDA, state and



foreign regulatory requirements. The FDA or similar foreign regulatory agencies may also implement new standards at any time or change their interpretation and enforcement of existing standards for manufacture, packaging or testing of products. We have limited control over our manufacturers' compliance with these regulations and standards. Failure to comply with these requirements may result in fines and civil penalties, suspension of production, suspension or delay in product approval, product seizure or recall or withdrawal of product approval. If the safety of any product supplied is compromised due to our manufacturers' failure to adhere to applicable laws or for other reasons, we may not be able to obtain regulatory approval for or successfully commercialize our products and we may be held liable for any injuries sustained as a result. Any of these factors could cause a delay of clinical trials, regulatory submissions, approvals or commercialization of our product candidates, entail higher costs or impair our reputation.

Our current agreements with our CMOs do not provide for the entire supply of the drug product necessary for all anticipated clinical trials or for full scale commercialization. If we and our CMOs cannot agree to the terms and conditions for them to provide the drug product necessary for our clinical and commercial supply needs, we may not be able to manufacture the product candidate until a qualified alternative manufacturer is identified, which could also delay the development of, and impair our ability to commercialize our product candidates.

The number of third-party CMOs with the necessary manufacturing and regulatory expertise and facilities is limited, and it could be expensive and take a significant amount of time to arrange for alternative CMOs, which could have an adverse effect on our business. New manufacturers of any product candidate would be required to qualify under applicable regulatory requirements and would need to have sufficient rights under applicable intellectual property laws to the method of manufacturing the product candidate. Obtaining the necessary FDA approvals or other qualifications under applicable regulatory requirements and ensuring non-infringement of third-party intellectual property rights could result in a significant interruption of supply and could require the new manufacturer to bear significant additional costs which may be passed on to us.

***We and third parties on which we rely may be adversely affected by natural disasters and catastrophic or other events outside of our control, and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster or event.***

Natural disasters could severely disrupt our operations and our facilities, including our current and planned manufacturing facilities in Brisbane, California and Valbonne, France and the manufacturing facilities of our CMOs, and any disruption would likely have a negative impact on our business, financial condition, results of operations and prospects. If a natural disaster, pandemic or epidemic, including the evolving COVID-19 pandemic, political crisis, power outage or any other event that is out of our control occurred that prevented us or third parties on which we rely from using all or a significant portion of our or their facilities, that damaged critical infrastructure or that otherwise disrupted our or their operations, it may be difficult or, in certain cases, impossible for us to continue our business and operations for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and may not prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have an adverse effect on our business, financial condition, results of operations and prospects. Such disasters or events occurring at facilities of third parties on which we rely could also negatively impact our business and operations.

## **Risks Relating to our Industry**

***Our product candidates are based on novel genomic medicine technologies, which makes it difficult to predict the timing and costs of development and of subsequently obtaining regulatory approval.***

We have concentrated our research and development efforts on genomic medicine, consisting of gene therapy, gene-edited cell therapy and genome engineering. The regulatory approval process for novel product candidates such as ours is unclear and may be lengthier and more expensive than the process for other, better-known or more extensively studied product candidates.

Adverse developments in clinical trials of genomic medicines conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of our product candidates.

These regulatory review committees and advisory groups, and any new guidelines they promulgate, may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our current or future product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates,

we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. 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, and our business, financial condition, results of operations and prospects would be harmed. Even if our product candidates are approved, we expect that the FDA will require us to submit follow-up data regarding our clinical trial patients for a number of years after any approval. If this follow-up data shows negative long-term safety or efficacy outcomes for these patients, the FDA may revoke its approval or change the label of our products in a manner that could have an adverse impact on our business.

In addition, adverse developments in clinical trials of genomic medicines conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of our product candidates. The FDA and EMA have only very recent and limited experience in the approval of *in vivo* gene therapy products. 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 product candidates.

***If we or our competitors develop, acquire, or market technologies or products that are more effective than ours, our financial condition and ability to successfully market or commercialize our product candidates or be profitable would be adversely affected.***

The biopharmaceutical industry is highly competitive and subject to significant and rapid technological change. We are aware of several companies focused on other methods for editing cells, editing genes and regulating gene expression and a growing number of commercial and academic groups pursuing the development of genome engineering technology. The field of genomic medicine is highly competitive, and we expect competition to persist and intensify in the future from a number of different sources, including biopharmaceutical companies, academic and research institutions, and government agencies that will seek to develop competing products as well as technologies that will compete with our ZFP technology platform. For example, in genome engineering and gene therapy products, competing proprietary technologies with our product development focus include but are not limited to, recombinant proteins, other gene therapy/cDNAs, antisense, siRNA and microRNA approaches, exon skipping, small molecule drugs, monoclonal antibodies, CRISPR/Cas technology and TALE proteins, meganucleases, and MegaTALs. See “Business—Competition” for more information on the competition we may face.

Any products that we or our collaborators or strategic partners develop will enter into highly competitive markets. Even if we are able to generate products that are safe and effective for their intended use, competing technologies may prove to be more effective or less expensive, which, to the extent these competing technologies achieve market acceptance, will limit our revenue opportunities. In some cases, competing technologies have proven to be effective and less expensive. Competing technologies may include other methods of regulating gene expression or modifying genes. ZFNs and ZFP-TFs have broad application in the life sciences industry and compete with a broad array of new technologies and approaches being applied to genetic research by many companies.

In addition to possessing competing technologies, our competitors include biopharmaceutical companies with:

- substantially greater capital resources than ours;
- larger research and development staffs and facilities than ours; and
- greater experience in product development and in obtaining regulatory approvals and patent protection.

These organizations also compete with us to attract qualified personnel, attract parties for acquisitions, joint ventures or other collaborations and license the proprietary technologies of academic and research institutions that are competitive with our technology, which may preclude us from pursuing similar opportunities. Accordingly, our competitors may succeed in obtaining patent protection or commercializing products before us. Even if our product candidate is more effective, it may be disadvantaged if it is not first to market. In addition, any products that we develop may compete with existing products or services that are well established in the marketplace. Further, some of our product candidates in development are designed for use once. Any success in developing single-dose therapeutics could cause us to lose potential recurring revenues from therapeutics that are designed to be taken over a patient’s lifetime.

***The evolving COVID-19 pandemic could continue to adversely impact our business and operations and the business and operations of our collaborators, manufacturers and other business partners.***

In March 2020, the World Health Organization declared the novel coronavirus, or COVID-19, outbreak a pandemic, and since such time, actions taken around the world to help mitigate the spread of COVID-19 have included varying restrictions on travel, quarantines in certain areas, and forced closures for certain types of public places and businesses, including in the

three countries where we have most of our day-to-day operations, the United States, France and the United Kingdom. Our business has been directly impacted by pandemic restrictions aimed at reducing the spread of the disease, including multiple California executive orders, several semi-coordinated San Francisco Bay Area orders, several other state and additional local orders across the country and similar orders outside the United States, which, among other things, direct individuals to shelter at their places of residence, direct businesses and governmental agencies to cease non-essential operations at physical locations, prohibit certain non-essential gatherings, and order cessation of non-essential travel.

To comply with these orders, we implemented an operating plan to continue business operations during the ongoing COVID-19 pandemic, including enhanced workplace safety protocols and modified working schedules in our laboratories. In the United States, employees who are able to fulfill their job duties working from home are required to work from home, and have been doing so since March 2020. These protocols and modifications have slowed our productivity and disrupted our business to a moderate degree and are likely to continue doing so through 2021. For example, we have experienced periodic short-term disruptions to our onsite laboratory and manufacturing operations while addressing positive cases of COVID-19 by onsite workers, and our laboratory and manufacturing operations could experience longer term disruptions in the future in the event of a significant outbreak of COVID-19 among our onsite workers. Moreover, from time to time, we have been required to reorganize and prioritize our research resources to mitigate moderate COVID-19 impacts arising from travel restrictions, laboratory density restrictions and laboratory supply constraints. If our research programs encounter longer-term disruptions, it could impact our ability to support our biopharmaceutical partners as contemplated in our collaboration agreements and could result in adjustments to our research timelines, although we do not believe that the short-term disruptions to date have resulted in any such impacts.

Additionally, our Phase 1/2 STAAR clinical study evaluating ST-920, our wholly-owned gene therapy product candidate for the treatment of Fabry disease, has experienced delays in its timeline due to COVID-19 impacts and the diversion of healthcare resources to fight the pandemic. For example, the clinical trial site in the UK for this study has not been able to open due to the significant prevalence of COVID-19 in the UK. Additionally, we have experienced delays in recruiting, enrolling and dosing patients for this study at our US trial sites, due in some part to the understandable hesitation of patients to travel by plane to trial sites not within driving distance and to enter medical facilities during the pandemic and in other part to trial sites prioritizing COVID-19 clinical care over research activities such as the STAAR study. Moreover, we have experienced some short-term delays in sourcing the necessary raw materials to manufacture supplies for the STAAR study due to COVID-19 impacts. We estimate that these challenges have set back our STAAR study timelines three to six months. While we currently still expect to share initial clinical study data by the end of 2021, this timeline could be revised if COVID-19 impacts to our enrollment and dosing of patients and to our sourcing of raw materials for this study intensify because of vaccination delays, new COVID-19 variants or unexpected events.

In addition, our STEADFAST clinical study evaluating TX200, our wholly-owned CAR-Treg cell therapy product candidate for the treatment of kidney transplant rejection, has experienced delays in its commencement timeline due to COVID-19 impacts related to manufacturing and technology transfer challenges with our CMOs. We estimate that these challenges have set back our commencement timeline by approximately three months. While we currently still expect to initiate this clinical study by the end of 2021, this timeline could be revised if COVID-19 impacts result in additional delays.

With respect to our partnered programs, the timelines for the studies and trials managed by our collaborators are also subject to potential delay in the future if these studies and trials experience similar challenges that we have experienced in our STAAR and STEADFAST studies.

While we have been working with our collaborators, clinical trial sites and CMOs to minimize any impact of COVID-19 on clinical trials and research and development operations conducted by us and our collaborators, we do expect that at least some of our programs will nonetheless experience delays and disruptions in the future due to COVID-19 impacts on the operations of us and our business partners. These delays and disruptions have in the past and could in the future relate to clinical site initiation, patient recruitment and enrollment or dosing of patients. Some patients and clinical trial staff may not be able or willing to comply with clinical trial protocols if quarantines impede movement or interrupt healthcare services.

It is uncertain when restrictions will be fully lifted, and if so, when we will be able to resume pre-pandemic work routines. Imposition of government orders, including quarantine and shelter-in-place orders related to COVID-19 or other infectious diseases, is expected to continue to impact personnel at our laboratories and our third-party manufacturing facilities in the United States and other countries, for the foreseeable future, and could impact the availability or cost of materials, which would disrupt our supply chain. Many of our third-party manufacturers which we use for the supply of materials for product candidates or other materials necessary to manufacture product to conduct preclinical tests and clinical trials are located in countries heavily affected by the COVID-19 pandemic, and should they experience disruptions in the future, such as temporary closures or suspension of services, we would likely experience delays in advancing these tests and trials.

While the potential economic impact brought by, and the duration of, the COVID-19 pandemic may be difficult to assess or predict, it could continue to result in significant disruption of global financial markets, impairing our ability to access capital, which could in the future negatively affect our liquidity. In addition, a recession or market correction resulting from the COVID-19 pandemic could materially affect our business and the value of our common stock.

The extent to which the COVID-19 pandemic impacts our business, our clinical development and regulatory efforts will depend on future developments that remain highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration and severity of the pandemic, travel restrictions, quarantines and social distancing requirements in the United States, France, United Kingdom and other countries, business closures or business disruptions and the effectiveness and timeliness of actions taken in the United States, France, United Kingdom and other countries to contain and treat the disease, including the effectiveness and timing of vaccination programs in the United States and worldwide. Accordingly, we do not yet know the full extent of potential delays or impacts on our business, sales of our products, our clinical and regulatory activities, healthcare systems or the global economy as a whole. However, these effects could have material adverse impacts on our business, financial condition, results of operations and growth prospects.

In addition, to the extent the evolving COVID-19 pandemic continues to adversely affect our business and results of operations, it may also have the effect of heightening many of the other risks and uncertainties described in this “Risk Factors” section.

***Negative public opinion and increased regulatory scrutiny of genomic medicines may damage public perception of the safety of our product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.***

Genetically modified products are currently subject to public debate and heightened regulatory scrutiny. Gene therapy remains a novel technology, with only two *in vivo* gene therapy products approved for a genetic disease to date in the United States and only a few *in vivo* gene therapy products for genetic diseases approved to date in the EU. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. For example, reports of serious adverse events in a retroviral gene transfer trial for infants with X-linked severe combined immunodeficiency (X-linked SCID) in France and subsequent FDA actions putting related trials on hold in the United States had a significant negative impact on the public perception and stock price of certain companies involved in gene therapy, whether or not the specific company was involved with retroviral gene transfer, or whether the specific company’s clinical trials were placed on hold in connection with these events. Other adverse events could occur in the field of genomic medicine that could result in increased regulatory scrutiny, potential regulatory delays or negative impact on public perception genomic medicines, which could cause our stock price to decline.

In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates, prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available.

Even if the regulatory approval for genetically modified products developed using our technology is obtained, our success will also depend on public acceptance of the use of genetically modified products including medicines, plants and plant products. Claims that genetically modified products are unsafe for consumption or pose a danger to the environment may influence public attitudes. Our genetically modified products may not gain public acceptance. More restrictive government regulations or negative public opinion would have an adverse effect on our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, earlier gene therapy trials led to several well-publicized adverse events, including cases of leukemia and death seen in other trials using other vectors. Serious adverse events in our clinical trials, or other clinical trials involving gene therapy 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 product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

***Our current and future relationships with healthcare providers, customers and third-party payors subject us to applicable anti-kickback, fraud and abuse, privacy, data security and other healthcare laws and regulations. If we fail to comply with such regulations, we could face substantial penalties and our business, results of operations, financial condition and prospects could be adversely affected.***

Healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain regulatory approval. Arrangements with healthcare providers, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we would market, sell and distribute our products. As a biotechnology company, even though we will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, federal and state healthcare laws and regulations pertaining to fraud and abuse, transparency, health privacy and security and patients' rights are and will be applicable to our business. For details regarding the restrictions under applicable federal and state healthcare laws and regulations that may affect our ability to operate see "Business—Government Regulation—Additional Regulation".

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Scrutiny has also increased, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. Responding to investigations can be time- and resource-consuming and can divert management's attention from the business. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. If our operations or if any physicians or other healthcare providers or entities with whom we expect to do business are found to not be in compliance with applicable laws or applicable regulations, we and they could be subjected to significant civil, criminal and administrative enforcement actions, see "Business—Government Regulation—Additional Regulation".

Further, we are required to comply with privacy and data security laws, such as the GDPR and the CCPA, and after January 1, 2023, the California Privacy Rights Act, which apply to the collection, use, disclosure, transfer, or other processing of personal data. For more information regarding these regulations, see "Business—Government Regulation—Privacy Regulation". To comply with the GDPR restrictions on the transfer of personal data from Europe, we have relied on Standard Contractual Clauses. However, a July 2020 decision of the EU's highest court has called into question this practice, and UK authorities may similarly question the viability of the Standard Contractual Clauses as a mechanism for the lawful transfer of personal data outside of that country. Moreover, there is uncertainty regarding how 'Brexit' will impact these practices. If we are unable to implement safeguards necessary to ensure that our transfers of personal data from and within Europe are lawful, we will face increased exposure to regulatory actions, substantial fines, and injunctions against processing personal data from Europe, and could be required to increase our data processing capabilities in Europe at significant expense. Restrictions on our ability to transfer personal data from Europe could impact our clinical trial activities in Europe and limit our ability to collaborate with CROs and other third parties subject to European data protection laws. Other countries may adopt restrictions similar to the GDPR, which could further impact our operations.

Any failure or alleged failure (including as a result of deficiencies in our policies, procedures or measures relating to privacy, data security, marketing or communications) by us to comply with laws, regulations, policies, legal or contractual obligations, industry standards or regulatory guidance relating to privacy or data security, may result in governmental investigations and enforcement actions, litigation, fines and penalties or adverse publicity. In addition, new regulation, legislative actions or changes in interpretation of existing laws or regulations regarding privacy and data security (together with applicable industry standards) may increase our costs of doing business. In this regard, we expect that there will continue to be new proposed laws, regulations and industry standards relating to privacy and data security in the United States, the EU and other jurisdictions, and we cannot determine the impact such future laws, regulations and standards will have on our business.

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

We face inherent risks of product liability exposure related to the testing of our product candidates in human clinical trials and will face even greater product liability risks if we commercially sell any approved products. Product liability claims may be brought against us by subjects enrolled in our clinical trials, patients, healthcare providers or others using, administering or selling our products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop;
- termination of clinical trial sites or entire trial programs;

- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to clinical trial patients;
- loss of revenue;
- diversion of management and scientific resources from our business operations; and
- the inability to commercialize any products that we may develop.

We currently hold product liability insurance coverage at a level that we believe is customary for similarly situated companies and adequate to provide us with insurance coverage for foreseeable risks, but which may not be adequate to cover all liabilities that we may incur. 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. We intend to expand our insurance coverage for products to include the sale of commercial products if we obtain regulatory approval for our product candidates in development, but we may be unable to obtain commercially reasonable product liability insurance for any products that receive regulatory approval. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us, particularly if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business.

## **Risks Relating to our Finances**

***We have incurred significant operating losses since inception and anticipate that we will incur continued losses for the foreseeable future.***

We have generated operating losses since we began operations in 1995. The extent of our future losses and the timing of profitability are uncertain, and we expect to incur losses for the foreseeable future. We have been engaged in developing our ZFP technology since inception, which has and will continue to require significant research and development expenditures. To date, we have generated our funding from issuance of equity securities, revenues derived from collaboration agreements, other strategic partnerships in non-therapeutic applications of our technology, federal government research grants and grants awarded by research foundations. We expect to continue to incur additional operating losses for the next several years as we continue to develop our product candidates. If the time required to generate significant product revenues and achieve profitability is longer than we currently anticipate or if we are unable to generate liquidity through equity financing or other sources of funding, we may be forced to curtail or suspend our operations.

***We may be unable to raise additional capital on favorable terms, if at all, which would harm our ability to develop our technology and product candidates and could delay or terminate some or all of our programs. Future sales and issuances of equity securities could also result in substantial dilution to our stockholders.***

We have incurred significant operating losses and negative operating cash flows since inception and have not achieved profitability. We expect capital outlays and operating expenditures to increase over the next several years as we expand our infrastructure and research and product development activities. While we believe our available cash, cash equivalents and marketable securities as of December 31, 2020, when combined with additional capital raises and expected revenues from collaborations, strategic partners and research grants, will be adequate to fund our currently planned operations through at least the next 12 months from the date the financial statements in this Annual Report on Form 10-K are issued, we will need to raise substantial additional capital to fund the development, manufacturing and potential commercialization of our product candidates. We regularly consider fund raising opportunities and may decide, from time to time, to raise capital based on various factors, including market conditions and our plans of operation. In addition, as we focus our efforts on proprietary human therapeutics, we will need to seek FDA approvals of our product candidates, a process that could cost in excess of hundreds of millions of dollars per product. We may experience difficulties in accessing the capital markets due to external factors beyond our control, such as volatility in the equity markets for emerging biotechnology companies and general economic and market conditions both in the United States and abroad. For example, our ability to raise additional capital may be adversely impacted by global economic conditions and disruptions to and volatility in the credit and financial markets in the United States and worldwide, which could be impacted by the evolving COVID-19 pandemic. We cannot be certain that we will be able to obtain financing on terms acceptable to us, or at all. Our failure to obtain adequate and timely funding will adversely affect our business and our ability to develop our technology and products candidates.

To the extent we raise additional capital by issuing equity securities, including sales pursuant to our at-the-market offering program with Jefferies LLC, our stockholders may experience substantial dilution. We may issue common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. New investors could gain rights superior to our existing stockholders.

***Our ability to use net operating losses to offset future taxable income may be subject to limitations.***

Although a certain amount of our federal net operating loss carryforwards carry forward indefinitely (but are subject to a percentage limitation), a significant amount of our federal and all of our state net operating loss carryforwards will begin to expire, if not utilized, beginning in 2024 and 2029, respectively. The net operating loss carryforwards subject to expiration could expire unused and be unavailable to offset future income tax liabilities. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an “ownership change,” which is generally defined as a greater than 50 percentage point change in its equity ownership value over a three-year period, the corporation’s ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. We have experienced an ownership change in the past and we may also experience additional ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. If an ownership change occurs and our ability to use our net operating loss carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations. In addition, at the state level, there may be periods during which the use of net operating loss carryforwards is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. For example, California has imposed limits on the usability of California state net operating losses to offset California taxable income in tax years beginning after 2019 and before 2023. As a result, if we earn net taxable income, we may be unable to use all or a material portion of our net operating loss carryforwards and other tax attributes, which could potentially result in increased future tax liability to us and adversely affect our future cash flows.

***If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.***

The market price of our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. In the event securities or industry analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

**Risks Relating to our Reliance on Third Parties**

***If conflicts arise with our contractors, collaborators or other business partners, these conflicts may limit our ability to implement our strategies and may harm our business and prospects.***

If conflicts arise with our contractors, collaborators or other business partners, the other party will likely act in its self-interest, which may limit our ability to implement our strategies. For example, some of our collaborators are conducting multiple product development efforts within each area that is the subject of their collaboration with us. Our collaborators may develop, either alone or with others, product candidates in related fields that are competitive with the product candidates that are the subject of their collaborations with us. Competing products, either developed by the collaborators or to which the collaborators or have rights, may result in the withdrawal of their support for our product candidates.

Some of our collaborators could also become our competitors in the future. Our collaborators could develop or invest in competing products, preclude us from entering into collaborations with their competitors, fail to obtain timely regulatory approvals, terminate or breach their agreements with us unexpectedly or prematurely, or fail to devote sufficient resources to the development and commercialization of product candidates covered by the collaboration.

In addition, conflicts could arise between us and our collaborators resulting from disputes regarding our or our collaborators’ or strategic partners’ performance under the applicable agreement, including disputes arising from alleged breaches of our agreements with our collaborators.

Any of these conflicts could harm our product development efforts and otherwise adversely affect our business and prospects.

***Our collaborators control certain aspects of our product development efforts, including certain of our clinical trials, which could result in unanticipated delays and other obstacles in the commercialization of our product candidates.***

We depend on collaborators to design and conduct certain of our clinical trials for some of our product candidates. As a result, these clinical trials may not be conducted in the manner or on the timeline we desire, which may negatively impact our

product development efforts. In addition, if any of these collaborators withdraws support for our product candidates or otherwise impairs their development, our business could be negatively affected.

Our lack of control over aspects of product development in our agreements with Novartis, Biogen, Kite, Sanofi, Takeda and Pfizer could cause delays or other difficulties in the development and commercialization of our product candidates, which may prevent us from completing the intended IND filings in a timely fashion and receiving any milestone, royalty payments and other benefits under the agreement. In addition, under their respective agreements, our third-party collaborators have certain rights to terminate the agreements by providing us with advance notices, therefore, the actual milestone payments that we may receive under these agreements may be substantially lower than the full amounts provided for under these agreements.

***Our collaborators licensing our ZFP technologies may decide to adopt alternative technologies or products or may be unable or unwilling to develop commercially viable products with our ZFP technologies, which would negatively impact our revenues and our strategy to develop product candidates using ZFP technologies.***

Several of our collaborations leverage our ZFP technology platform. These collaborators may elect to adopt alternative technologies in the future, which could decrease the value of our ZFP technology platform and impede the development of product candidates using the platform. Additionally, because many of our collaborators are likely to be working on more than one development project, they could choose to shift their resources to projects other than those they are working on with us. If they do so, this would delay our ability to test and develop our ZFP technology platform and would delay or terminate the development of our product candidates using the platform. Further, our collaborators may elect not to develop product candidates arising out of our collaborations or not to devote sufficient resources to the development, manufacturing, marketing or sale of these product candidates. If they terminate the collaborations with us and we wish to continue developing the product candidates, we will be required to seek the support of other collaborators or develop the products ourselves. We may not be able to identify a suitable partner or negotiate a favorable collaboration agreement, and we may not have sufficient resources and expertise internally, to allow us to continue the development of these product candidates.

***Commercialization of our technologies will depend, in part, on collaborations with other companies. If we are not able to find collaborators in the future or if our collaborators do not diligently pursue product development efforts, we may not be able to develop our technologies or product candidates, which could slow our growth and decrease the market value of our common stock.***

We do not have financial resources ourselves to fully develop, obtain regulatory approval for and commercialize our product candidates. We rely significantly on our collaborations with other biopharmaceutical companies to provide funding for our research and development efforts, including preclinical studies and clinical tests, and expect to rely significantly on such collaborations to provide funding for the lengthy regulatory approval processes required to commercialize our product candidates.

For example, we have collaborations with Novartis to develop product candidates to treat certain neurodevelopment disorders, including autism and intellectual disability; with Biogen to develop product candidates to treat tauopathies including Alzheimer's disease, alpha-synuclein related diseases including Parkinson's disease and other neurological diseases; with Kite to develop product candidates to treat cancer; with Pfizer to develop product candidates to treat hemophilia A and amyotrophic lateral sclerosis and frontotemporal lobar degeneration linked to mutations of the *C9ORF72* gene; and with Sanofi to develop product candidates to treat beta thalassemia and sickle cell disease.

If we are unable to secure additional collaborations or if our collaborators are unable or unwilling to diligently advance the development, regulatory approval and commercialization of our product candidates, our growth may slow and adversely affect our ability to generate funding for development of our technologies and product candidates. In addition, our collaborators may sublicense or abandon development programs with little advance notice, or we may have disagreements or disputes with our collaborators, which would cause associated product development to slow or cease. In addition, the business or operations of our collaborators may change significantly through restructurings, acquisitions, other strategic transactions that may negatively impact their ability to advance our programs. The evolving COVID-19 pandemic could similarly impact our ability to realize the expected benefits of our collaborations due to the impacts of the pandemic on our collaborators and their business and operations.

Under typical collaborations, we expect to receive revenue for the research and development of our product candidates based on achievement of specific milestones, as well as royalties based on a percentage of sales of any commercialized products. Achieving these milestones will depend, in part, on the efforts of our collaborators as well as our own efforts. If we or any collaboration partner fails to meet specific milestones, then the collaboration agreement may be terminated, which could



reduce our revenues. In addition, if sales of commercialized products fail to meet expectations, we could receive lower royalties than expected.

### **Risks Relating to our Intellectual Property**

*Because it is difficult, time consuming and costly to obtain, maintain and enforce patent protections for our technologies and product candidates, and because third parties may have made inventions that are similar to ours, we may not be able to secure optimal patent protections of our technologies and product candidates.*

Our commercial success may depend in part on obtaining, maintaining and enforcing patent protection for our technologies and product candidates and successfully defending any of our patents that may be challenged. Obtaining and enforcing biopharmaceutical patents is costly, time consuming and complex, and we may not be able to file and prosecute all necessary or desirable patent applications in all desired jurisdictions, or maintain, enforce and license any patents that may issue from such patent applications, at a reasonable cost or in a timely manner or at all. 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. The patent positions of biopharmaceutical companies can be highly uncertain and can involve complex legal and factual questions. No consistent policy regarding the breadth of claims allowed in biotechnology patents has emerged to date. Accordingly, we cannot predict the breadth of claims that may issue from any patent applications that we own or license, nor are we able to predict whether any third-party patents might issue with claims that are relevant to our product candidates or technologies. Even if patents do successfully issue and even if such patents cover our technologies and product candidates, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed or invalidated. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Furthermore, if third parties have made similar inventions, there are multiple ways they could impact the coverage of our own applications.

We are a party to various license agreements that grant us rights under specified patents and patent applications. We are also party to various license agreements by which we grant third parties rights under specified patents and patent applications. Our current licenses contain performance obligations. If we fail to meet those obligations, the licenses could be terminated. If we are unable to continue to license these technologies on commercially reasonable terms, or at all, we may be forced to delay or terminate aspects of our product development and research activities.

We are unable to exercise the same degree of control over intellectual property that we license from third parties as we exercise over our internally developed intellectual property. We do not control the prosecution of certain of the patent applications that we license from third parties; therefore, the patent applications may not be prosecuted as we desire or in a timely manner.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

- we or our licensors were the first to make the inventions covered by each of our pending patent applications;
- we or our licensors were the first to file patent applications for these inventions;
- the patents of others will not have an adverse effect on our ability to do business;
- others will not independently develop similar or alternative technologies or reverse engineer any of our products, processes or technologies;
- any of our pending patent applications will result in issued patents;
- any patents issued or licensed to us, our collaborators or strategic partners will provide a basis for commercially viable products or will provide us with any competitive advantages;
- any patents issued or licensed to us will not be challenged and invalidated by third parties;
- the laws in the U.S. and foreign countries will not change or be interpreted in a way that modifies our patent rights or impacts our ability to enforce or maintain our patent rights; or
- we will develop additional products, processes or technologies that are patentable.

Others have filed and in the future are likely to file patent applications that are similar to ours. We are aware that there are academic groups and other companies that are attempting to develop technology that is based on the use of zinc finger, TALE, CRISPR/Cas and other DNA-binding proteins, and that these groups and companies have filed patent applications. Several patents with claims directed to this technology have issued, although we have no current plans to use the claimed inventions. If these or other patent applications issue as patents, it is possible that the holder of any patent or patents granted on these applications may bring an infringement action against us, our collaborators, or strategic partners claiming damages and seeking to enjoin commercial activities relating to the affected products and processes. The costs of litigating the claim could be substantial regardless of outcome. Moreover, we cannot predict whether we, our collaborators, or strategic partners would

prevail in any actions. In addition, if the relevant patent claims were upheld as valid and enforceable and our products or processes were found to infringe a patent or patents, we or our collaborators may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, and we may be prevented from making, using, or selling the relevant product or process unless we or our collaborators could obtain a license or were able to design around the patent claims. We can give no assurance that such a license would be available to us or our collaborators on commercially reasonable terms, or at all, or that we would be able to successfully design around the relevant patent claims. There may be significant litigation in the genomics or cell therapy industry regarding patent and other intellectual property rights, which could subject us to costly, lengthy and distracting litigation with unpredictable results.

We rely on trade secrets to protect technology where we believe patent protection is not appropriate or obtainable. Trade secrets, however, are difficult to protect. While we require employees, academic collaborators and consultants to enter into confidentiality agreements, we may not be able to adequately protect our trade secrets or other proprietary information or enforce these confidentiality agreements.

Our collaborators, strategic partners, and scientific advisors have rights to publish data and information in which we may have rights. If we cannot maintain the confidentiality of our technology and other confidential information in connection with our collaborations and strategic partnerships, then we may not be able to receive patent protection or protect our proprietary information.

***Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time and may vary based on jurisdiction.***

Patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date or from the filing date of the corresponding international application. Various extensions may be available. However, the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product, we may be open to competition from generic medications. 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 owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

***If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be adversely affected, and our business would be harmed.***

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 our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, collaborators, partners and contractors. 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 individuals, organizations and systems, agreements or security measures have been and may in the future be breached, and we may not have adequate remedies for any breach. See also the risk factor titled, "Significant disruptions of our information technology systems or data security incidents could result in significant financial, legal, regulatory, business and reputational harm to us." In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees, consultants, advisors, collaborators, partners and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have an adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information.

Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual

property both in the United States and abroad. If we are unable to prevent material disclosure of the non-patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, we may not be able to establish or maintain a competitive advantage in our market, which could adversely affect our business, results of operations and financial condition.

***We may not be successful in obtaining or maintaining necessary rights to product components, platforms and processes for our development pipeline through acquisitions and in-licenses.***

Presently, we believe we have rights to the intellectual property, through licenses from third parties and under patents that we own, to develop our gene and cell therapy product candidates. Because our programs may involve additional product candidates, such as TX200 and potential future CAR-Treg therapies that may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights. In addition, our product candidates may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify on commercially reasonable terms, if at all. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights, including from other companies and academic institutions, that we may consider attractive. Other companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

In addition, companies that perceive us to be a competitor may be unwilling to assign or 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. If we are unable to successfully obtain rights to required third-party intellectual property rights, our business, financial condition and prospects for growth could suffer.

***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 are a party to a number of intellectual property license agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone, royalty and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license.

We may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we have done so from time to time. We may fail to obtain any of these 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 develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly. We cannot provide any assurances that third-party patents do not exist that might be enforced against our current product candidates or future products, resulting in either an injunction prohibiting our 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 many cases, patent prosecution of our in-licensed technology is controlled solely by the licensor. 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. In certain cases, we control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues and is complicated by the rapid pace of scientific discovery in our industry.

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, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have an adverse effect on our business, financial condition, results of operations and prospects. 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 the affected product candidates, which could

have an adverse effect on our business, financial conditions, results of operations and prospects. As an example, Sangamo France has exclusively licensed the right to the CAR for use in TX200 from the University of British Columbia, or UBC. Should UBC terminate this license agreement, we may have to develop or acquire the appropriate CAR which would extend our anticipated development timeline and add expense, and which could result in our failure to realize the anticipated benefits of the acquisition of Sangamo France.

***We may be involved in patent or intellectual property lawsuits or similar disputes involving patents under our control or patents of third parties claiming infringement, which lawsuits could be expensive, time-consuming and impair or prevent development and commercialization activities.***

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions, *ex parte* reexaminations, post-grant review and *inter partes* review proceedings before the U.S. PTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization, and such parties may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. For example, we are aware of certain patents held by third parties related to certain vector and vector manufacturing methods that are related to certain of our product candidates. We have not yet finalized the commercial scale manufacturing process for any of our product candidates. If our commercial scale manufacturing process utilizes these vector manufacturing methods, and if these third-party patents are valid and in force at the time of commercialization, we may need to challenge these patents, use or develop non-infringing alternatives or seek a license to these patents. In any event, if any third-party patents were held by a court of competent jurisdiction to cover our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block or hinder our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations or processes for manufacture or methods of use, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license, or until such patents expires. Moreover, because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe.

Defense of these claims, regardless of their merit, would involve substantial litigation expense, could expose proprietary information and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

Competitors may also infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid, is unenforceable, in whole or in part, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated, held unenforceable or interpreted narrowly and could put our patent applications at risk of not issuing. Moreover, if we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidate. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection could have an adverse impact on our business.

Interference or derivation proceedings provoked by third parties or brought by us or declared by the U.S. PTO may be necessary to determine the priority of inventions or other matters of inventorship with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could expose us to significant monetary damages, result in the loss of valuable intellectual property, require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation, interference, derivation or other proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

***We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.***

We employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any of our employee's former employer or other third parties. 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, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

***We may be unable to license gene transfer technologies that we may need to commercialize our ZFP technology and potential products, if approved.***

In order to regulate or modify a gene in a cell, the ZFP must be efficiently delivered to the cell. We have licensed certain gene transfer technologies for our ZFP in research, including AAV and mRNA technology, and we are evaluating these systems and other technologies that may need to be used in the delivery of ZFP into cells for *in vitro* and *in vivo* applications. We have not developed our own gene transfer technologies, and we rely on our ability to enter into license agreements to provide us with rights to the necessary gene transfer technology. Our approach has been to license appropriate technology as required. For example, we are aware of certain patents held by a third party related to certain vector manufacturing methods that are currently being used in certain of our product candidates. We have not yet finalized the commercial scale manufacturing process for any of our product candidates. If our commercial scale manufacturing process utilizes these vector manufacturing methods, and if these third-party patents are in force at the time of commercialization, we may need to use or develop a non-infringing manufacturing method or seek a license to these patents. However, we may not be able to license the gene transfer technologies on reasonable terms, if at all, required to develop and commercialize our product candidates. The inability to obtain a license to use gene transfer technologies with entities that own such technology on reasonable commercial terms, if at all, could delay or prevent the preclinical evaluation, drug development collaborations, clinical testing and/or commercialization of our therapeutic product candidates.

## **Risks Relating to our Business Operations**

***Significant disruptions of our information technology systems or data security incidents could result in significant financial, legal, regulatory, business and reputational harm to us.***

We are increasingly dependent on information technology systems and infrastructure to operate our business, which are large and complex. In the ordinary course of our business, we collect, store, process and transmit large amounts of sensitive information, including intellectual property, proprietary business information, personal data (including personal health information) and other confidential information. It is critical that we do so in a secure manner to maintain the confidentiality, integrity and availability of such sensitive information. We have also outsourced elements of our operations (including elements of our information technology infrastructure) to third parties, and as a result, we manage a number of third-party vendors who may or could have access to our computer networks or our confidential information. Many of those third parties in turn subcontract or outsource some of their responsibilities to third parties. While all information technology operations are inherently vulnerable to inadvertent or intentional security breaches, incidents, attacks and exposures, the size, complexity, accessibility and distributed nature of our information technology systems, and the large amounts of sensitive information stored on those systems, make such systems potentially vulnerable to unintentional or malicious, internal and external attacks

on our technology environment. Attacks of this nature are increasing in their frequency, levels of persistence, sophistication and intensity. In addition, the effects of the COVID-19 pandemic have intensified our dependence on information technology systems as many of our critical business activities are currently being conducted remotely and our increased reliance on personnel working from home could increase our cybersecurity risk.

Significant disruptions of our, our third-party vendors' and/or business partners' information technology systems or other similar data security incidents could adversely affect our business operations and/or result in the loss, misappropriation, and/or unauthorized access, use or disclosure of, or the prevention of access to, sensitive information, which could result in financial and reputational harm to us. For example, in April 2018, we announced a data security incident involving the compromise of a senior executive's company email account. Our investigation of the incident did not reveal any evidence that our systems were otherwise compromised in connection with the incident or that personal data about patients or other individuals besides the executive were accessed or disclosed. However, proprietary, confidential and other sensitive information of ours and that of other entities was accessed and may have been compromised as a result of the incident. Unforeseen developments related to this incident could occur, which could have a further adverse impact on us. Any litigation or regulatory review or investigation arising from this incident could result in significant legal exposure to us. In addition, information technology system disruptions, whether from attacks on our technology environment or from computer viruses, natural disasters, terrorism, war and telecommunication and electrical failures, could result in a material disruption of our facility, development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

While we are aware of the company email incident described above, there is no way of knowing with certainty whether we have experienced any other data security incidents that have not been discovered. While we have no reason to believe this to be the case, attackers have become very sophisticated in the way they conceal access to systems, and many companies that have been attacked are not aware that they have been attacked. Any delay in the discovery of an attack may result in increased expense and may harm our reputation. Any event, including the company email incident described above, that leads to unauthorized access, use or disclosure of personal data could, among other consequences, disrupt our business, harm our reputation, compel us to comply with applicable federal and/or state data breach notification laws and foreign law equivalents. In addition, failure to maintain effective internal accounting controls related to security breaches and cybersecurity in general could impact our ability to produce timely and accurate financial statements and subject us to regulatory scrutiny. While we have implemented security measures intended to protect our information technology systems and infrastructure, there can be no assurance that such measures will successfully prevent service interruptions or further security incidents.

***We have business operations in France and the United Kingdom, which exposes us to additional costs and risks.***

Our business operations in France and the United Kingdom subject us to certain additional costs and risks associated with doing business outside the United States, including:

- the increased complexity and costs inherent in managing international operations in geographically disparate locations;
- challenges of complying with diverse regulatory, financial and legal requirements, which are subject to change at any time;
- potentially adverse tax consequences, including changes in applicable tax laws and regulations;
- potentially costly trade laws, tariffs, export quotas, custom duties or other trade restrictions, and any changes to them;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- liabilities for activities of, or related to, our international operations;
- challenges inherent in efficiently managing employees in diverse geographies, including the need to adapt systems, policies, benefits and compliance programs to differing labor and other regulations;
- natural disasters, political and economic instability, including wars, terrorism and political unrest, outbreak of health epidemics, including the evolving COVID-19 pandemic, and the resulting global economic and social impacts;
- workforce uncertainty in countries where labor unrest is more common than in the United States; and
- differing laws and regulations relating to data security and the unauthorized use of, or access to, commercial and personal information.

In addition, our international operations in France and the United Kingdom expose us to fluctuations in currency exchange rates between the Euro and the U.S. dollar and between the Pound Sterling and the U.S. dollar. Given the volatility of currency exchange rates, there is no assurance that we will be able to effectively manage currency transaction and/or conversion risks. To date, we have not entered into derivative instruments to offset the impact of foreign exchange fluctuations, which fluctuations could have an adverse effect on our financial condition and results of operations. In any event, difficulties resulting

from these and other risks related to our operations outside of the United States could expose us to increased expenses, impair our development efforts, adversely affect our financial condition and results of operations and harm our competitive position.

***We are in the process of growing the size of our organization globally, and we may experience difficulties in hiring, integrating and retaining qualified skilled employees.***

We need to grow the size of our organization in order to support our research, product development, manufacturing and regulatory efforts, and such growth is critical to our success. We may not be able to hire, integrate and retain a sufficient number of qualified employees with the appropriate levels of experience and skills to accomplish our growth objectives. There currently is a shortage of skilled individuals with substantial experience discovering, developing and manufacturing genomic medicines, which is likely to continue. As a result, competition for these individuals is intense and the turnover rate can be high. We may not be able to hire, integrate and retain employees with these skills on acceptable terms given the competition among numerous biopharmaceutical companies and academic institutions for individuals with these skills. In addition, any negative or unexpected results in our preclinical or clinical trials or applications for marketing approval would make it more challenging to hire and retain qualified skilled employees. Moreover, the evolving COVID-19 pandemic has further challenged our ability to hire skilled employees. If we do not achieve our growth objectives, the progress of our research, development, manufacturing and regulatory efforts will slow down, which will adversely impact our business, financial condition, results of operations and prospects.

We are dependent on certain key members of our executive team and certain of our scientific, clinical development and manufacturing personnel, the loss of whose services may impede the progress of our research, development, manufacturing and regulatory efforts. While we have entered into employment agreements with each of our executive officers, any of them could leave our employment at any time, as all of our employees are “at will” employees. We do not have “key person” insurance on any of our employees.

***We may not be successful in our efforts to discover, license or acquire new potential product candidates and may fail to capitalize on product candidates with a greater commercial opportunity or for which there is a greater likelihood of success.***

If our existing product candidates do not receive regulatory approval or are not successfully commercialized, then the success of our business will depend on our ability to continue to expand our product pipeline through discovery, in-licensing or acquisitions. We may be unable to do so. If we do identify potential product candidates for licensing or acquisition, we may be unable to reach acceptable terms with the licensors or sellers. Further, there may be risks and liabilities associated with the product candidates which our due diligence efforts fail to discover, that are not disclosed to us, that we inadequately assess, or that we are unable to manage effectively. Additionally, we may not realize the anticipated benefits of such licenses or acquisitions for a variety of reasons, including the possibility that the product candidates prove not to be safe or effective in clinical trials, that we are unable to successfully integrate the product candidate into our operations, or that the anticipated benefits will not otherwise be realized within the expected timeframe.

Additionally, because we have limited resources, we may forego or delay pursuit of opportunities with certain product candidates or indications that later prove to have greater commercial potential. Our spending on current and future research and development programs may not yield any commercially viable products. 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 collaboration, 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 collaboration arrangement.

#### **Risks Relating to our Common Stock and Corporate Organization**

***Our stock price has been volatile and will likely continue to be volatile, which could result in substantial losses for investors, and could be influenced by public perception of genomic medicines and the biotechnology sector.***

Our stock price has been volatile and may continue to be volatile, which could cause stockholders to incur substantial losses. An active public market for our common stock may not be sustained, and the market price of our common stock may continue to be volatile. The market price of our common stock has fluctuated significantly in response to various factors, some of which are beyond our control, including but not limited to the following:

- announcements by us or collaborators providing updates on the progress or development status of product candidates or data from clinical trials;
- initiation or termination of clinical trials;
- changes in market valuations of similar companies;
- overall market and economic conditions, including the equity markets for emerging biotechnology companies;
- deviations in our results of operations from the guidance given by us;
- announcements by us or our competitors of new or enhanced products or technologies or significant contracts, acquisitions, strategic relationships, joint ventures or capital commitments;
- announcement of changes in business and operations by our collaborators, or changes to our existing collaboration agreements;
- changes in public opinions of genomic medicines;
- regulatory developments, including increased regulatory scrutiny of genomic medicines;
- changes, by one or more of our securities analysts, in recommendations, ratings or coverage of our stock;
- additions or departures of key personnel; and
- sales of our common stock or other securities by us, officers or directors, liquidation of institutional funds that comprised large holdings of our stock and decreases in our cash balances.

In addition, the stock markets have experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many companies, including very recently in connection with the evolving COVID-19 pandemic, which has resulted in decreased stock prices for many companies notwithstanding the lack of a fundamental change in their underlying business models or prospects. These fluctuations have often been unrelated or disproportionate to the operating performance of those companies. Broad market and industry factors, including potentially worsening economic conditions and other adverse effects or developments relating to the evolving COVID-19 pandemic, and political, regulatory and other market conditions, may negatively affect the market price of shares of our common stock, regardless of our actual operating performance.

***Actual or potential sales of significant amounts of shares of our common stock into the market could cause the market price of our common stock to fall or prevent it from increasing for numerous reasons.***

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 generally 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 the issuance of such shares has already been registered under the Securities Act and are held by non-affiliates of ours. While Biogen agreed not to sell any of the shares that we issued to Biogen in April 2020 until the first anniversary of the effectiveness of the Biogen collaboration, and to limit resales through the second anniversary, such restrictions are only temporary. Further, we also agreed, subject to certain limitations, to register for resale under the Securities Act any of the shares we issued to Biogen. We have also filed registration statements registering all shares of common stock that we may issue under our equity compensation plans. Such shares can be freely sold in the public market upon issuance, subject to volume limitations and black-out periods applicable to affiliates. Additionally, we are party to a sales agreement with Jefferies LLC which permits us from time to time at our discretion to sell up to \$150.0 million of shares of our common stock in the public markets at prevailing market prices. As of February 19, 2021, we have sold 1,034,762 shares of our common stock under the sales agreement for net proceeds of approximately \$15.7 million.

In addition, in accordance with the guidelines specified under Rule 10b5-1 of the Securities Exchange Act of 1934, as amended, or the Exchange Act, and our policies regarding stock transactions, certain of our employees, executive officers and directors have adopted, and may continue to adopt, stock trading plans pursuant to which they have arranged to sell shares of our common stock from time to time in the future. Generally, sales under such plans by our executive officers and directors require public filings. Our employees, executive officers, directors and affiliated stockholders also may buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information. Actual or potential sales of our common stock by such persons could be viewed negatively by other investors and could cause the price of our common stock to fall or prevent it from increasing.

***We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.***

We have never declared or paid any cash dividends on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock.



***Anti-takeover provisions in our certificate of incorporation, Delaware law and our bylaws could make an acquisition of our company more difficult and could prevent attempts by our stockholders to remove or replace current management.***

Anti-takeover provisions of Delaware law and in our certificate of incorporation and our bylaws may discourage, delay or prevent a change in control of our company, even if a change in control would be beneficial to our stockholders. In addition, 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. In particular, under our certificate of incorporation our board of directors may issue up to 5,000,000 shares of preferred stock with rights and privileges that might be senior to our common stock, without the consent of the holders of the common stock. Moreover, without any further vote or action on the part of the stockholders, the board of directors would have the authority to determine the price, rights, preferences, privileges, and restrictions of the preferred stock. This preferred stock, if it is ever issued, may have preference over, and harm the rights of, the holders of common stock. Although the issuance of this preferred stock would provide us with flexibility in connection with possible acquisitions and other corporate purposes, this issuance may make it more difficult for a third party to acquire a majority of our outstanding voting stock.

Similarly, our authorized but unissued common stock is available for future issuance without stockholder approval. Our certificate of incorporation further provides that stockholders may not take action by written consent.

In addition, our amended and restated bylaws:

- establish advance notice requirements for nominations for election to the board of directors or proposing matters that can be acted upon at stockholders' meetings; and
- prohibit stockholders from calling a special meeting of stockholders.

We are also subject to Section 203 of the General Corporation Law of the State of Delaware, which provides, subject to certain exceptions, that if a person acquires 15% of our voting stock, the person is an "interested stockholder" and may not engage in "business combinations" with us for a period of three years from the time the person acquired 15% or more of our voting stock. The application of Section 203 may, in some circumstances, deter or prevent a change in control of our company even when such change may be beneficial to our stockholders.

***Our amended and restated bylaws designate exclusive forums for the adjudication of certain disputes, which could limit our stockholders' ability to bring claims in a judicial forum it finds favorable for disputes with us or our directors, officers, or employees.***

Our amended and restated bylaws provide that a state or federal court located within the State of Delaware is the sole and exclusive forum for:

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim of breach of a fiduciary duty owed by any director, officer or other employee or stockholder of Sangamo to us or our stockholders;
- any action asserting a claim arising pursuant to any provision of the General Corporation Law of the State of Delaware, our charter or our bylaws, as to which the General Corporation Law of the State of Delaware confers jurisdiction on the Court of Chancery of the State of Delaware; and
- any action asserting a claim governed by the internal affairs doctrine.

Our amended and restated bylaws further provide that a federal district court of the United State is the sole and exclusive forum for any complaint asserting a cause of action arising under the Securities Act of 1933, as amended. These provisions further provide that any person or entity that acquires any interest in shares of our capital stock will be deemed to have notice of and consented to these provisions.

These provisions 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 lawsuits against us and our directors, officers, and other employees. If a court were to find any of these provisions to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving the dispute in other jurisdictions, which could seriously harm our business.

**ITEM 1B – UNRESOLVED STAFF COMMENTS**

None.

**ITEM 2 – PROPERTIES**

Our corporate headquarters occupies approximately 87,700 square feet of office and research and development laboratory facilities in Brisbane, California, pursuant to a lease that expires in May 2029. We also lease approximately 54,200 square feet of research and office space in Richmond, California, subject to leases that expire in August 2026. We also lease approximately 20,800 square feet of office, research and development space in Valbonne, France, subject to leases that expire beginning in June 2025 through March 2028. We believe that our facilities are currently adequate to meet our needs. As we continue to expand our operations, we may need to lease or purchase additional facilities.

**ITEM 3 – LEGAL PROCEEDINGS**

We are not a party to any material pending legal proceeding. From time to time, we may be involved in legal proceedings arising in the ordinary course of business.

**ITEM 4 – MINE SAFETY DISCLOSURES**

Not Applicable.

**PART II**

**ITEM 5 – MARKET FOR THE REGISTRANT’S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES**

**Market Information**

Our common stock trades on the Nasdaq Global Select Market under the symbol “SGMO.”

**Holders**

As of February 19, 2021, there were 53 holders of record of our common stock. This number does not include “street name,” or beneficial holders, whose shares are held of record by banks, brokers, financial institutions and other nominees.

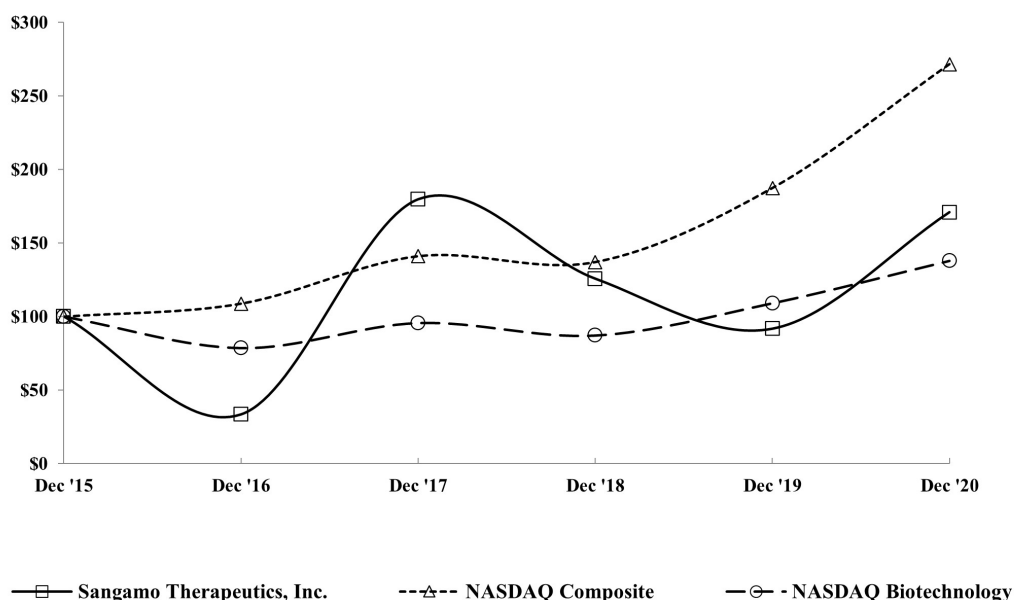
**Dividends**

We have not paid dividends on our common stock, and currently do not plan to pay any cash dividends in the foreseeable future.

**Stock Performance Graph**

**COMPARISON OF 5 YEAR CUMULATIVE TOTAL RETURN\***

Among Sangamo Therapeutics, Inc., the NASDAQ Composite Index and the NASDAQ Biotechnology Index



\*\$100 invested on December 31, 2015 in stock or index, including reinvestment of dividends.  
Fiscal year ending December 31.

*The above Stock Performance Graph and related information shall not be deemed “soliciting material” or to be “filed” with the SEC nor shall such information be incorporated by reference into any future filing under the Securities Act or the Exchange Act, each as amended, except to the extent that the Company specifically incorporates it by reference into such filing.*

**ITEM 6 – SELECTED FINANCIAL DATA**

The following Selected Financial Data should be read in conjunction with “Item 7 – Management’s Discussion and Analysis of Financial Condition and Results of Operations” and “Item 8 – Financial Statements and Supplementary Data” included elsewhere in this Annual Report on Form 10-K. Historical results are not necessarily indicative of future results.

	Year Ended December 31,				
	2020	2019	2018	2017	2016
	(In thousands, except per share data)				
<b>Consolidated Statement of Operations Data:</b>					
Revenues	\$ 118,192	\$ 102,428	\$ 84,452	\$ 36,567	\$ 19,389
Operating expenses:					
Research and development	180,647	145,922	114,866	65,728	65,618
General and administrative	67,097	61,686	46,736	27,200	26,330
Total operating expenses	247,744	207,608	161,602	92,928	91,948
Loss from operations	(129,552)	(105,180)	(77,150)	(56,361)	(72,559)
Interest and other income, net	8,775	9,761	8,261	1,793	887
Loss before income taxes	(120,777)	(95,419)	(68,889)	(54,568)	(71,672)
Income tax expense (benefit)	345	—	—	—	(14)
Net loss	(121,122)	(95,419)	(68,889)	(54,568)	(71,658)
Net loss attributable to non-controlling interest	(126)	(233)	(555)	—	—
Net loss attributable to Sangamo Therapeutics, Inc. stockholders	\$ (120,996)	\$ (95,186)	\$ (68,334)	\$ (54,568)	\$ (71,658)
Basic and diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders	\$ (0.90)	\$ (0.85)	\$ (0.70)	\$ (0.70)	\$ (1.02)
Shares used in computing basic and diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders	134,449	112,114	96,941	78,084	70,553

	As of December 31,				
	2020	2019	2018	2017	2016
	(In thousands)				
<b>Consolidated Balance Sheet Data:</b>					
Cash, cash equivalents, marketable securities, and interest receivable	\$ 692,988	\$ 384,988	\$ 400,508	\$ 244,560	\$ 142,759
Working capital	\$ 516,121	\$ 335,601	\$ 332,010	\$ 203,538	\$ 136,289
Total assets	\$ 938,550	\$ 637,516	\$ 590,395	\$ 286,741	\$ 157,891
Long-term portion of lease liabilities <sup>(1)</sup>	\$ 38,396	\$ 41,192	\$ 27,689	\$ 24,738	\$ 3,945
Accumulated deficit	\$ (777,981)	\$ (656,985)	\$ (562,696)	\$ (495,479)	\$ (440,911)
Total stockholders’ equity	\$ 497,366	\$ 432,739	\$ 367,257	\$ 187,900	\$ 136,195

(1) In 2019, we adopted Accounting Standards Update No. 2016-02 (Topic 842) “Leases,” which requires lessees to recognize right-of-use assets and lease liabilities for operating leases with a lease term greater than one year. We adopted Topic 842 using the modified retrospective method. As such, results for reporting periods beginning after January 1, 2019 are presented under Topic 842, while prior period amounts are not adjusted and continue to be reported in accordance with our historical accounting under Topic 840 “Leases.”

(2) Sangamo France was acquired in October 2018 and the results of operations have been included in the selected consolidated financial data since the date of acquisition (see Note 5 – *Acquisition of Sangamo France* in our Consolidated Financial Statements).

## ITEM 7 – MANAGEMENT’S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The discussion in “Management’s Discussion and Analysis of Financial Condition and Results of Operations” contains trend analysis, estimates and other forward-looking statements within the meaning of Section 27A of the Securities Act, as amended, and Section 21E of the Exchange Act, as amended. These forward-looking statements include, without limitation, statements containing the words “anticipates,” “believes,” “continues,” “could,” “estimates,” “expects,” “intends,” “may,” “plans,” “seeks,” “should,” “will,” and other words of similar import or the negative of those terms or expressions. Such forward-looking statements are subject to known and unknown risks, uncertainties, estimates and other factors that may cause our actual results, performance or achievements, or industry results, to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. Actual results could differ materially from those set forth in such forward-looking statements as a result of, but not limited to, the “Risk Factors” described in Part I, Item 1A of this Annual Report on Form 10-K. You should read the following discussion and analysis along with the “Selected Financial Data” and the Consolidated Financial Statements and notes attached to those statements included elsewhere in this report.

In addition, the section of this “Management’s Discussion and Analysis of Financial Condition and Results of Operations” generally discusses 2020 and 2019 items and year-to-year comparisons between 2020 and 2019. Discussions of 2018 items and year-to-year comparisons between 2019 and 2018 are not included in this Annual Report on Form 10-K and can be found in “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in Part II, Item 7 of our [Annual Report on Form 10-K for the fiscal year ended December 31, 2019](#), filed with the SEC on February 28, 2020.

### Overview

We are a clinical-stage genomic medicine company committed to translating ground-breaking science into medicines that transform the lives of patients with serious diseases. We plan to deliver on this mission through (i) development of our clinical and preclinical product candidates, (ii) our novel science and (iii) our in-house manufacturing capabilities.

Our current clinical-stage product candidates are:

- Giroctocogene fitelparvovec, also known as SB-525, our lead product candidate, is a gene therapy for the treatment of hemophilia A and is currently being evaluated in the registrational Phase 3 AFFINE (efficacy and safety Factor VIII gene therapy) clinical trial. We are developing giroctocogene fitelparvovec with our collaborator Pfizer Inc., or Pfizer;
- ST-920, our wholly-owned gene therapy product candidate for the treatment of Fabry disease, is currently being evaluated in our Phase 1/2 STAAR clinical study;
- BIVV003, our cell therapy product candidate for the treatment of sickle cell disease, or SCD, is currently being evaluated in our Phase 1/2 PRECIZN-1 clinical study. We are developing BIVV003 with our collaborator Sanofi S.A., or Sanofi; and
- ST-400, our cell therapy product candidate for the treatment of transfusion dependent beta thalassemia, is currently being evaluated in our Phase 1/2 Thales clinical study. We are developing ST-400 with our collaborator Sanofi.

In addition, we expect to initiate clinical studies for two additional product candidates in 2021:

- TX200, our wholly-owned Chimeric Antigen Receptor, or CAR, engineered regulatory T cell, or CAR-Treg, cell therapy product candidate for the treatment of HLA-A2 mismatched kidney transplant rejection; and
- KITE-037, our allogeneic anti-CD19 CAR-T cell therapy product candidate for the treatment of cancer. We are developing KITE-037 with our collaborator Kite Pharma Inc., or Kite, a wholly-owned subsidiary of Gilead Sciences, Inc.

Moreover, we are focusing our preclinical development in emerging areas for us including CAR-Treg cell therapies for autoimmune disorders and genome engineering for neurological diseases. Indications for our other preclinical programs include neurodevelopmental disorders, cancer, inflammatory bowel disease, or IBD, tauopathies such as Alzheimer’s and neurodegenerative diseases such as amyotrophic lateral sclerosis, or ALS, multiple sclerosis, or MS, and Huntington’s disease, some of which we are developing with our collaborators Biogen MA, Inc. and Biogen International GmbH, which we refer to together as Biogen, Kite, Novartis Institutes for BioMedical Research, Inc., or Novartis, Pfizer and Takeda Pharmaceutical Company Limited, or Takeda.

Our multiple collaborations with biopharmaceutical companies bring us important financial and strategic benefits and reinforce the potential of our research and development efforts and our zinc finger protein, or ZFP, technology platform. They leverage our collaborators' therapeutic and clinical expertise and commercial resources with the goal to bring our medicines more rapidly to patients. We believe these collaborations reflect of the value of our ZFP technology platform and will potentially expand the addressable markets of our product candidates. To date, we have received approximately \$815.0 million in upfront licensing fees, milestone payments, and proceeds from sale of our common stock to collaborators and have the right to earn up to \$7.0 billion in future milestone payments from our collaborations, in addition to potential product royalties.

For additional information regarding our business, see "Business" in Part I, Item 1 of this Annual Report on Form 10-K.

### Recent Business Highlights

- In December 2020, we and Pfizer jointly announced updated follow-up data from the Phase 1/2 Alta study of giroctocogene fitelparvovec, an investigational gene therapy for the treatment of severe hemophilia A.
- In October 2020, we and Pfizer jointly announced that we dosed the first participant in the registrational Phase 3 AFFINE clinical trial of giroctocogene fitelparvovec.
- In August and September 2020, we dosed the first two patients in our Phase 1/2 STAAR study evaluating our ST-920 gene therapy product candidate to treat Fabry disease, a rare inherited metabolic disease. In February 2021, we dosed the first patient in the second cohort.
- In July 2020, we entered into a collaboration agreement with Novartis to develop and commercialize gene regulation therapies to treat three neurodevelopmental targets, including genes linked to autism spectrum disorder and intellectual disability.
- In February 2020, we entered into a collaboration agreement with Biogen to develop gene regulation therapies for Alzheimer's, Parkinson's, neuromuscular and other neurological diseases.
- In 2020, we brought in-house AAV manufacturing capabilities online in our Brisbane, California headquarters.

### Estimated Impacts of Evolving COVID-19 Pandemic

In March 2020, the World Health Organization declared the novel coronavirus disease, or COVID-19, outbreak a global pandemic, and since such time, actions taken around the world to help mitigate the spread of COVID-19 have included varying restrictions on travel, quarantines in certain areas, and forced closures for certain types of public places and businesses, including in the three countries where we have most of our day-to-day operations, the United States, France and the United Kingdom. Our business has been directly impacted by pandemic restrictions aimed at reducing the spread of the disease, including multiple California executive orders, several semi-coordinated San Francisco Bay Area orders, several other state and additional local orders across the country and similar orders outside the United States, which, among other things, direct individuals to shelter at their places of residence, direct businesses and governmental agencies to cease non-essential operations at physical locations, prohibit certain non-essential gatherings, and order cessation of non-essential travel.

To comply with these orders, we implemented an operating plan to continue business operations during the ongoing COVID-19 pandemic, including enhanced workplace safety protocols and modified working schedules in our laboratories. Employees who are able to fulfill their job duties working from home are required to work from home, and have been doing so since March 2020. These protocols and modifications have slowed our productivity and disrupted our business to a moderate degree and are likely to continue doing so through 2021. For example, we have experienced periodic short-term disruptions to our onsite laboratory and manufacturing operations while addressing positive cases of COVID-19 by onsite workers, and our laboratory and manufacturing operations could experience longer term disruptions in the future in the event of a significant outbreak of COVID-19 among our onsite workers. Moreover, from time to time, we have been required to reorganize and prioritize our research resources to mitigate moderate COVID-19 impacts arising from travel restrictions, laboratory density restrictions and laboratory supply constraints. If our research programs encounter longer-term disruptions, it could impact our ability to support our biopharmaceutical partners as contemplated in our collaboration agreements and could result in adjustments to our research timelines, although we do not believe that the short-term disruptions to date have resulted in any such impacts.

Additionally, our Phase 1/2 STAAR clinical study evaluating ST-920, our wholly-owned gene therapy product candidate for the treatment of Fabry disease, has experienced delays in its timeline due to COVID-19 impacts and the diversion of healthcare resources to fight the pandemic. For example, the clinical trial sites in the UK for this study have not been able to open due to the significant prevalence of COVID-19 in the UK. Additionally, we have experienced delays in recruiting, enrolling and dosing patients for this study at our US trial sites, due in some part to the understandable hesitation of patients to travel by plane to trial sites not within driving distance and to enter medical facilities during the pandemic and in other part to

trial sites prioritizing COVID-19 clinical care over research activities such as the STAAR study. Moreover, we have experienced some short-term delays in sourcing the necessary raw materials to manufacture supplies for the STAAR study due to COVID-19 impacts. We estimate that these challenges have set back our STAAR study timelines three to six months. While we currently still expect to share initial clinical study data by the end of 2021, this timeline could be revised if COVID-19 impacts to our enrollment and dosing of patients and to our sourcing of raw materials for this study intensify because of vaccination delays, new COVID-19 variants or unexpected events.

In addition, our STEADFAST clinical study evaluating TX200, our wholly-owned CAR-Treg cell therapy product candidate for the treatment of kidney transplant rejection, has experienced delays in its commencement timeline due to COVID-19 impacts related to manufacturing and technology transfer challenges with our CMOs. We estimate that these challenges have set back our commencement timeline by approximately three months. While we currently still expect to initiate this clinical study by the end of 2021, this timeline could be revised if COVID-19 impacts result in additional delays.

With respect to our partnered programs, the timelines for the studies and trials managed by our collaborators are also subject to potential delay in the future if these studies and trials experience similar challenges that we have experienced in our STAAR and STEADFAST studies.

Going forward, we will continue to monitor the impact of COVID-19 on our operations, research commitments and clinical trials and those of our collaborators, clinical trial sites and CMOs. The magnitude of these impacts will depend, in part, on the length and severity of the COVID-19 pandemic and related government orders and restrictions, and how the pandemic limits the ability of us and our business partners to operate business in the ordinary course. Disruptions to these operations, and possibly more severe disruptions in the future that could arise due to the extension of government orders or new government orders applicable in the places we operate or our industry generally or to us and our facilities specifically, could impede our ability to conduct research in a timely manner, comply with our research obligations to our collaborators and advance the development of our therapeutic programs. These delays and disruptions could result in adverse material impacts to our business, operating results and financial condition.

We do not anticipate any material negative impact on our financial condition in 2021 as a result of the COVID-19 pandemic. We believe we are well positioned financially in the near term to execute on our wholly-owned and partnered research and clinical programs. We ended 2020 with \$692.0 million in cash, cash equivalents and marketable securities and have raised an additional \$15.7 million in cash through February 19, 2021 through sales of our common stock under our at-the-market offering program. Although we believe we are well capitalized currently, the effects of the evolving pandemic could result in disruption of global financial markets, impairing our ability to access capital, which could in the future negatively affect our liquidity. We do not currently anticipate any material impairments to the valuation of the financial assets or goodwill on our balance sheet as a result of COVID-19. We do not believe that the remote workplace arrangements we have implemented for our office-based employees have affected our financial reporting or control systems.

The extent to which the COVID-19 pandemic will impact our business, operations and financial condition, either directly or indirectly, will depend on future developments that remain highly uncertain at the present time. These developments include the ultimate duration and severity of the pandemic, the impacts of new COVID-19 variants, travel restrictions, quarantines and social distancing requirements in the United States, France, United Kingdom and other countries, business closures or business disruptions and the effectiveness and timeliness of actions taken in the United States, France, United Kingdom and other countries to contain and treat the disease, including the effectiveness and timing of vaccination programs. Although certain government orders and restrictions have eased, and phased re-openings are underway, it is not certain when such restrictions and orders will be fully lifted, and recent resurgences in number and rates of infections and the surge of new variants of the virus may result in the return of prior orders and restrictions or new quarantine and shelter-in-place orders or other restrictions. As our understanding of events evolves and additional information becomes available, we may materially change our guidance relating to our revenues, expenses and timelines for manufacturing, clinical trials and research and development.

See also the section titled “Risk Factors” included in Part I, Item 1A of this Annual Report on Form 10-K for additional information on risks and uncertainties related to the evolving COVID-19 pandemic.

### **Certain Components of Results of Operations**

Our revenues have consisted primarily of revenues from upfront licensing fees, reimbursements for research services, milestones achievements and research grant funding. We expect revenues to continue to fluctuate from period to period and there can be no assurance that new collaborations or partner reimbursements will continue beyond their initial terms or that we are able to meet the milestones specified in these agreements.

We have incurred net losses since inception and expect to incur losses for at least the next several years as we continue our research and development activities. To date, we have funded our operations primarily through the issuance of equity securities and revenues from collaborations and research grants.

We expect to continue to devote substantial resources to research and development in the future and expect research and development expenses to increase in the next several years if we are successful in advancing our product candidates from research stage through clinical trials. Pursuant to the terms of our agreements with Biogen, Kite, Novartis, Pfizer and Sanofi, certain expenses related to research and development activities will be reimbursed to us. The reimbursement funds to be received from Biogen, Kite, Novartis, Pfizer and Sanofi will be recognized as revenue as the related costs are incurred and collection is reasonably assured.

General and administrative expenses consist primarily of salaries and personnel related expenses for executive, finance and administrative personnel, stock-based compensation expenses, professional fees, allocated facilities expenses, patent prosecution expenses and other general corporate expenses. As we continue to advance our product candidates into and through the clinic, we expect the growth of our business to require increased general and administrative expenses.

### **Critical Accounting Policies and Estimates**

The accompanying discussion and analysis of our financial condition and results of operations are based upon our Consolidated Financial Statements and the related disclosures, which have been prepared in accordance with accounting principles generally accepted in the United States. The preparation of these Consolidated Financial Statements requires us to make estimates, assumptions and judgments that affect the reported amounts in our Consolidated Financial Statements and accompanying notes. We base our estimates on historical experience and on various other assumptions that we believe to be 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. Actual results may differ from these estimates under different assumptions or conditions. We believe the following policies to be the most critical to an understanding of our financial condition and results of operations because they require us to make estimates, assumptions and judgments about matters that are inherently uncertain.

We believe our critical accounting policies and estimates relating to revenue recognition, including the estimated fair value of common shares issued as part of the transaction price and valuation of long-lived assets including goodwill and intangible assets are the more significant estimates and assumptions used in the preparation of our Consolidated Financial Statements.

For a complete description of our significant accounting policies, see Note 1 – *Organization and Summary of Significant Accounting Policies* in the accompanying notes to the Consolidated Financial Statements included in Part II, Item 8, "Financial Statements and Supplementary Data" of this Annual Report on Form 10-K.

### **Revenue Recognition**

We recognize revenues from research services generally as services are provided while revenues from non-refundable upfront fees are recognized over time either by measuring progress towards satisfaction of the relevant performance obligation using the input method (i.e., cumulative actual costs incurred relative to total estimated costs) or on a straight-line basis when a performance obligation is expected to be satisfied evenly over a period of time (when there is a stand-ready obligation).

The estimation of measure of progress is complex, involves significant judgment, and is affected by our estimates of the total costs required to complete the performance obligations including the total internal personnel costs and external costs to be incurred as well as, in certain cases, the estimated stand-ready obligation period. Changes in these estimates can have a material effect on our revenue recognition.

For a further description of our revenue recognition, see Note 4 – *Major Customers, Partnerships and Strategic Alliances* in the accompanying notes to the Consolidated Financial Statements included in Part II, Item 8, "Financial Statements and Supplementary Data" of this Annual Report on Form 10-K.

### **Valuation of Long-lived Assets including Goodwill and Intangible Assets**

We allocate the fair value of purchase consideration in a business combination to the tangible assets acquired, liabilities assumed, and intangible assets acquired based on their estimated fair values. Any excess of purchase consideration over the fair value of net assets acquired is recorded as goodwill. Such valuations require management to make significant estimates and assumptions, especially with respect to intangible assets. Significant estimates in valuing certain intangible assets include, but are not limited to, future expected cash flows from acquired in-process research and development, or IPR&D, projects. Management's estimates of fair value are based upon assumptions believed to be reasonable, but which are inherently uncertain and unpredictable and, as a result, actual results may differ from estimates. Allocation of purchase consideration to



identifiable assets and liabilities affects our amortization expense, as acquired finite-lived intangible assets are amortized over the useful lives, whereas any indefinite lived intangible assets, including goodwill, are not amortized. During the measurement period, which is not to exceed one year from the acquisition date, we may record adjustments to the assets acquired and liabilities assumed, with the corresponding offset to goodwill. Upon the conclusion of the measurement period, any subsequent adjustments are recorded to earnings.

We review goodwill and indefinite-lived intangible assets for impairment at least annually or more frequently if events or changes in circumstances would more likely than not reduce the fair value these assets below their carrying values. As of December 31, 2020, no impairment of goodwill or indefinite-lived intangible assets was identified.

Long-lived assets, including property and equipment and finite-lived intangible assets, are reviewed for possible impairment whenever events or circumstances indicate that the carrying amount of such assets may not be recoverable. The evaluation is performed at the lowest level for which identifiable cash flows are largely independent of the cash flows of other assets and liabilities. Recoverability of these assets is measured by a comparison of the carrying amounts to the future undiscounted cash flows the assets are expected to generate from the use and eventual disposition. If such review indicates that the carrying amount of property and equipment and intangible assets is not recoverable, the carrying amount of such assets is reduced to fair value. We have not recorded any significant impairment charges during the years presented.

## Results of Operations

### Years Ended December 31, 2020, 2019 and 2018

#### Revenues

	Year Ended December 31,							
	(In thousands, except percentage values)							
	2020	2019	Change	%	2019	2018	Change	%
Revenues	\$ 118,192	\$ 102,428	\$ 15,764	15 %	\$ 102,428	\$ 84,452	\$ 17,976	21 %

Total revenues consisted of revenues from collaboration agreements and research grants. We anticipate revenues over the next several years will be derived primarily from our collaboration agreements with Biogen, Kite, Novartis, Pfizer and Sanofi as we continue to recognize upfront and milestone payments received under such agreements over time.

The increase of \$15.8 million in revenues in 2020 compared to 2019 was primarily due to the recognition of upfront license fees of \$21.4 million and \$4.1 million under our collaboration agreements with Biogen and Novartis entered into in 2020, respectively. In addition, an increase of \$6.2 million in recognition of upfront license fee related to our C9ORF72 collaboration agreement with Pfizer due to advancement of the program.

These increases were partially offset by a decrease of \$12.6 million in revenues related to our hemophilia A collaboration agreement with Pfizer due to a decrease in activities following the IND transfer in December 2019, and a decrease of \$3.2 million in license revenue from our collaboration agreement with Sanofi primarily due to a change in estimate driven by a change in project scope and related project cost.

#### Operating Expenses

	Year Ended December 31,							
	(In thousands, except percentage values)							
	2020	2019	Change	%	2019	2018	Change	%
Operating expenses:								
Research and development	\$ 180,647	\$ 145,922	\$ 34,725	24 %	\$ 145,922	\$ 114,866	\$ 31,056	27 %
General and administrative	67,097	61,686	5,411	9 %	61,686	46,736	14,950	32 %
Total operating expenses	\$ 247,744	\$ 207,608	\$ 40,136	19 %	\$ 207,608	\$ 161,602	\$ 46,006	28 %

#### Research and Development Expenses

Research and development expenses consisted primarily of compensation related expenses, including stock-based compensation, laboratory supplies, preclinical and clinical studies, manufacturing clinical supply, contracted research, allocated facilities and information technology expenses.

The increase of \$34.7 million in research and development expenses in 2020 compared to 2019 was primarily driven by a \$20.0 million increase in compensation expense as a result of increased headcount to support our programs, clinical trials and start-up of our internal manufacturing operations, a \$14.2 million increase in allocated facility overhead costs as we ramped up our internal manufacturing operations, and a \$2.1 million increase in preclinical, clinical, research and manufacturing supply expenses. These increases were partially offset by a decrease of \$1.7 million in travel and corporate costs due to COVID-19 travel restrictions. Stock-based compensation expense included in research and development expenses was \$13.5 million and \$10.1 million for the years ended December 31, 2020 and 2019, respectively.

The table below shows research and development expenses related to our clinical and preclinical programs. As shown in the table below, preclinical and research programs contributed \$50.7 million of the increase in our research and development expenses primarily due to advancement of our technical platform and our wholly-owned preclinical programs, and our CNS programs partnered with Biogen and Novartis in 2020, offset by a decrease of \$16.0 million in our clinical programs in 2020 as compared to 2019, primarily driven by a decrease in activities following the IND transfer for our giroctocogene fitelparvovec program in 2019.

Programs	Year Ended December 31,		
	(In thousands)		
	2020	2019	2018
<b>Clinical Programs:</b>			
Inherited metabolic disorders clinical programs	\$ 59,030	\$ 61,845	\$ 37,668
Beta thalassemia clinical program	8,672	13,634	12,317
HIV clinical programs	3,289	1,762	1,612
Hemophilia clinical programs	3,108	12,805	23,006
Subtotal	74,099	90,046	74,603
<b>Preclinical Programs:</b>			
Wholly-owned programs and early research activities	79,193	37,760	28,018
CNS programs	21,073	5,721	3,916
Oncology programs	6,214	12,281	7,845
Other	68	114	484
Subtotal	106,548	55,876	40,263
<b>Total research and development expenses</b>	<b>\$ 180,647</b>	<b>\$ 145,922</b>	<b>\$ 114,866</b>

The length of time required to complete our development programs and our development costs for those programs may be impacted by the scope and timing of enrollment in clinical trials for our product candidates, our decisions to pursue development programs in other therapeutic areas, and whether we pursue development of our product candidates with a partner or collaborator or independently. For example, our product candidates are being developed in multiple therapeutic areas, and we do not yet know-how many of those therapeutic areas we will continue to pursue. Furthermore, the scope and number of clinical trials required to obtain regulatory approval for each pursued therapeutic area is subject to the input of the applicable regulatory authorities, and we have not yet sought such input for all potential therapeutic areas that we may elect to pursue, and even after having given such input, applicable regulatory authorities may subsequently require additional clinical studies prior to granting regulatory approval based on new data generated by us or other companies, or for other reasons outside of our control. As a condition to any regulatory approval, we may also be subject to post-marketing development commitments, including additional clinical trial requirements. As a result of the uncertainties discussed above, we are unable to determine the duration of or completion costs associated with our development programs.

In any event, our potential therapeutic products are subject to a lengthy and uncertain regulatory process that may not result in our receipt of any necessary regulatory approvals. Failure to receive the necessary regulatory approvals would prevent us from commercializing the product candidates affected. In addition, clinical trials of our product candidates may fail to demonstrate safety and efficacy, which could prevent or significantly delay regulatory approval. A discussion of the risks and uncertainties with respect to our research and development activities, including completing the development of our product candidates, and the consequences to our business, financial position and growth prospects can be found in “Risk Factors” in Part I, Item 1A of this Annual Report on Form 10-K.

#### *General and Administrative Expenses*

The increase of \$5.4 million in general and administrative expenses in 2020 compared to 2019 was primarily due to an increase of \$7.3 million in headcount driven compensation costs, and an increase of \$6.3 million in professional fees associated

with our 2020 business activities. These increases were partially offset by a decrease of \$7.4 million in allocated facility overhead costs, and a decrease of \$1.1 million in travel related expenses due to COVID-19 travel restrictions. Stock-based compensation expense included in general and administrative expenses was \$12.2 million and \$9.2 million for the years ended December 31, 2020 and 2019, respectively.

### ***Interest and other income, net***

The decrease of \$1.0 million in interest and other income, net in 2020 compared to 2019 was primarily due to a decrease of \$5.1 million in interest income reflecting the decline in market interest rates. This decrease was partially offset by an increase of \$3.1 million in foreign exchange gains as a result of favorable foreign exchange rates and an increase of \$1.1 million in other income.

### ***Income tax expense***

Provision for income taxes was \$0.3 million, zero, and zero for 2020, 2019 and 2018, respectively. The income tax expense for 2020 was primarily due to an increase in the long-term liability associated with uncertain tax positions, foreign income taxes and state income taxes. This expense was partially offset by a foreign deferred tax benefit.

As of December 31, 2020, we had net operating loss carryforwards for federal and state income tax purposes of approximately \$622.6 million and \$261.7 million, respectively. The federal net operating loss generated before 2018 will begin to expire in 2024 and will keep expiring through 2037, if not utilized. Federal net operating losses generated in 2018 will carry forward indefinitely. If not utilized, the state net operating loss carryforwards will begin to expire in 2029. We also have federal and state research tax credit carryforwards of \$21.9 million and \$18.3 million, respectively. The federal research credits will begin to expire in 2021, while the state research credits have no expiration date. Utilization of our net operating loss carryforwards and research tax credit carryforwards may be subject to substantial annual limitations due to the ownership change limitations provided by the Internal Revenue Code and similar state provisions. The annual limitation could result in the expiration of the net operating loss carryforwards and research tax credit carryforwards before use. Due to the carryforwards related to the net operating losses and research and development tax credits, we do not expect to pay any U.S. federal taxes related to income in the near future.

## **Liquidity and Capital Resources**

### ***Liquidity***

Since inception, we have incurred significant net losses, and we have funded our operations primarily through the issuance of equity securities, payments from corporate collaborators and strategic partners and research grants.

As of December 31, 2020, we had cash, cash equivalents and marketable securities totaling \$692.0 million compared to \$384.3 million as of December 31, 2019 with the increase primarily attributable to our collaboration with and sale of our common stock to Biogen, which became effective and closed in April 2020, and our collaboration with Novartis, which became effective in July 2020 and included an upfront fee. Our most significant use of capital was for employee compensation and external research and development expenses, such as manufacturing, clinical trials and preclinical activity related to our therapeutic programs. Our cash and investment balances are held in a variety of interest-bearing instruments, including commercial paper, money market funds, corporate debt securities, certificates of deposit, asset-backed securities, and U.S. government-sponsored entity debt securities. Cash in excess of immediate requirements is invested in accordance with our investment policy with a view toward capital preservation and liquidity.

Since the beginning of 2017, we have received significant amounts of capital as upfront payments under our collaboration agreements. In addition, our collaboration agreements provide for the payment of development, regulatory, and commercial milestones. For example, in May 2020, we received an upfront license fee of \$125.0 million from Biogen upon the effectiveness of our collaboration and license agreement for the research, development and commercialization of gene regulation therapies for the treatment of neurological diseases. And in August 2020, we received an upfront license fee of \$75.0 million from Novartis upon the effectiveness of our collaboration and license agreement for the development and commercialization of gene regulation therapies for the treatment of neurodevelopment disorders. For more information, see “Business – Collaborations” in Part I, Item 1 of this Annual Report on Form 10-K.

In August 2020, we entered into an Open Market Sale Agreement with Jefferies LLC, or Jefferies, providing for the sale of up to \$150.0 million of our common stock from time to time in ‘at-the-market’ offerings under our existing shelf registration statement. As of February 19, 2021, we have sold 1,034,762 shares of our common stock under the sales agreement for net proceeds of approximately \$15.7 million.

While we expect our rate of cash usage to increase in the future, in particular to support our product development endeavors, we currently believe that our available cash, cash equivalents and marketable securities, when combined with

additional capital raises and expected revenues from collaborations, strategic partnerships and research grants, will be adequate to fund our currently planned operations through at least the next 12 months from the date the financial statements are issued. During this period of uncertainty and volatility related to the evolving COVID-19 pandemic, we will continue to monitor our liquidity.

### **Cash Flows**

#### *Operating activities*

Net cash provided by operating activities was \$169.9 million in 2020 compared to net cash used in operating activities of \$144.4 million in 2019. The increase was primarily attributable to favorable changes in working capital of \$325.6 million driven by an increase in deferred revenues of \$252.2 million, reflecting the cash received under the Biogen and Novartis collaboration agreements, and a decrease in accounts receivable of \$63.9 million. This increase was partially offset by an increase in our net loss of \$25.7 million.

#### *Investing activities*

Net cash used in investing activities was \$271.6 million in 2020, and \$59.8 million in 2019. The increase was primarily due to a net increase of \$217.3 million in the purchase of marketable securities, partially offset by a decrease of \$6.0 million in purchases of property and equipment.

#### *Financing activities*

Net cash provided by financing activities was \$153.1 million in 2020, and \$142.0 million in 2019. The increase was primarily attributed to the \$145.4 million estimated fair value of the Biogen Shares issued in April 2020, net of \$2.9 million of issuance costs, partially offset by \$136.3 million received in 2019 related to our April 2019 underwritten public offering.

### **Operating Capital and Capital Expenditure Requirements**

We anticipate continuing to incur operating losses for at least the next several years. While we expect our rate of cash usage to increase in the future, in particular to support our product development endeavors, we currently believe that our available cash resources, when combined with additional capital raises and expected revenues from collaborations, strategic partners and research grants, will be adequate to fund our currently planned operations through at least the next 12 months from the date the financial statements are issued. Although we believe we are well capitalized currently, the effects of the ongoing COVID-19 pandemic could result in significant disruption of global financial markets, impairing our ability to access capital, which could in the future negatively affect our liquidity. Future capital requirements beyond the next 12 months will be substantial and we will need to raise substantial additional capital to fund the development, manufacturing and potential commercialization of our product candidates through equity or debt financing. In addition, as we focus our efforts on proprietary human therapeutics, we will need to seek FDA approvals of our product candidates, a process that could cost in excess of hundreds of millions of dollars per product. We regularly consider fund-raising opportunities and may decide, from time to time, to raise capital based on various factors, including market conditions and our plans of operation. Additional capital may not be available on terms acceptable to us, or at all. If adequate funds are not available, or if the terms of potential funding sources are unfavorable, our business and our ability to advance our product candidate pipeline would be harmed. Furthermore, any sales of additional equity securities, including sales pursuant to our at-the-market financing offering programs, may result in dilution to our stockholders, and any debt financing may include covenants that restrict our business.

Our future capital requirements will depend on many forward-looking factors, including the following:

- the initiation, progress, timing and completion of clinical trials for our product candidates and potential product candidates;
- the outcome, timing and cost of regulatory approvals;
- the success of our collaboration agreements;
- delays that may be caused by changing regulatory requirements;
- the number of product candidates that we pursue;
- the costs involved in filing and prosecuting patent applications and enforcing and defending patent claims;
- the timing and terms of future in-licensing and out-licensing transactions;
- the cost and timing of establishing sales, marketing, manufacturing and distribution capabilities;
- the cost of procuring clinical and commercial supplies of our product candidates;
- the extent to which we acquire or invest in businesses, products or technologies, including the costs associated with such acquisitions and investments; and
- the costs of potential disputes and litigation.

**Off-Balance Sheet Arrangements**

We do not have any off-balance sheet arrangements as defined in Item 303(a)(4)(ii) of Regulation S-K.

**Contractual Obligations**

As of December 31, 2020, we had contractual obligations and commercial commitments as follows (in thousands):

Contractual Obligations	Payments Due by Period				
	Total	Less Than 1 Year	1-3 Years	4-5 Years	More Than 5 Years
Operating leases	\$ 53,422	\$ 6,191	\$ 13,607	\$ 14,053	\$ 19,571
License obligations	958	178	290	280	210
Manufacturing obligations	21,507	14,605	6,902	—	—
Total contractual obligations	\$ 75,887	\$ 20,974	\$ 20,799	\$ 14,333	\$ 19,781

Operating leases consist of base rents for facilities we occupy in Brisbane, California; Richmond, California; and Valbonne, France.

License obligations includes an ongoing license maintenance fee associated with cancellable in-licensed patent agreements.

Manufacturing obligations include the following non-cancelable material contractual commitments under manufacturing-related supplier arrangements as of December 31, 2020 (in thousands):

Party	Total	Expiry date
Brammer	\$ 7,736	December 2022
Lonza Netherlands, B.V.	13,771	December 2022
Total manufacturing obligations	\$ 21,507	

**ITEM 7A – QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK**

Our exposure to market risk relates to our cash, cash equivalents and marketable securities. The goals of our investment policy are preservation of capital, fulfillment of liquidity needs and capturing a market rate of return based on our investment policy parameters and market conditions. We select investments that maximize interest income to the extent possible within these guidelines. To achieve our goals, we maintain a portfolio of cash equivalents and investments in securities of high credit quality and with varying maturities to match projected cash needs.

The securities in our investment portfolio are not leveraged and are classified as available-for-sale. The majority of these available-for-sale securities are short-term in nature and subject to minimal interest rate risk. Our investments currently consist of commercial paper, money market funds, corporate debt securities, certificates of deposit, asset-backed securities and U.S. government-sponsored entity debt securities. Our investment policy, approved by our Board of Directors, limits the amount we may invest in any one type of investment issuer, thereby reducing credit risk concentrations. All investments have a fixed interest rate and are carried at market value, which approximates cost. We do not use derivative financial instruments in our investment portfolio. We do not believe that a change in interest rates would have a material negative impact on the value of our investment portfolio.

**Foreign Currency Exchange Risk**

We have operations in the United States as well as in Europe. The functional currency of each foreign subsidiary is the local currency. We are exposed to foreign currency risk, primarily through operations of our subsidiaries in Europe which conduct business primarily in Euros. We record gains and losses within our stockholders' equity due to the translation of our subsidiaries' financial statements into U.S. dollars.

A 10% strengthening/(weakening) in the rates used to translate the results of our foreign subsidiaries would have increased/(decreased) net loss for the year ended December 31, 2020 by approximately \$2.1 million and would not have materially impacted our operating loss.

Additionally, we incur foreign currency transaction gains and losses related to the level of activity between the United States and Europe. In 2020, we incurred foreign currency transaction gains of \$2.2 million. A 10% unfavorable change in the Euro and U.S. dollar exchange rate on December 31, 2020 would have had an immaterial impact on foreign currency transaction gains for 2020.

As of December 31, 2020 and 2019, we maintained cash balances of approximately \$16.8 million and \$22.6 million, respectively, denominated in a foreign currency in the United States. A hypothetical 10% change in foreign exchange rates would have increased/(decreased) net loss for the year ended December 31, 2020 by approximately \$1.7 million and would not have materially impacted our operating loss.

**ITEM 8 – FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA**

**SANGAMO THERAPEUTICS, INC.**

**INDEX TO CONSOLIDATED FINANCIAL STATEMENTS**

	<u>Page</u>
<a href="#">Report of Independent Registered Public Accounting Firm</a>	80
<a href="#">Consolidated Balance Sheets</a>	82
<a href="#">Consolidated Statements of Operations</a>	83
<a href="#">Consolidated Statements of Comprehensive Loss</a>	84
<a href="#">Consolidated Statements of Stockholders' Equity</a>	85
<a href="#">Consolidated Statements of Cash Flows</a>	86
<a href="#">Notes to Consolidated Financial Statements</a>	87

## REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Stockholders and the Board of Directors of Sangamo Therapeutics, Inc.

### Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Sangamo Therapeutics, Inc. (the Company) as of December 31, 2020 and 2019, the related consolidated statements of operations, comprehensive loss, stockholders' equity, and cash flows for each of the three years in the period ended December 31, 2020, and 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 at December 31, 2020 and 2019, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2020, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2020, based on criteria established in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) and our report dated February 24, 2021 expressed an unqualified opinion thereon.

### Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the 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 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 financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the 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 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 financial statements. We believe that our audits provide a reasonable basis for our opinion.

### Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of the critical audit matter 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.



*Estimation of Fair value of shares sold to Biogen*

*Description of the Matter*

As described in Note 4 to the consolidated financial statements, in 2020, the Company entered into a collaboration and license agreement with Biogen MA, Inc. and Biogen International GmbH (together, “Biogen”) for the research, development and commercialization of gene regulation therapies for the treatment of neurological diseases. Concurrent with the execution of the Biogen collaboration agreement, the Company entered into a stock purchase agreement pursuant to which Biogen agreed to purchase 24.4 million shares of the Company’s common stock for an aggregate purchase price of approximately \$225.0 million, a price which exceeded the fair value of the shares at the time of the purchase. Since the purchase price exceeded the fair value of the common shares issued in this transaction, management had to estimate the fair value of these shares using an option pricing model to reflect certain holding period restrictions and other unique terms of the agreement. The fair value of these shares was estimated to be \$145.4 million, and the \$79.6 million excess consideration was included in the transaction price of the collaboration and license agreement.

Auditing the estimated fair value of the shares issued in this transaction was complex as management had to use significant judgment in selecting the valuation methodology and estimating the significant unobservable inputs used in the valuation model. The use of a different valuation methodology and inputs could significantly affect the fair value assigned to the common shares thereby impacting the total transaction price of the collaboration and license agreement and ultimately the amount of revenue that is recognized under this agreement.

*How We Addressed the Matter in Our Audit*

We obtained an understanding, evaluated the design and tested the operating effectiveness of controls over the Company’s accounting for this new collaboration agreement, including management’s review controls over development of the significant unobservable inputs that affected the determination of the fair value of the common shares issued to Biogen.

To test the fair value of the common shares, our audit procedures included, among others, evaluating the Company’s valuation methodology and the significant unobservable inputs and testing the mathematical accuracy of the Company’s valuation calculations. We involved our valuation specialists to assist us with evaluating the Company’s valuation methodology and assessing the reasonableness of the inputs by comparing them to information available from third-party sources and market data. We also independently developed fair value estimates with the assistance of our valuation specialists and compared them to the Company’s valuation results.

/s/ ERNST & YOUNG LLP

We have served as the Company’s auditor since 1997.

Redwood City, California  
February 24, 2021

**SANGAMO THERAPEUTICS, INC.**  
**CONSOLIDATED BALANCE SHEETS**  
(in thousands, except share and per share amounts)

	December 31, 2020	December 31, 2019
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 131,329	\$ 80,428
Marketable securities	510,094	282,046
Interest receivable	1,035	682
Accounts receivable	5,224	36,909
Prepaid expenses and other current assets	11,986	5,408
Total current assets	659,668	405,473
Marketable securities, non-current	50,530	21,832
Property and equipment, net	41,324	29,926
Intangible assets	58,128	53,156
Goodwill	42,798	39,273
Operating lease right-of-use assets	71,045	77,289
Other non-current assets	13,557	9,067
Restricted cash	1,500	1,500
Total assets	\$ 938,550	\$ 637,516
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 12,553	\$ 6,671
Other accrued liabilities	18,612	10,885
Accrued compensation and employee benefits	20,738	13,605
Deferred revenues	91,644	38,711
Total current liabilities	143,547	69,872
Deferred revenues, non-current	245,045	81,432
Long-term portion of lease liabilities	38,396	41,192
Deferred income tax	7,185	6,570
Other non-current liabilities	7,011	5,711
Total liabilities	441,184	204,777
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.01 par value, 5,000,000 shares authorized, and no shares issued or outstanding	—	—
Common stock, \$0.01 par value; 320,000,000 shares authorized, 142,063,203 and 115,972,708 shares issued and outstanding at December 31, 2020 and 2019, respectively	1,421	1,160
Additional paid-in capital	1,269,375	1,090,828
Accumulated deficit	(777,981)	(656,985)
Accumulated other comprehensive income (loss)	5,419	(2,449)
Total Sangamo Therapeutics, Inc. stockholders' equity	498,234	432,554
Non-controlling interest	(868)	185
Total stockholders' equity	497,366	432,739
Total liabilities and stockholders' equity	\$ 938,550	\$ 637,516

See accompanying Notes to Consolidated Financial Statements.

**SANGAMO THERAPEUTICS, INC.**  
**CONSOLIDATED STATEMENTS OF OPERATIONS**

(in thousands, except per share amounts)

	Year Ended December 31,		
	2020	2019	2018
Revenues	\$ 118,192	\$ 102,428	\$ 84,452
Operating expenses:			
Research and development	180,647	145,922	114,866
General and administrative	67,097	61,686	46,736
Total operating expenses	247,744	207,608	161,602
Loss from operations	(129,552)	(105,180)	(77,150)
Interest and other income, net	8,775	9,761	8,261
Loss before income taxes	(120,777)	(95,419)	(68,889)
Income tax expense	345	—	—
Net loss	(121,122)	(95,419)	(68,889)
Net loss attributable to non-controlling interest	(126)	(233)	(555)
Net loss attributable to Sangamo Therapeutics, Inc. stockholders	\$ (120,996)	\$ (95,186)	\$ (68,334)
Basic and diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders	\$ (0.90)	\$ (0.85)	\$ (0.70)
Shares used in computing basic and diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders	134,449	112,114	96,941

See accompanying Notes to Consolidated Financial Statements.

**SANGAMO THERAPEUTICS, INC.**  
**CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS**  
**(in thousands)**

	Year Ended December 31,		
	2020	2019	2018
Net loss	\$ (121,122)	\$ (95,419)	\$ (68,889)
Foreign currency translation adjustment	8,345	(1,573)	(1,148)
Net pension losses	(193)	(28)	(21)
Change in unrealized (loss) gain on marketable securities, net of tax	(284)	592	(4)
Comprehensive loss	(113,254)	(96,428)	(70,062)
Comprehensive loss attributable to non-controlling interest	(126)	(233)	(574)
Comprehensive loss attributable to Sangamo Therapeutics, Inc.	\$ (113,128)	\$ (96,195)	\$ (69,488)

See accompanying Notes to Consolidated Financial Statements.

**SANGAMO THERAPEUTICS, INC.**  
**CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY**  
(in thousands, except share amounts)

	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Income (Loss)	Non-Controlling Interest	Total Stockholders' Equity
	Shares	Amount					
Balances at December 31, 2017	85,598,534	\$ 856	\$ 682,809	\$ (495,479)	\$ (286)	\$ —	\$ 187,900
Cumulative-effect adjustment of ASC Topic 606 on January 1, 2018	—	—	—	1,117	—	—	1,117
Issuance of common stock upon exercise of stock options and in connection with restricted stock units, net of tax	2,103,727	20	14,447	—	—	—	14,467
Issuance of common stock under employee stock purchase plan	328,710	4	1,480	—	—	—	1,484
Issuance of common stock under public offering, net of issuance costs	14,156,500	142	215,616	—	—	—	215,758
Stock-based compensation	—	—	14,677	—	—	—	14,677
Additional paid-in capital for acquisition of Sangamo France	—	—	603	—	—	—	603
Non-controlling interest upon acquisition of Sangamo France	—	—	—	—	—	1,313	1,313
Foreign currency translation adjustment	—	—	—	—	(1,129)	(19)	(1,148)
Net pension losses	—	—	—	—	(21)	—	(21)
Net unrealized loss on marketable securities, net of tax	—	—	—	—	(4)	—	(4)
Net loss	—	—	—	(68,334)	—	(555)	(68,889)
Balances at December 31, 2018	102,187,471	1,022	929,632	(562,696)	(1,440)	739	367,257
Cumulative-effect adjustment of ASC Topic 842 on January 1, 2019	—	—	—	897	—	—	897
Issuance of common stock upon exercise of stock options and in connection with restricted stock units, net of tax	885,873	9	3,668	—	—	—	3,677
Issuance of common stock under employee stock purchase plan	249,364	2	2,042	—	—	—	2,044
Issuance of common stock under public offering, net of issuance costs	12,650,000	127	136,181	—	—	—	136,308
Stock-based compensation	—	—	19,330	—	—	—	19,330
Acquisition of additional shares of Sangamo France	—	—	—	—	—	(321)	(321)
Issuance costs related to Sangamo France acquisition	—	—	(25)	—	—	—	(25)
Foreign currency translation adjustment	—	—	—	—	(1,573)	—	(1,573)
Net pension losses	—	—	—	—	(28)	—	(28)
Net unrealized gain on marketable securities, net of tax	—	—	—	—	592	—	592
Net loss	—	—	—	(95,186)	—	(233)	(95,419)
Balances at December 31, 2019	115,972,708	1,160	1,090,828	(656,985)	(2,449)	185	432,739
Issuance of common stock upon exercise of stock options and in connection with restricted stock units, net of tax	1,395,956	14	8,545	—	—	—	8,559
Issuance of common stock under employee stock purchase plan	274,382	3	2,012	—	—	—	2,015
Issuance of common stock in connection with the Biogen collaboration agreement, net of issuance costs	24,420,157	244	142,282	—	—	—	142,526
Stock-based compensation	—	—	25,708	—	—	—	25,708
Acquisition of additional shares of Sangamo France	—	—	—	—	—	(927)	(927)
Foreign currency translation adjustment	—	—	—	—	8,345	—	8,345
Net pension losses	—	—	—	—	(193)	—	(193)
Net unrealized loss on marketable securities, net of tax	—	—	—	—	(284)	—	(284)
Net loss	—	—	—	(120,996)	—	(126)	(121,122)
Balances at December 31, 2020	142,063,203	\$ 1,421	\$ 1,269,375	\$ (777,981)	\$ 5,419	\$ (868)	\$ 497,366

See accompanying Notes to Consolidated Financial Statements.

**SANGAMO THERAPEUTICS, INC.**  
**CONSOLIDATED STATEMENTS OF CASH FLOWS**  
(in thousands)

	Year Ended December 31,		
	2020	2019	2018
<b>Operating Activities:</b>			
Net loss	\$ (121,122)	\$ (95,419)	\$ (68,889)
Adjustments to reconcile net loss to net cash provided by (used in) operating activities:			
Depreciation and amortization	5,682	3,930	2,359
Amortization of discount on marketable securities	(825)	(4,708)	(5,829)
Amortization and other changes in right-of-use assets	7,687	5,677	—
Gain on free shares	(63)	(488)	—
Net loss on disposal of property and equipment	222	68	—
Stock-based compensation	25,708	19,330	14,677
Net loss on lease termination	—	218	—
Build-to-suit leases	—	—	966
Net changes in operating assets and liabilities:			
Interest receivable	(353)	(307)	(135)
Accounts receivable	31,685	(32,236)	(1,330)
Prepaid expenses and other assets	(10,411)	(6,660)	(2,828)
Accounts payable and other accrued liabilities	10,703	(4,192)	(6,372)
Accrued compensation and employee benefits	6,877	4,129	2,604
Deferred revenues	216,546	(35,693)	99,364
Long-term portion of lease liabilities	(3,761)	(1,800)	—
Other non-current liabilities	1,300	3,749	1,963
Net cash provided by (used in) operating activities	169,875	(144,402)	36,550
<b>Investing Activities:</b>			
Acquisition, net of cash acquired	—	—	(75,647)
Purchases of marketable securities	(570,779)	(443,711)	(451,239)
Maturities of marketable securities	314,570	404,847	391,845
Purchases of property and equipment	(14,714)	(20,675)	(43,065)
Purchase of additional Sangamo France shares	(704)	(262)	—
Net cash used in investing activities	(271,627)	(59,801)	(178,106)
<b>Financing Activities:</b>			
Proceeds from public offering of common stock, net of issuance costs	—	136,308	215,758
Proceeds from issuance of common stock in connection with the Biogen collaboration agreement, net of issuance costs	142,526	—	—
Taxes paid related to net share settlement of equity awards	(765)	(422)	(254)
Proceeds from issuance of common stock under employee stock purchase plan	2,015	2,044	1,484
Proceeds from exercise of stock options and restricted stock units	9,324	4,099	14,721
Net cash provided by financing activities	153,100	142,029	231,709
Effect of exchange rate changes on cash and cash equivalents	(447)	184	439
Net increase (decrease) in cash, cash equivalents, and restricted cash	50,901	(61,990)	90,592
Cash, cash equivalents, and restricted cash, beginning of period	81,928	143,918	53,326
Cash, cash equivalents, and restricted cash, end of period	\$ 132,829	\$ 81,928	\$ 143,918
<b>Supplemental cash flow disclosures:</b>			
Non-controlling interest for acquisition	\$ —	\$ —	\$ 1,313
Property and equipment included in unpaid liabilities	\$ 4,569	\$ 2,114	\$ 4,953
Build-to-suit leases included in build-to-suit liabilities	\$ —	\$ —	\$ 2,950
Right-of-use assets obtained in exchange for lease obligations	\$ 1,333	\$ 31,291	\$ —

See accompanying Notes to Consolidated Financial Statements

**SANGAMO THERAPEUTICS, INC.****NOTES TO CONSOLIDATED FINANCIAL STATEMENTS****NOTE 1 – ORGANIZATION AND SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES****Business Overview**

Sangamo Therapeutics, Inc. (“Sangamo” or “the Company”) was incorporated in the State of Delaware in June 1995 and changed its name from Sangamo Biosciences, Inc. in January 2017. Sangamo is a clinical-stage genomic medicine company committed to translating ground-breaking science into medicines that transform the lives of patients with serious diseases using its platform technologies in gene therapy, cell therapy and genome engineering.

**Basis of Presentation**

The accompanying Consolidated Financial Statements have been prepared in conformity with generally accepted accounting principles in the United States of America (“U.S. GAAP”) and include the accounts of the Company and its subsidiaries. All intercompany balances and transactions have been eliminated in the Consolidated Financial Statements. For consolidated entities where the Company owns or is exposed to less than 100% of the economics, the Company records net loss attributable to non-controlling interests on its Consolidated Statements of Operations equal to the percentage of the economic or ownership interest retained in such entities by the respective non-controlling parties.

***Liquidity and Management’s Plan***

Sangamo is currently working on a number of long-term development projects that involve experimental technologies. The projects may require several years and substantial expenditures to complete and ultimately may be unsuccessful. The Company plans to finance operations with available cash resources, collaborations and strategic partnerships funds, research grants and from the issuance of equity or debt securities. Sangamo believes that its available cash, cash equivalents and marketable securities as of December 31, 2020, when combined with additional capital raises and expected revenues from collaborations, strategic partnerships and research grants, will be adequate to fund its operations at least through the next 12 months from the date these Consolidated Financial Statements are issued. Sangamo will require substantial additional financial resources to complete the development and commercialization of its product candidates. Additional capital may not be available on terms acceptable to the Company, or at all. If adequate funds are not available, or if the terms of potential funding sources are unfavorable, the Company’s business and ability to develop its technology and therapeutic products would be harmed. Furthermore, any sales of additional equity securities may result in dilution to the Company’s stockholders, and any debt financing may include covenants that restrict the Company’s business.

**Summary of Significant Accounting Policies*****Use of Estimates***

The preparation of the accompanying Consolidated Financial Statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts reported in the Consolidated Financial Statements and the accompanying notes. On an ongoing basis, management evaluates its estimates including critical accounting policies or estimates related to revenue recognition, including the estimated fair value of common shares issued as part of the transaction price, clinical trial accruals, fair value of assets and liabilities, including from acquisitions, and stock-based compensation. Estimates are based on historical experience and on various other market specific and other relevant assumptions that the Company believes to be 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. Actual results could differ from those estimates.

During the year ended December 31, 2020, the Company recorded adjustments to revenue related to changes in estimates in connection with the collaboration agreements with Sanofi Genzyme (“Sanofi”) and Pfizer Inc. (“Pfizer”). These changes in estimates were driven by changes in project scope and related project costs which resulted in changes to the measure of proportional cumulative performance. These adjustments increased revenue by \$8.9 million, decreased net loss by \$8.9 million and decreased the Company’s basic net loss per share by \$0.06 for the year ended December 31, 2020.

During the year ended December 31, 2019, the Company recorded adjustments to revenue related to a change in estimate in connection with the giroctocogene fitelparvovec collaboration agreement with Pfizer Inc. (“Pfizer”). This change in estimate was driven by changes in project scope and related project costs which resulted in changes to the measure of proportional cumulative performance. These adjustments increased revenue by \$5.7 million, decreased net loss by \$5.7 million and decreased the Company’s basic net loss per share by \$0.05 for the year ended December 31, 2019.

## Revenue Recognition

The Company accounts for its revenues pursuant to the provisions of Accounting Standards Codification (“ASC”) Topic 606, *Revenue from Contracts with Customers* (“ASC Topic 606”). The Company’s contract revenues are derived from collaboration agreements including licensing arrangements and research activity grants. Research and licensing agreements typically include upfront signing or license fees, cost reimbursements for research services, minimum sublicense fees, milestone payments and royalties on future licensee’s product sales. The Company has agreements with both fixed and variable consideration. Non-refundable upfront fees and funding of research and development activities are considered fixed, while milestone payments are generally identified as variable consideration. Sangamo’s research grants are typically multi-year agreements and provide for the reimbursement of qualified expenses for research and development as defined under the terms of the grant agreement. Revenues under research grant agreements are generally recognized when the related qualified research expenses are incurred. Deferred revenue primarily represents the portion of research or license payments received but not earned.

In determining the appropriate amount of revenue to be recognized as the Company fulfills its obligations under its agreements, the Company performs the following steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations, including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations based on estimated selling prices; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation.

A performance obligation is a promise in a contract to transfer a distinct good or service to the customer and is the unit of account in ASC Topic 606. The Company’s performance obligations include license rights, development services and services associated with regulatory submission and approval processes. Revenues from research services earned under collaboration agreements are generally recognized as revenue as the related services are provided. Revenues from non-refundable upfront fees are recognized over time either by measuring progress towards satisfaction of the relevant performance obligation, using the input method (i.e., cumulative actual costs incurred relative to total estimated costs) or on a straight-line basis when a performance obligation is expected to be satisfied evenly over a period of time (or when the entity has a stand-ready obligation). Significant management judgment is required to determine the level of effort required under an arrangement, and the period over which the Company expects to complete its performance obligations under the arrangement, which may include total internal personnel costs and external costs to be incurred as well as, in certain cases, the estimated stand-ready obligation period. Changes in these estimates can have a material effect on revenue recognized. If the Company cannot reasonably estimate when its performance obligations either are completed or become inconsequential, then revenue recognition is deferred until the Company can reasonably make such estimates. The Company includes the unconstrained amount of estimated variable consideration in the transaction price. The amount included in the transaction price is constrained to the amount for which it is probable that a significant reversal of cumulative revenue recognized will not occur. At the end of each subsequent reporting period, the Company re-evaluates the estimated variable consideration included in the transaction price and any related constraint and, if necessary, adjusts its estimate of the overall transaction price. Revenue is then recognized over the remaining estimated period of performance using the cumulative catch-up method. The estimated period of performance and project costs, such as personnel and manufacturing cost, are reviewed quarterly and adjusted, as needed, to reflect the Company’s current assumptions regarding the timing of its deliverables.

As part of the accounting for these arrangements, the Company must develop assumptions that require judgment to determine the stand-alone selling price of each performance obligation identified in the contract. The Company uses key assumptions to determine the stand-alone selling price, which may include forecasted revenues, development timelines, reimbursement rates for personnel costs, discount rates and probabilities of technical and regulatory success. Related costs and expenses under these arrangements have historically approximated the revenues recognized.

Revenues from major collaboration agreements and research activity grants as a percentage of total revenues were as follows:

	Year Ended December 31,		
	2020	2019	2018
Pfizer Inc.	40 %	40 %	47 %
Kite Pharma, Inc.	24 %	34 %	30 %
Biogen MA, Inc.	24 %	—	—
Sanofi Genzyme	5 %	22 %	16 %
Novartis Institutes for BioMedical Research, Inc.	4 %	—	—



Receivables from collaborations are typically unsecured and are concentrated in the biopharmaceutical industry. Accordingly, the Company may be exposed to credit risk generally associated with biopharmaceutical companies or specific to its collaboration agreements. To date, the Company has not experienced any losses related to these receivables.

Funds received from the Company's collaboration partners are generally not refundable and are recorded as revenue as it fulfills its performance obligations. The Company's performance obligations are satisfied over time (i.e., stand ready obligations) or using the input method (i.e., cumulative actual costs incurred relative to total estimated costs). Revenue is also recognized when the Company has incurred qualified research and development costs that are reimbursable from its collaboration partners and when there is reasonable assurance that such costs will be reimbursed. Any payments received from a collaboration partner in advance of the completion of the relevant performance obligation are recorded as deferred revenue.

### ***Business Combinations***

The Company accounts for acquisitions using the acquisition method of accounting, which requires that assets acquired, including in-process research and development ("IPR&D") projects, liabilities assumed and any non-controlling interests in the acquired target in an acquisition be recorded at their fair values as of the acquisition date on the Company's Consolidated Balance Sheets. Any excess of purchase consideration over the fair value of net assets acquired is recorded as goodwill. The determination of estimated fair value requires the Company to make significant estimates and assumptions. As a result, the Company may record adjustments to the fair values of assets acquired and liabilities assumed within the measurement period (up to one year from the acquisition date) with the corresponding offset to goodwill. Transaction costs associated with business combinations are expensed as they are incurred.

### ***Goodwill and Intangible Assets***

Goodwill represents the excess of the purchase consideration transferred over the estimated fair values of assets acquired and liabilities assumed in a business combination. Intangible assets with indefinite useful lives are related to purchased IPR&D projects and are measured at their respective fair values as of the acquisition date. Goodwill and intangible assets with indefinite useful lives are not amortized. Intangible assets related to IPR&D projects are considered to be indefinite-lived until the completion or abandonment of the associated research and development efforts. If and when development is complete, which generally occurs if and when regulatory approval to market a product is obtained, the associated assets would be deemed finite-lived and would then be amortized based on their respective estimated useful lives at that point in time. The Company tests goodwill and indefinite-lived intangible assets for impairment on an annual basis and between annual tests if the Company becomes aware of any events occurring or changes in circumstances that would indicate the fair values of the assets are below their respective carrying amounts. As of December 31, 2020, no impairment of goodwill or indefinite-lived intangible assets was identified.

### ***Valuation of Long-Lived Assets***

Long-lived assets, including property and equipment and finite-lived intangible assets, are reviewed for impairment whenever facts or circumstances either internally or externally may suggest that the carrying value of an asset may not be recoverable. Recoverability of these assets is measured by comparison of the carrying amount of each asset to the future undiscounted cash flows expected to result from the use of the asset and its eventual disposition. If the asset is considered to be impaired, the amount of any impairment is measured as the difference between the carrying value and the fair value of the impaired asset. As of December 31, 2020, no impairment of any long-lived assets was identified.

### ***Fair Value Measurements***

The carrying amounts for financial instruments consisting of cash and cash equivalents, accounts receivable, accounts payable and accrued liabilities approximate fair value due to their short maturities. Marketable securities are stated at their estimated fair values. The free shares asset or liability is measured using a binomial-lattice pricing model and is reviewed each reporting period and adjusted, as needed, and is expected to approximate fair value.

### ***Cash, Cash Equivalents and Restricted Cash***

Sangamo considers all highly-liquid investments purchased with original maturities of three months or less at the purchase date to be cash equivalents. Cash and cash equivalents consist of cash, deposits in demand money market accounts, and commercial paper. Restricted cash consists of a letter of credit for \$1.5 million as a deposit for the Brisbane lease.

A reconciliation of cash, cash equivalents and restricted cash reported within the accompanying Consolidated Balance Sheets to the amounts reported within the accompanying Consolidated Statements of Cash Flows is as follows (in thousands):

	As of December 31,		
	2020	2019	2018
Cash and cash equivalents	\$ 131,329	\$ 80,428	\$ 140,418
Non-current restricted cash	1,500	1,500	3,500
Cash, cash equivalents and restricted cash as reported within the Consolidated Statements of Cash Flows	<u>\$ 132,829</u>	<u>\$ 81,928</u>	<u>\$ 143,918</u>

### ***Marketable Securities***

Sangamo classifies its marketable securities as available-for-sale and records its investments at estimated fair value based on quoted market prices or observable market inputs of almost identical assets, with the unrealized holding gains and losses included in accumulated other comprehensive loss ("AOCI"). The Company classifies those investments that are not required for use in current operations and that mature in more than 12 months as non-current marketable securities in the accompanying Consolidated Balance Sheets.

The Company's investments are subject to a periodic impairment review. The Company recognizes an impairment charge, if material, when a decline in the fair value of its investments below the cost basis is judged to be other-than-temporary. The Company considers various factors in determining whether to recognize an impairment charge, including the length of time and extent to which the fair value has been less than the Company's cost basis, the financial condition and near-term prospects of the investee and the Company's intent and ability to hold the investment for a period of time sufficient to allow for any anticipated recovery in the market value. Realized gains and losses on marketable securities are included in interest and other income, net, which are determined using the specific identification method.

### ***Concentrations of Credit Risk and Other Risks***

Cash, cash equivalents, and marketable securities consist of financial instruments that potentially subject the Company to a concentration of credit risk to the extent of the fair value recorded in the Consolidated Balance Sheets. The Company invests cash that is not required for immediate operating needs primarily in highly liquid instruments with high credit ratings which are expected to bear minimal risk. The Company has established policies relating to the quality, diversification, and maturities of securities to enable the Company to manage its credit risk. The Company is exposed to credit risk in the event of a default by the financial institutions holding its cash, cash equivalents, and marketable securities and issuers of investments to the extent recorded on the Consolidated Balance Sheets.

Certain materials and key components that the Company utilizes in its operations are obtained through single suppliers. Since the suppliers of key components and materials must be named in an investigational new drug ("IND") application filed with the U.S. Food and Drug Administration for a product, significant delays can occur if the qualification of a new supplier is required. If delivery of material from the Company's suppliers was interrupted for any reason, the Company may be unable to supply any of its product candidates for clinical trials.

### ***Property and Equipment***

Property and equipment are stated at cost, less accumulated depreciation and amortization. Depreciation is calculated using the straight-line method based on the estimated useful lives of the related assets which is generally three to five years. For leasehold improvements, amortization is calculated using the straight-line method based on the shorter of the useful life or the lease term. The Company reviews its property and equipment for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable.

### ***Research and Development Expenses***

Research and development expenses consist primarily of personnel costs, including salaries, benefits and stock-based compensation, clinical studies performed by contract research organizations, materials and supplies and overhead allocations consisting of various support and facility-related costs. Research and development costs are expensed as incurred.

### ***General and Administrative Expenses***

General and administrative expenses consist of finance, human resources, legal and other administrative activities. These expenses consist primarily of personnel costs, including salaries, benefits and stock-based compensation, facilities and overhead costs, legal expenses, and other general and administrative costs.

### ***Stock-based Compensation***

The Company measures and recognizes compensation expense for all stock-based payment awards made to Sangamo employees and directors, including employee share options, restricted stock units (“RSUs”) and employee stock purchases related to the Employee Stock Purchase Plan (“ESPP”) based on estimated fair values at the award grant date. The fair value of stock-based awards is amortized over the vesting period of the award using a straight-line method.

To estimate the fair value of an award, the Company uses the Black-Scholes option pricing model. This model requires inputs such as expected life, expected volatility and risk-free interest rate. These inputs are subjective and generally require significant analysis and judgment to develop. While estimates of expected life and volatility are derived primarily from the Company’s historical data, the risk-free rate is based on the U.S. Treasury yield curve in effect at the time of grant commensurate with the expected life assumption. The Company accounts for forfeitures in the period they occur.

### ***Income Taxes***

Income tax expense has been provided using the liability method. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities as measured by the enacted tax rates that will be in effect when these differences reverse. The Company provides a valuation allowance against net deferred tax assets if, based upon the available evidence, it is not more likely than not that the deferred tax assets will be realized.

The Company recognizes a tax benefit from an uncertain tax position only if it is more likely than not that the tax position will be sustained on examination by the taxing authorities, based on the technical merits of the position. The tax benefits recognized in the Company’s Consolidated Financial Statements from such positions are measured based on the largest benefit that has a greater than 50% likelihood of being realized. The Company recognizes interest and penalties associated with tax matters as part of the income tax provision and includes accrued interest and penalties with the related income tax liability within other accrued liabilities on its Consolidated Balance Sheets.

### ***Leases***

The Company adopted the Accounting Standard Update (“ASU”) No. 2016-02, *Leases (Topic 842)* on January 1, 2019, using the modified retrospective approach with a cumulative-effect adjustment. The Company determines if an arrangement is or contains a lease at inception by assessing whether the arrangement contains an identified asset and whether it has the right to control the identified asset. Right-of-use (“ROU”) assets represent the Company’s right to use an underlying asset for the lease term and lease liabilities represent the Company’s obligation to make lease payments arising from the lease. Lease liabilities are recognized at the lease commencement date based on the present value of future lease payments over the lease term. ROU assets are based on the measurement of the lease liability and also include any lease payments made prior to or on lease commencement and exclude lease incentives and initial direct costs incurred, as applicable.

As the implicit rate in the Company’s leases is generally unknown, the Company uses its incremental borrowing rate based on the information available at the lease commencement date in determining the present value of remaining lease payments. The incremental borrowing rate represents an estimate of the interest rate the Company would incur at lease commencement to borrow an amount equal to the lease payments on a collateralized basis over the term of a lease in a similar economic environment. The Company considers its credit risk, term of the lease, and total lease payments and adjusts for the impacts of collateral, as necessary, when calculating its incremental borrowing rates. The lease terms may include options to extend or terminate the lease when it is reasonably certain the Company will exercise any such options. Rent expense for the Company’s operating leases is recognized on a straight-line basis over the lease term.

The Company has elected to not separate lease and non-lease components for its real estate and copier leases and, as a result, accounts for any lease and non-lease components as a single lease component. The Company has also elected to not apply the recognition requirement to any leases with a term of 12 months or less and does not include an option to purchase the underlying asset that the Company is reasonably certain to exercise.

### ***Foreign Currency Translation***

The functional currency of the Company’s foreign subsidiaries is primarily the Euro. Assets and liabilities denominated in foreign currencies are translated to U.S. dollars using the exchange rates at the balance sheet date. Foreign currency translation adjustments are recorded as a component of AOCI within stockholders’ equity. Revenues and expenses from the Company’s foreign subsidiaries are translated using the monthly average exchange rates in effect during the period in which the transactions occur. Foreign currency transaction gains and losses are recorded in interest and other income, net, on the Company’s Consolidated Statements of Operations.

### **Net Loss Per Share**

Basic net loss per share attributable to Sangamo Therapeutics, Inc. stockholders has been computed by dividing net loss attributable to Sangamo Therapeutics, Inc. stockholders by the weighted-average number of shares of common stock outstanding during the period. Diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders is calculated by dividing net loss attributable to Sangamo Therapeutics, Inc. stockholders by the weighted-average number of shares of common stock plus potentially dilutive securities outstanding during the period.

The total number of shares subject to stock options and RSUs outstanding and the ESPP shares reserved for issuance, which are all anti-dilutive, were excluded from consideration in the calculation of diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders. Stock options and RSUs outstanding and ESPP shares reserved for issuance as of December 31, 2020, 2019 and 2018 were 14,237,871, 10,750,550, and 9,048,793, respectively.

### **Segments**

The Company operates in one segment. Management uses one measure of profitability and does not segregate its business for internal reporting. As of December 31, 2020 and 2019, substantially all of the Company's assets were maintained in the United States. For the years ended December 31, 2020, 2019 and 2018, substantially all of the Company's revenues and operating expenses were generated and incurred in the United States.

### **Recent Accounting Pronouncements**

#### *Recently Adopted*

#### Collaborative Arrangements

In November 2018, the FASB issued ASU No. 2018-18, *Collaborative Arrangements (ASC Topic 808): Clarifying the Interaction between Topic 808 and Topic 606* ("ASU 2018-18"), which clarifies that certain transactions between participants in a collaborative arrangement should be accounted for under ASC Topic 606 when the counterparty is a customer. In addition, ASU 2018-18 precludes an entity from presenting consideration from a transaction in a collaborative arrangement as revenue from contracts with customers if the counterparty is not a customer for that transaction. ASU 2018-18 is effective for all interim and annual reporting periods beginning after December 15, 2019. On January 1, 2020, the Company adopted ASU 2018-18. The adoption of ASU 2018-18 did not have a material impact on the Company's Consolidated Financial Statements.

#### Goodwill Impairment Testing

In January 2017, the FASB issued ASU No. 2017-04, *Intangibles – Goodwill and Other (Topic 350): Simplifying the Test of Goodwill Impairment* ("ASU 2017-04"). The new guidance simplifies the subsequent measurement of goodwill by eliminating Step 2 from the goodwill impairment test. ASU 2017-04 requires goodwill impairment to be measured as the amount by which a reporting unit's carrying amount exceeds its fair value, not to exceed the carrying amount of its goodwill. ASU 2017-04 requires prospective application and is effective for annual periods beginning after December 15, 2019. ASU 2017-04 required the Company to amend its methodology for determining any goodwill impairment beginning in 2020. On January 1, 2020, the Company adopted ASU 2017-04. The adoption of ASU 2017-04 did not have a material impact on the Company's Consolidated Financial Statements.

#### Credit Losses

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments – Credit Losses (Topic 326)* ("ASU 2016-13"). ASU 2016-13 implements an impairment model, known as the current expected credit loss model, that is based on expected losses rather than incurred losses. Under the new guidance, an entity will recognize as an allowance its estimate of expected credit losses. ASU 2016-13 is effective for all interim and annual reporting periods beginning after December 15, 2019 and must be adopted using a modified retrospective approach, with certain exceptions. Early adoption is permitted. On January 1, 2020, the Company adopted ASU 2016-13 by using a modified retrospective approach. The adoption of ASU 2016-13 did not have a material impact on the Company's Consolidated Financial Statements.

#### Income Taxes

In December 2019, the FASB issued ASU No. 2019-12, *Income Taxes – Simplifying the Accounting for Income Taxes* ("ASU 2019-12"). The guidance removes exceptions to the general principles in Income Taxes (Topic 740) for allocating tax expense between financial statement components, accounting basis differences stemming from an ownership change in foreign investments and interim period income tax accounting for year-to-date losses that exceed projected losses. The guidance becomes effective for annual reporting periods beginning after December 15, 2020 and interim periods within those fiscal years with early adoption permitted. On January 1, 2020, the Company early adopted ASU 2019-12. The adoption of ASU 2019-12 did not have a material impact on the Company's Consolidated Financial Statements.

### Cloud Computing Arrangements

In August 2018, the FASB issued ASU 2018-15, *Intangibles-Goodwill and Other-Internal Use Software: Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract* ("ASU 2018-15"). ASU 2018-15 aligns the requirements for capitalizing implementation costs incurred in a cloud computing arrangement that is a service contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal-use software. The guidance becomes effective for annual reporting periods beginning after December 15, 2019 and interim periods within those fiscal years with early adoption permitted. The Company adopted this standard on January 1, 2020 using the prospective method. The adoption of ASU 2018-15 did not have a material impact on the Company's Consolidated Financial Statements.

### NOTE 2 – FAIR VALUE MEASUREMENT

The Company measures certain assets and liabilities at fair value on a recurring basis, including cash equivalents, marketable securities and the free shares asset. The accounting guidance establishes a three-tier hierarchy, which prioritizes the inputs used in the valuation methodologies in measuring fair value as follows:

Level 1: Unadjusted quoted prices in active markets that are accessible at the measurement date for identical, unrestricted assets or liabilities;

Level 2: Quoted prices in markets that are not active, or inputs which are observable, either directly or indirectly, for substantially the full term of the asset or liability; and

Level 3: Prices or valuation techniques that require inputs that are both significant to the fair value measurement and unobservable (*i.e.*, supported by little or no market activity).

The fair value measurements of the Company's cash equivalents, marketable securities and the free shares asset are identified at the following levels within the fair value hierarchy (in thousands):

	December 31, 2020			
	Fair Value Measurement			
	Total	Level 1	Level 2	Level 3
<b>Assets:</b>				
Cash equivalents:				
Money market funds	\$ 53,165	\$ 53,165	\$ —	\$ —
Total	53,165	53,165	—	—
Marketable securities:				
Commercial paper	213,533	—	213,533	—
Corporate debt securities	59,574	—	59,574	—
Certificates of deposit	12,311	—	12,311	—
Asset-backed securities	17,908	—	17,908	—
U.S. government-sponsored entity debt securities	257,298	—	257,298	—
Total	560,624	—	560,624	—
Total cash equivalents and marketable securities	\$ 613,789	\$ 53,165	\$ 560,624	\$ —
Free shares asset	\$ 70	\$ —	\$ —	\$ 70

	December 31, 2019			
	Fair Value Measurement			
	Total	Level 1	Level 2	Level 3
<b>Assets:</b>				
Cash equivalents:				
Money market funds	\$ 30,496	\$ 30,496	\$ —	\$ —
Commercial paper	2,999	—	2,999	—
Total	33,495	30,496	2,999	—
Marketable securities:				
Commercial paper	155,368	—	155,368	—
Corporate debt securities	95,017	—	95,017	—
U.S. government-sponsored entity debt securities	53,493	—	53,493	—
Total	303,878	—	303,878	—
Total cash equivalents and marketable securities	\$ 337,373	\$ 30,496	\$ 306,877	\$ —
Free shares asset	\$ 236	\$ —	\$ —	\$ 236

### **Cash Equivalents and Marketable Securities**

The Company generally classifies its marketable securities and some cash equivalents as Level 2. Instruments are classified as Level 2 when observable market prices for identical securities that are traded in less active markets are used. When observable market prices for identical securities are not available, such instruments are priced using benchmark curves, benchmarking of like securities, sector groupings, matrix pricing and valuation models. These valuation models are proprietary to the pricing providers or brokers and incorporate a number of inputs, including in approximate order of priority: benchmark yields, reported trades, broker/dealer quotes, issuer spreads, two-sided markets, benchmark securities, bids, offers and reference data including market research publications. For certain security types, additional inputs may be used, or some of the standard inputs may not be applicable. Evaluators may prioritize inputs differently on any given day for any security based on market conditions, and not all inputs listed are available for use in the evaluation process for each security evaluation on any given day.

### **Free Shares Asset**

As a result of the July 20, 2018 Share Purchase Agreement (“Sangamo France SPA”) to acquire Sangamo France (see Note 5 – *Acquisition of Sangamo France*), the Company entered into arrangements with the holders of approximately 477,000 “free shares” of Sangamo France pursuant to which the Company has the right to purchase such shares from the holders (a call option) and such holders have the right to sell to the Company such shares from time to time through mid-2021 (a put option). The Company initially recorded a liability of \$0.2 million on the acquisition date. The put options were classified within Level 3 of the fair value hierarchy as the Company utilized a binomial-lattice pricing model (the “Monte Carlo simulation model”) that involved certain market conditions to estimate the fair value of the options. The assumptions used in this simulation model are reviewed on a quarterly basis and adjusted, as needed. Subsequent changes in the fair value of the free shares are recorded in general and administrative expenses in the Consolidated Statements of Operations. The Company purchased approximately 111,000 and 322,000 shares during 2019 and 2020 respectively, of the 477,000 total free shares for a cash payment of approximately \$0.3 million and \$0.7 million respectively, upon exercise of the put options. As of December 31, 2020, approximately 44,000 free shares remain outstanding and subject to purchase by the Company.

The fair value of the free shares’ asset was approximately \$0.2 million at December 31, 2019. The Company recognized a gain due to an increase in the fair value of the free shares of approximately \$0.1 million for the year ended December 31, 2020, offset by approximately \$0.2 million for the shares purchased during the year, resulting in an asset balance of approximately \$0.1 million at December 31, 2020.

Free Shares valuation assumptions:	December 31,	
	2020	2019
Sangamo stock price (USD)	\$ 15.61	\$ 8.68
Sangamo France stock price (EUR)	€ 3.85	€ 2.14
EUR/ USD exchange rate	0.82	0.91
Estimated correlation Sangamo and Sangamo France stock prices	100.0 %	100.0 %
Sangamo stock price (USD) volatility estimate	88.9 %	72.5 %
Sangamo France stock price (EUR) volatility estimate	88.9 %	72.5 %
EUR/ USD exchange rate volatility estimate	6.3 %	6.6 %
Risk free rate and cost of debt by expected exercise date	Varies	Varies

**NOTE 3 – CASH EQUIVALENTS AND MARKETABLE SECURITIES**

The table below summarizes the Company's cash equivalents and marketable securities (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
<b>December 31, 2020</b>				
Cash equivalents:				
Money market funds	\$ 53,165	\$ —	\$ —	\$ 53,165
Total	53,165	—	—	53,165
Marketable securities:				
Commercial paper	213,500	41	(8)	213,533
Corporate debt securities	59,575	16	(17)	59,574
Certificates of deposit	12,311	—	—	12,311
Asset-backed securities	17,905	10	(7)	17,908
U.S. government-sponsored entity debt securities	257,284	19	(5)	257,298
Total	560,575	86	(37)	560,624
Total cash equivalents and marketable securities	\$ 613,740	\$ 86	\$ (37)	\$ 613,789
<b>December 31, 2019</b>				
Cash equivalents:				
Money market funds	\$ 30,496	\$ —	\$ —	\$ 30,496
Commercial paper	2,998	1	—	2,999
Total	33,494	1	—	33,495
Marketable securities:				
Commercial paper	155,230	145	(7)	155,368
Corporate debt securities	94,905	115	(3)	95,017
U.S. government-sponsored entity debt securities	53,411	91	(9)	53,493
Total	303,546	351	(19)	303,878
Total cash equivalents and marketable securities	\$ 337,040	\$ 352	\$ (19)	\$ 337,373

The fair value of marketable securities by contractual maturity were as follows (in thousands):

	December 31,	
	2020	2019
Maturing in one year or less	\$ 510,094	\$ 282,046
Maturing after one year through five years	50,530	21,832
Total	\$ 560,624	\$ 303,878

The Company had no realized losses from the sale of marketable securities for the years ended December 31, 2020, 2019 or 2018. No investments were other-than-temporarily impaired at either December 31, 2020 or 2019. The Company considers factors such as the duration, the magnitude and the reason for the decline in value, the potential recovery period, creditworthiness of the issuers of the securities and its intent to sell. For marketable securities, it also considers whether (i) it is more likely than not that the Company will be required to sell the debt securities before recovery of their amortized cost basis, and (ii) the amortized cost basis cannot be recovered as a result of credit losses. No significant facts or circumstances have arisen to indicate that there has been any significant deterioration in the creditworthiness of the issuers of the securities held by the Company. Based on the Company's review of these securities, including the assessment of the duration and severity of the unrealized losses and the Company's ability and intent to hold the investments until maturity, there were no other-than-temporary impairments for these marketable securities at December 31, 2020.



**NOTE 4 – MAJOR CUSTOMERS, PARTNERSHIPS AND STRATEGIC ALLIANCES****Collaboration Agreements*****Novartis Institutes for BioMedical Research, Inc.***

On July 27, 2020, the Company entered into a collaboration and license agreement with Novartis Institutes for BioMedical Research, Inc. (“Novartis”) for the research, development and commercialization of gene regulation therapies to treat three neurodevelopmental disorders. Under the agreement, which was effective upon execution, the Company granted Novartis an exclusive, royalty bearing and worldwide license, under its relevant patents and know-how, to develop, manufacture and commercialize certain of its zinc finger protein (“ZFP”) transcription factors (“ZFP-TFs”) targeted to three undisclosed genes that are associated with certain neurodevelopmental disorders, including autism spectrum disorder and intellectual disability. The Company performs early research activities over the collaboration period for each gene target and manufacture the ZFP-TFs required for such research, costs of which will be funded by Novartis. Novartis is responsible for additional research activities, investigational new drug-enabling studies, clinical development, regulatory approvals, manufacturing of preclinical, clinical and approved products, and global commercialization. Subject to certain exceptions set forth in the agreement, the Company is prohibited from developing, manufacturing or commercializing any therapeutic product targeting any of the three genes that are the subject of the collaboration. Novartis also has the option to license certain of the Company’s proprietary adeno-associated viruses (“AAVs”) for the sole purpose of developing, manufacturing and commercializing licensed products arising from the collaboration.

Under the agreement, Novartis paid the Company a \$75.0 million upfront license fee in August 2020. In addition to this fee and the cost reimbursements for early research activities, the Company is eligible to earn from Novartis up to \$420.0 million in development milestones and up to \$300.0 million in commercial milestones. The Company is also eligible to earn from Novartis tiered high single-digit to sub-teen double-digit royalties on potential net commercial sales of licensed products arising from the collaboration. These royalty payments will be subject to reduction due to patent expiration, loss of market exclusivity and payments made under certain licenses for third-party intellectual property. The agreement will continue, on a product-by-product and country-by-country basis, until the expiration of the applicable royalty term. Novartis has the right to terminate the agreement, in its entirety or on a target-by-target basis, for any reason after a specified notice period. Each party also has the right to terminate the agreement on account of the other party’s bankruptcy or material, uncured breach.

All payments received under the agreement, when earned, are non-refundable and non-creditable. The transaction price of \$95.1 million includes the upfront license fee of \$75.0 million and estimated research costs of \$20.1 million for identified research projects over the estimated research period. None of the development and commercial milestones have been included in the transaction price, as all such amounts are fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones at this time is uncertain and contingent upon future periods when the uncertainty related to the variable consideration is resolved. The Company will re-evaluate the transaction price, including the estimated variable consideration included in the transaction price and all constrained amounts, in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

The Company assessed the agreement with Novartis in accordance with ASC Topic 606 and concluded that Novartis is a customer. The Company has identified a single performance obligation within this arrangement as a license to the technology and ongoing research services. The Company concluded that the license is not discrete as it does not have stand-alone value to Novartis apart from the research services to be performed pursuant to the agreement. As a result, the Company recognizes revenue from the upfront payment based on proportional performance of the ongoing research services through the estimated research period. The estimation of progress towards the satisfaction of performance obligation and project cost is reviewed quarterly and adjusted, as needed, to reflect the Company’s current assumptions regarding the timing of its performance obligation. As of December 31, 2020, the Company had deferred revenue of \$70.9 million related to the upfront license fee received.

The Company recognized \$4.1 million of upfront license fee and \$1.1 million research reimbursement costs as revenue related to the Novartis agreement during the year ended December 31, 2020.

The Company paid \$1.5 million for financial advisory fees related to the Novartis collaboration and license agreement during the year ended December 31, 2020, equal to 2% of \$75.0 million received for the upfront license fee related to the collaboration and license agreement with Novartis. The Company recognized this \$1.5 million as a contract asset as such amount represents a cost of obtaining the agreement. This balance will be amortized and included in general and administrative costs on a systematic basis consistent with the transfer of the services to Novartis in accordance with ASC Topic 340, Other Assets and Deferred Costs. The Company amortized \$0.1 million during the year ended December 31, 2020.

***Biogen MA, Inc.***

In February 2020, the Company entered into a collaboration and license agreement with Biogen MA, Inc. (“BIMA”) and Biogen International GmbH (together with BIMA, “Biogen”) for the research, development and commercialization of gene

regulation therapies for the treatment of neurological diseases. The Company and Biogen plan to leverage the Company's proprietary ZFP technology delivered via AAV to modulate expression of key genes involved in neurological diseases. Concurrently with the execution of the collaboration agreement, the Company entered into a stock purchase agreement with BIMA, pursuant to which BIMA agreed to purchase 24,420,157 shares of the Company's common stock (the "Biogen Shares"), at a price per share of \$9.2137, for an aggregate purchase price of approximately \$225.0 million.

The collaboration agreement became effective in April 2020 following the termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and satisfaction of other customary closing conditions, including the payment of \$225.0 million for the purchase of the Biogen Shares.

Under the collaboration agreement, Biogen paid the Company an upfront license fee of \$125.0 million in May 2020. The Company is also eligible to receive research, development, regulatory and commercial milestone payments that could total up to approximately \$2.37 billion if Biogen selects all of the targets allowed under the agreement and all the specified milestones set forth in the agreement are achieved, which includes up to \$925.0 million in pre-approval milestone payments and up to \$1.45 billion in first commercial sale and other sales-based milestone payments. In addition, the Company is also eligible to receive tiered high single-digit to sub-teen royalties on potential net commercial sales of licensed products arising from the collaboration. These royalty payments are subject to reduction due to patent expiration, entry of biosimilar products to the market and payments made under certain licenses for third-party intellectual property.

Under the collaboration agreement, the Company granted to Biogen an exclusive, royalty bearing and worldwide license, under its relevant patents and know-how, to develop, manufacture and commercialize certain ZFP and/or AAV-based products directed to up to 12 neurological disease gene targets selected by Biogen. Biogen has already selected three of these: ST-501 for tauopathies including Alzheimer's disease, ST-502 for synucleinopathies including Parkinson's disease, and a third undisclosed neuromuscular disease target. Biogen has exclusive rights to nominate up to nine additional targets over a target selection period of five years. For each gene target selected by Biogen, the Company performs early research activities, costs for which are shared by the companies, aimed at the development of the combination of proprietary central nervous system delivery vectors and ZFP-TFs (or potential other ZFP products) targeting therapeutically relevant genes. Biogen has assumed responsibility and costs for the IND enabling studies, clinical development, related regulatory interactions, and global commercialization. The Company is responsible for manufacturing activities for the initial clinical trials for the first three products of the collaboration and plans to leverage its in-house manufacturing capacity, where appropriate, which is currently in development. Biogen is responsible for manufacturing activities beyond the first clinical trial for each of the first three products. The Company's research activities for any targets will be performed over the period not to exceed seven years from the effective date of the agreement (i.e., through April 2027). Subject to certain exceptions set forth in the collaboration agreement, the Company is prohibited from developing, manufacturing or commercializing any therapeutic product directed to the targets selected by Biogen.

The collaboration agreement continues on a product-by-product and country-by-country basis until the expiration of all applicable royalty terms. Biogen has the right to terminate the collaboration agreement, in its entirety or on target-by-target basis, for any reason after a specified notice period, and also has the right to replace up to 10 targets. Each party has the right to terminate this agreement on account of the other party's bankruptcy or material, uncured breach. In addition, the Company may terminate the collaboration agreement if Biogen challenges any patents licensed by the Company to Biogen.

Pursuant to the terms of the stock purchase agreement, Biogen has agreed not to, without the Company's prior written consent and subject to specified conditions and exceptions, directly or indirectly acquire shares of the Company's outstanding common stock, seek or propose a tender or exchange offer or merger between the parties, solicit proxies or consents with respect to any matter, or undertake other specified actions related to the potential acquisition of additional equity interests in the Company. Such standstill restrictions expire on the earlier of the three-year anniversary of the effectiveness of the collaboration agreement and the date that Biogen beneficially owns less than 5% of the Company's common stock.

The stock purchase agreement also provides that until the first anniversary of the effectiveness of the collaboration agreement, Biogen will hold and not sell any of the Biogen Shares and from the first anniversary through the second anniversary, Biogen will hold and not sell at least 50% of the Biogen Shares, in addition to being subject to certain volume limitations. The stock purchase agreement further provides that, subject to certain limitations, until such time as all remaining Biogen Shares may be sold pursuant to Rule 144 promulgated under the Securities Exchange Act of 1933, as amended, within a 90-day period, Biogen may request the Company to register for resale any of the Biogen Shares on a registration statement to be filed with the Securities and Exchange Commission.

In addition, Biogen has agreed that, excluding specified extraordinary matters, it will vote the Biogen Shares in accordance with the Company's recommendation and has granted the Company an irrevocable proxy with respect to the foregoing. Such voting provisions expire on the earlier of (i) the two-year anniversary of the effectiveness of the collaboration agreement, (ii) the date that Biogen beneficially owns less than 5% of the Company's common stock and (iii) the date the

collaboration agreement is terminated; provided, however, that in no event shall such expiration date be prior to the one-year anniversary of the effectiveness of the collaboration agreement.

The Company assessed the collaboration agreement with Biogen in accordance with ASC Topic 606 and concluded that Biogen is a customer. As of December 31, 2020, the transaction price includes the upfront license fee of \$125.0 million and the excess consideration from the stock purchase of \$79.6 million, which represents the difference between the \$225.0 million received for the purchase of the Biogen Shares and the \$145.4 million estimated fair value of the equity issued. The equity issued to Biogen was valued using an option pricing model to reflect certain holding period restrictions. None of the target selection fees and clinical or regulatory milestones have been included in the transaction price, as all such amounts are fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that nomination of additional targets and achievement of the milestones at this time is uncertain and contingent upon future periods when the uncertainty related to the variable consideration is resolved. The Company will re-evaluate the transaction price as uncertain events are resolved or other changes in circumstances occur.

The Company has identified a single performance obligation within the Biogen collaboration agreement, which is a stand-ready obligation consisting of a series of distinct days of research services, during which Biogen obtains access to the Company's license and research resources. Revenue from the upfront license fee relates to access to the license and Company's obligation to stand-ready to perform such research services corresponding to the targets selected by Biogen. As a result of this obligation to perform research services when and if requested throughout the duration of the contract, the upfront license fee and the excess consideration from the stock purchase will be recognized over time on a straight-line basis consistent with the resources expected to be dedicated to providing the research services through April 2027, the estimated period of the obligation. The estimated period of performance is reviewed quarterly and adjusted, as needed, to reflect the Company's current assumptions regarding the timing of its deliverable. Revenue from the reimbursement by Biogen of shared costs of early research activities performed by Sangamo is recognized as the research services are performed. As of December 31, 2020, the Company had deferred revenue of \$183.2 million related to this agreement.

The Company recognized \$21.4 million of upfront license fee and the excess consideration from the stock purchase, and \$6.5 million research reimbursement costs as revenue related to the Biogen agreement during the year ended December 31, 2020.

The Company paid \$7.0 million for financial advisory fees during the year ended December 31, 2020, equal to 2% of \$225.0 million received for the sale of shares and 2% of \$125.0 million received for the upfront fee. The fees incurred related to both the collaboration agreement with Biogen and to the stock purchase agreement for the sale of shares. The Company believes that the allocation of fees on a relative fair value basis between the two agreements is reasonable. The Company recognized \$4.1 million, which represents 2% of the upfront license fee of \$125.0 million and 2% of the excess consideration from the stock purchase of \$79.6 million, as a contract asset. This balance will be amortized and included in general and administrative costs on a systematic basis consistent with the transfer of the services to Biogen in accordance with ASC Topic 340, Other Assets and Deferred Costs. The Company amortized \$0.4 million during the year ended December 31, 2020. The Company recognized \$2.9 million, which represents 2% of the \$145.4 million estimated fair value of the equity issued, as a share issuance cost and recorded this amount in equity as reduction in proceeds during the year ended December 31, 2020.

#### ***Kite Pharma, Inc.***

In February 2018, the Company entered into a global collaboration and license agreement with Kite Pharma, Inc. ("Kite"), which became effective on April 5, 2018, and was amended and restated in September 2019, for the research, development and commercialization of potential engineered cell therapies for cancer. In this collaboration, Sangamo is working together with Kite on a research program under which the companies are designing zinc finger nucleases ("ZFNs") and viral vectors to disrupt and insert certain genes in T cells and natural killer cells ("NK-cells") including the insertion of genes that encode chimeric antigen receptors ("CARs"), T cell receptors ("TCRs"), and NK-cell receptors ("NKRs") directed to mutually agreed targets. Kite is responsible for all clinical development and commercialization of any resulting products.

Subject to the terms of this agreement, the Company granted Kite an exclusive, royalty-bearing, worldwide sublicensable license under the Company's relevant patents and know-how to develop, manufacture and commercialize, for the purpose of treating cancer, specific cell therapy products that may result from the research program and that are engineered *ex vivo* using selected ZFNs and viral vectors developed under the research program to express CARs, TCRs or NKRs directed to candidate targets.

During the research program term and subject to certain exceptions, except pursuant to this agreement, the Company is prohibited from researching, developing, manufacturing and commercializing, for the purpose of treating cancer, any cell therapy product that, as a result of *ex vivo* genome editing, expresses a CAR, TCR or NKR that is directed to a target expressed on or in a human cancer cell. After the research program term concludes and subject to certain exceptions, except pursuant to this agreement, the Company will be prohibited from developing, manufacturing and commercializing, for the purpose of

treating cancer, any cell therapy product that, as a result of *ex vivo* genome editing, expresses a CAR, TCR or NKR that is directed to a candidate target.

Following the effective date, in April 2018, the Company received a \$150.0 million upfront payment from Kite. In addition, Kite will reimburse the Company's direct costs to conduct service under the joint research program provisions of the agreement, and Kite will be responsible for all subsequent development, manufacturing and commercialization of any licensed products. Sangamo is also eligible to receive contingent development- and sales-based milestone payments that could total up to \$3.01 billion if all of the specified milestones in this agreement are achieved. Of this amount, approximately \$1.26 billion relates to the achievement of specified research, clinical development, regulatory and first commercial sale milestones, and approximately \$1.75 billion relates to the achievement of specified sales-based milestones if annual worldwide net sales of licensed products reach specified levels. Each development- and sales-based milestone payment is payable (i) only once for each licensed product, regardless of the number of times that the associated milestone event is achieved by such licensed product, and (ii) only for the first 10 times that the associated milestone event is achieved, regardless of the number of licensed products that may achieve such milestone event. In addition, the Company will be entitled to receive escalating, tiered royalty payments with a percentage in the single digits based on potential future annual worldwide net sales of licensed products. These royalty payments will be subject to reduction due to patent expiration, entry of biosimilar products to the market and payments made under certain licenses for third-party intellectual property.

The initial research term of the agreement is six years. Kite has an option to extend the research term for up to two additional one-year periods for a separate fee of \$10.0 million per year. All contingent payments under the agreement, when earned, will be non-refundable and non-creditable. In connection with the amendment and restatement of the agreement in September 2019, the Company entered into a new research plan with Kite, with estimated reimbursable service costs of approximately \$3.4 million. The Company concluded the transaction price under this agreement is \$189.3 million and includes the upfront license fee of \$150.0 million and \$39.3 million estimated reimbursable service costs for identified research projects over the estimated performance period. The Company concluded that the estimated fees for the presumed exercise of the research term extension options and all milestone amounts are fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones at this time is uncertain and contingent upon future periods when the uncertainty related to the variable consideration is resolved. The Company will re-evaluate the transaction price, including the estimated variable consideration included in the transaction price and all constrained amounts, in each reporting period and as uncertain events are resolved or other changes in circumstances occur. None of the development and sales-based milestone payments have been included in the transaction price.

The Company assessed the agreement with Kite in accordance with ASC Topic 606 and concluded that Kite is a customer. Kite has the right to terminate this agreement, in its entirety or on a per licensed product or per candidate target basis, for any reason after a specified notice period. Each party has the right to terminate this agreement on account of the other party's bankruptcy or material, uncured breach.

The Company has identified the primary performance obligations within the Kite agreement as: (1) a license to the technology along with the stand-ready obligation to perform research services, and (2) the ongoing research services. Revenue from the upfront license fee relates to access to the license and Company's obligation to stand-ready to perform such research services as additional targets are selected by Kite. As a result of this obligation to perform research services when and if requested throughout the duration of the contract, the fee for the license and the stand-ready obligation will be recognized over time on a straight-line basis through June 2024, the estimated period of the stand-ready obligation. Revenue from the reimbursable costs related to the integrated service deliverable is recognized as the research services are performed. Related costs and expenses under these arrangements have historically approximated the revenues recognized. The estimated period of performance is reviewed quarterly and adjusted, as needed, to reflect the Company's current assumptions regarding the timing of its deliverables. As of December 31, 2020, and 2019 the Company had deferred revenue of \$81.4 million and \$106.5 million, respectively, related to this agreement.

Revenues recognized under the agreement were as follows (in thousands):

	Year Ended December 31,		
	2020	2019	2018
Revenue related to Kite agreement:			
Recognition of license and stand-ready fee	\$ 25,046	\$ 24,977	\$ 18,545
Research services	3,562	9,373	6,972
Total	\$ 28,608	\$ 34,350	\$ 25,517

**Pfizer Inc.**

*Giroctocogene Fitelparvovec Global Collaboration and License Agreement*

In May 2017, the Company entered into an exclusive, global collaboration and license agreement with Pfizer, pursuant to which it established a collaboration for the research, development and commercialization of giroctocogene fitelparvovec, its gene therapy product candidate for hemophilia A, and closely related products.

Under this agreement, the Company is responsible for conducting the Phase 1/2 clinical trial and certain manufacturing activities for giroctocogene fitelparvovec, while Pfizer is responsible for subsequent worldwide development, manufacturing, marketing and commercialization of giroctocogene fitelparvovec. Sangamo may also collaborate in the research and development of additional AAV based gene therapy products for hemophilia A.

The Company originally received an upfront fee of \$70.0 million and is eligible to receive up to \$208.5 million in payments upon the achievement of specified clinical development, intellectual property and regulatory milestones and up to \$266.5 million in payments upon first commercial sale milestones for giroctocogene fitelparvovec and potentially other products. The total amount of potential clinical development, intellectual property, regulatory, and first commercial sale milestone payments, assuming the achievement of all specified milestones in the giroctocogene fitelparvovec Pfizer agreement, is up to \$475.0 million, which includes up to \$300.0 million for giroctocogene fitelparvovec and up to \$175.0 million for other products that may be developed under the agreement, subject to reduction on account of payments made under certain licenses for third party intellectual property. In addition, Pfizer agreed to pay the Company royalties for each potential licensed product developed under the agreement based on an escalating tiered, double-digit percentage of the annual net sales of such product. These royalties are subject to reduction due to patent expiration, entry of biosimilar products to the market and payment made under certain licenses for third-party intellectual property. To date, two milestones of \$55.0 million in aggregate have been achieved and paid, however no products have been approved and therefore no royalty fees have been earned under the giroctocogene fitelparvovec Pfizer agreement.

The Company assessed the agreement with Pfizer in accordance with ASC Topic 606 and concluded that Pfizer is a customer. As of December 31, 2020, the total transaction price under this agreement is \$134.0 million, which represents the upfront and research services fees of \$79.0 million and two unconstrained milestones achieved of an aggregate amount of \$55.0 million. Sangamo is responsible for internal and external research costs as part of the upfront fee and has the ability to request additional reimbursement from Pfizer if certain conditions are met. None of the constrained clinical or regulatory milestones have been included in the transaction price, as all such milestone amounts are fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones at this time is uncertain and contingent upon future periods when the uncertainty related to the variable consideration is resolved. The Company will re-evaluate the transaction price, including its estimated variable consideration included in the transaction price and all constrained amounts in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

Subject to the terms of the agreement, the Company granted Pfizer an exclusive worldwide royalty-bearing license, with the right to grant sublicenses, to use certain technology controlled by the Company for the purpose of developing, manufacturing and commercializing giroctocogene fitelparvovec and related products. Pfizer granted the Company a non-exclusive, worldwide, royalty free, fully paid license, with the right to grant sublicenses, to use certain manufacturing technology developed under the agreement and controlled by Pfizer to manufacture the Company's products that utilize the AAV delivery system. During a specified period, neither the Company nor Pfizer will be permitted to clinically develop or commercialize, outside of the collaboration, certain AAV-based gene therapy products for hemophilia A.

Unless earlier terminated, the agreement has a term that continues on a per product and per country basis until the later of (i) the expiration of patent claims that cover the product in a country, (ii) the expiration of regulatory exclusivity for a product in a country, and (iii) 15 years after the first commercial sale of a product in a country. Pfizer has the right to terminate the agreement without cause in its entirety or on a per product or per country basis. The agreement may also be terminated by either party based on an uncured material breach by the other party or the bankruptcy of the other party. Upon termination for any reason, the license granted by the Company to Pfizer to develop, manufacture and commercialize giroctocogene fitelparvovec and related products will automatically terminate. Upon termination by the Company for cause or by Pfizer in any country or countries, Pfizer will automatically grant the Company an exclusive, royalty-bearing license under certain technology controlled by Pfizer to develop, manufacture and commercialize giroctocogene fitelparvovec in the terminated country or countries.

The Company has identified one performance obligation within the giroctocogene fitelparvovec Pfizer agreement as a license to the technology and ongoing research services. The Company concluded that the license is not discrete, as it does not have stand-alone value to Pfizer apart from the research services to be performed by the Company pursuant to the agreement. As a result, the Company recognizes revenue from the upfront payment based on proportional performance of the ongoing

services through 2020, the estimated period the Company will perform research services. The estimate of progress towards the satisfaction of its performance obligation and project cost is reviewed quarterly and adjusted, as needed, to reflect the Company's current assumptions regarding the timing of its deliverables. The Company satisfied the deliverables and research services responsibilities within the arrangement which were completed in December 2020. As a result, the Company recognized the remaining deferred revenue from the upfront payment in December 2020. As of December 31, 2019, the Company had deferred revenue of \$4.0 million related to this agreement.

In December 2019, the Company entered into an amendment to the collaboration agreement, pursuant to which the Company transferred the IND for giroctocogene fitelparvec to Pfizer. Upon this transfer the Company achieved a \$25.0 million milestone as the conditions for achieving the milestone were met. The cumulative revenue recognized in connection with this milestone was \$25.0 million as of December 31, 2020 and included \$1.3 million recognized during the year ended December 31, 2020.

In September 2020, the Company determined that there was a high probability of achievement of a \$30.0 million milestone with Pfizer for giroctocogene fitelparvec. The milestone was subsequently achieved upon dosing of the first subject in a Phase 3 clinical trial in early October 2020. The cumulative revenue recognized in connection with this milestone was \$30.0 million during the year ended December 31, 2020.

Revenues recognized under the agreement were as follows (in thousands):

	Year Ended December 31,		
	2020	2019	2018
Revenue related to Pfizer giroctocogene fitelparvec agreement:			
Recognition of upfront fee and research services	\$ 3,111	\$ 15,697	\$ 37,810
Milestone achievement	31,338	23,662	—
Total	\$ 34,449	\$ 39,359	\$ 37,810

In March 2019, the Company updated its estimated project cost and related revenues under this program. This adjustment was a direct result of the increase in project scope during the first quarter of 2019 and the corresponding costs, which resulted in a decrease in the measure of proportional performance. In December 2019, the Company updated its estimated project cost and related revenues upon transfer of the IND for giroctocogene fitelparvec to Pfizer. This adjustment was a direct result of the decrease in project scope during the fourth quarter of 2019 and the corresponding costs, which resulted in an increase in the measure of proportional performance. During the year ended December 31, 2019, the Company recognized \$15.7 million in revenues related to the Pfizer giroctocogene fitelparvec agreement, which included approximately \$8.7 million acceleration in revenues recorded in the three months ended December 31, 2019 related to the updated estimated project cost, offset by approximately \$3.0 million reduction in revenues recorded in the three months ended March 31, 2019 related to the updated estimated project cost.

In March 2020, the Company recorded an adjustment to revenue related to a change in estimate in connection with the giroctocogene fitelparvec collaboration agreement with Pfizer. This adjustment was a direct result of the decision to decrease the project scope and the corresponding costs, after the successful IND transfer of the giroctocogene fitelparvec product candidate to Pfizer, both of which resulted in an increase in the measure of proportional cumulative performance. This adjustment increased revenue by \$2.4 million, decreased net loss by \$2.4 million and decreased the Company's basic net loss per share by \$0.02 for year ended December 31, 2020.

#### C9ORF72 Research Collaboration and License Agreement

In December 2017, the Company entered into a separate exclusive, global collaboration and license agreement with Pfizer for the development and commercialization of potential gene therapy products that use ZFP-TFs to treat amyotrophic lateral sclerosis ("ALS") and frontotemporal lobar degeneration ("FTLD") linked to mutations of the C9ORF72 gene. Pursuant to this agreement, the Company agreed to work with Pfizer on a research program to identify, characterize and preclinically develop ZFP-TFs that bind to and specifically reduce expression of the mutant form of the C9ORF72 gene.

Subject to the terms of this agreement, the Company granted Pfizer an exclusive, royalty-bearing, worldwide license under the Company's relevant patents and know-how to develop, manufacture and commercialize gene therapy products that use resulting ZFP-TFs that satisfy pre-agreed criteria. During a specified period, neither the Company nor Pfizer will be permitted to research, develop, manufacture or commercialize outside of the collaboration any ZFPs that specifically bind to the C9ORF72 gene.

Unless earlier terminated, the agreement has a term that continues on a per licensed product and per country basis until the later of (i) the expiration of patent claims that cover the licensed product in a country, (ii) the expiration of regulatory exclusivity for a licensed product in a country, and (iii) 15 years after the first commercial sale of a licensed product in a major

market country. Pfizer also has the right to terminate the agreement without cause in its entirety or on a per product or per country basis. The agreement may also be terminated by either party based on an uncured material breach by the other party or the bankruptcy of the other party. The agreement will also terminate if the Company is unable to identify any lead candidates for development within a specified period of time or if Pfizer elects not to advance a lead candidate beyond a certain development milestone within a specified period of time. Upon termination for any reason, the license granted by the Company to Pfizer to develop, manufacture and commercialize licensed products under the agreement will automatically terminate. Upon termination by the Company for cause or by Pfizer without cause for any licensed product or licensed products in any country or countries, the Company will have the right to negotiate with Pfizer to obtain a non-exclusive, royalty-bearing license under certain technology controlled by Pfizer to develop, manufacture and commercialize the licensed product or licensed products in the terminated country or countries.

Following termination by the Company for Pfizer's material breach, Pfizer will not be permitted to research, develop, manufacture or commercialize ZFPs that specifically bind to the *C9ORF72* gene for a period of time. Following termination by Pfizer for the Company's material breach, the Company will not be permitted to research, develop, manufacture or commercialize ZFPs that specifically bind to the *C9ORF72* gene for a period of time.

The Company received a \$12.0 million upfront payment from Pfizer and is eligible to receive up to \$60.0 million in development milestone payments from Pfizer contingent on the achievement of specified preclinical development, clinical development and first commercial sale milestones, and up to \$90.0 million commercial milestone payments if annual worldwide net sales of the licensed products reach specified levels. In addition, Pfizer will pay the Company royalties based on an escalating tiered, mid- to high-single digit percentage of the annual worldwide net sales of the licensed products. These royalty payments are subject to reduction due to patent expiration, entry of biosimilar products to the market and payments made under certain licenses for third party intellectual property. Each party will be responsible for the cost of its performance of the research program. Pfizer will be operationally and financially responsible for subsequent development, manufacturing and commercialization of the licensed products. To date, a milestone of \$5.0 million has been achieved and paid, however no products have been approved and therefore no royalty fees have been earned under the *C9ORF72* Pfizer agreement.

The Company assessed the agreement with Pfizer in accordance with ASC Topic 606 and concluded that Pfizer is a customer. The Company concluded the total transaction price under this agreement is \$17.0 million, which represents the upfront fee of \$12.0 million and one unconstrained milestone in the amount of \$5.0 million. None of the constrained clinical or regulatory milestones have been included in the transaction price. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones at this time is uncertain and contingent upon future periods when the uncertainty related to the variable consideration is resolved. The Company will re-evaluate the transaction price, including its estimated variable consideration included in the transaction price and all constrained amounts, in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

The Company has identified the performance obligation within this agreement as a license to the technology and ongoing research services. The Company concluded that the license is not discrete as it does not have stand-alone value to Pfizer apart from the services to be performed by the Company pursuant to the agreement. As a result, the Company recognizes revenue from the upfront payment based on proportional performance of the ongoing services over the estimated period the Company will perform research services. The estimation of progress towards the satisfaction of its performance obligation and project cost is reviewed quarterly and adjusted, as needed, to reflect the Company's current assumptions regarding the timing of its deliverables. The Company satisfied the deliverables and research services responsibilities within the arrangement which were completed in September 2020. As a result, the Company recognized the remaining deferred revenue from the upfront payment in September 2020. As of December 31, 2019, the Company had deferred revenue of \$8.0 million related to this agreement.

In September 2020, the Company earned a \$5.0 million milestone associated with the completion of the Company's research activities in its collaboration with Pfizer to develop genome regulation therapies using ZFP-TFs for the treatment of *C9ORF72*-related ALS and frontotemporal lobar degeneration. This milestone was achieved upon Pfizer's notification to the Company of its election to pay the first development milestone payment under the collaboration agreement. This milestone payment is non-refundable, and the Company recognized on a cumulative basis \$5.0 million for the year ended December 31, 2020.

Revenues recognized under the agreement were as follows (in thousands):

	Year Ended December 31,		
	2020	2019	2018
Revenue related to Pfizer C9ORF72 agreement:			
Recognition of upfront fee	\$ 7,985	\$ 1,827	\$ 2,188
Milestone achievement	5,000	—	—
Total	\$ 12,985	\$ 1,827	\$ 2,188

During the year ended December 31, 2020, the Company recorded adjustments to revenue related to changes in estimate in connection with the C9ORF72 collaboration agreement with Pfizer. These adjustments were a direct result of the decision to decrease the project scope and the corresponding costs due to advancement of the program, which resulted in an increase in the measure of proportional cumulative performance. These adjustments increased revenue by \$8.8 million, decreased net loss by \$8.8 million and decreased the Company's basic net loss per share by \$0.06 for the year ended December 31, 2020.

### Sanofi Genzyme

In January 2014, the Company entered into an exclusive worldwide collaboration and license agreement with Bioverativ Inc., (now Sanofi Genzyme, a global business unit of Sanofi S.A. ("Sanofi")), to develop therapeutics for hemoglobinopathies, focused on beta thalassemia and sickle cell disease ("SCD"). The agreement was originally signed with BIMA, who subsequently assigned it to Bioverativ Inc., which was later acquired by Sanofi. Under the agreement, the Company is jointly conducting two research programs: the beta thalassemia program and the SCD program. In the beta thalassemia program, the Company is responsible for all discovery, research and development activities through the first human clinical trial. In the SCD program, both parties are responsible for research and development activities through the submission of an IND application for ZFP therapeutics intended to treat SCD.

Under both programs, Sanofi is responsible for subsequent worldwide clinical development, manufacturing and commercialization of licensed products developed under the agreement. At the end of the specified research terms for each program or under certain specified circumstances, Sanofi has the right to step in and take over any of the Company's remaining activities. Furthermore, the Company has an option to co-promote in the U.S. any licensed products to treat beta thalassemia and SCD developed under the agreement, and Sanofi will compensate the Company for such co-promotion activities. Subject to the terms of the agreement, the Company has granted Sanofi an exclusive, royalty-bearing license, with the right to grant sublicenses, to use certain ZFP and other technology controlled by the Company for the purpose of researching, developing, manufacturing and commercializing licensed products developed under the agreement. The Company also granted Sanofi a non-exclusive worldwide, royalty-free fully paid license with the right to grant sublicenses, under the Company's interest in certain other intellectual property developed pursuant to the agreement. During the term of the agreement, the Company is not permitted to research, develop, manufacture or commercialize, outside of the agreement, certain gene therapy products that target genes relevant to the licensed products.

Under the agreement, the Company received an upfront license fee of \$20.0 million and is eligible to receive up to \$115.8 million in payments upon the achievement of specified clinical development and regulatory milestones, as well as up to \$160.5 million in payments upon the achievement of specified sales milestones. The total amount of potential regulatory, clinical development, and sales milestone payments, assuming the achievement of all specified milestones in the agreement, is up to \$276.3 million. In addition, the Company will receive royalty payments for each licensed product that are a tiered double-digit percentage of annual net sales of each product. Sanofi reimburses Sangamo for agreed upon costs incurred in connection with research and development activities conducted by Sangamo. To date, a \$6.0 million milestone has been achieved related to ST-400 for beta thalassemia and another \$7.5 million milestone has been achieved related to SCD, however no products have been approved and therefore no royalty fees have been earned under the Sanofi agreement.

The agreement may be terminated by (i) the Company or Sanofi for the uncured material breach of the other party, (ii) the Company or Sanofi for the bankruptcy or other insolvency proceeding of the other party; (iii) Sanofi, upon 180 days' advance written notice to the Company and (iv) Sanofi, for certain safety reasons upon written notice to, and after consultation with, the Company. As a result, actual future milestone payments could be lower than the amounts stated above.

All contingent payments under the agreement, when earned, will be non-refundable and non-creditable. The transaction price as of December 31, 2020 of \$93.3 million includes the upfront license fee of \$20.0 million, two unconstrained milestones of \$13.5 million and estimated research costs of \$59.8 million for identified research projects over the estimated performance period, as all unachieved milestone amounts are fully constrained. As part of its evaluation of the constraint, the Company considered numerous factors, including the fact that achievement of the milestones at this time is uncertain and contingent upon future periods when the uncertainty related to the variable consideration is resolved. The Company will re-



evaluate the transaction price, including the estimated variable consideration included in the transaction price and all constrained amounts, in each reporting period and as uncertain events are resolved or other changes in circumstances occur. None of the constrained clinical or regulatory milestones have been included in the transaction price.

The Company assessed the agreement with Sanofi in accordance with ASC Topic 606 and concluded that Sanofi is a customer. The Company has identified the performance obligations within this arrangement as a license to the technology and ongoing research services activities. The Company concluded that the license is not discrete as it does not have stand-alone value to Sanofi apart from the research services to be performed pursuant to the agreement. As a result, the Company recognizes revenue from the upfront payment based on proportional performance of the ongoing services through 2022, the estimated period the Company will perform research services. The estimate of progress towards the satisfaction of performance obligation and project cost is reviewed quarterly and adjusted, as needed, to reflect the Company's current assumptions regarding the timing of its deliverables. Revenue from the reimbursable costs related to the integrated service deliverable is recognized as the research services are performed. Related costs and expenses under these arrangements have historically approximated the revenues recognized. As of December 31, 2020 and 2019, the Company had deferred revenue of \$1.2 million and \$1.7 million, respectively, related to this agreement.

In August 2019, the Company achieved a \$6.0 million milestone with Sanofi upon dosing of the third subject in the ST-400 beta thalassemia Phase 1 clinical trial. The cumulative revenue recognized in connection with this milestone was approximately \$5.8 million as of December 31, 2020 and included \$0.1 million recognized during the year ended December 31, 2020.

In December 2019, the Company achieved a \$7.5 million milestone with Sanofi upon dosing of the first subject in the SCD Phase 1 clinical trial. The cumulative revenue recognized in connection with this milestone was approximately \$7.2 million as of December 31, 2020 and included \$0.1 million recognized during the year ended December 31, 2020.

Revenues recognized under the agreement were as follows (in thousands):

	Year Ended December 31,		
	2020	2019	2018
Revenue related to Sanofi agreement:			
Recognition of upfront fee	\$ 298	\$ 3,494	\$ 4,013
Research services	4,823	6,367	9,503
Milestone achievement	201	12,819	—
<b>Total</b>	<b>\$ 5,322</b>	<b>\$ 22,680</b>	<b>\$ 13,516</b>

In March 2020, the Company recorded an adjustment to revenue related to a change in estimate in connection with the collaboration agreement with Sanofi. This adjustment was a direct result of the decision in March 2020 to increase the project scope and the corresponding costs, both of which resulted in a decrease in the measure of proportional cumulative performance. This adjustment decreased revenue by \$2.2 million, increased net loss by \$2.2 million and increased the Company's basic net loss per share by \$0.02 for the year ended December 31, 2020.

#### ***California Institute for Regenerative Medicine***

In May 2018, the California Institute for Regenerative Medicine ("CIRM") granted a Strategic Partnership Award for \$8.0 million to fund the clinical studies of a potentially curative ZFP therapeutic for the treatment of beta thalassemia based on the application of Sangamo's ZFN genome editing technology. The grant exists through December 31, 2022 and provides matching funds to support the evaluate ST-400, a gene-edited cell therapy candidate for people with transfusion-dependent beta thalassemia. As of December 31, 2020, the Company had received \$5.2 million under the award.

Under the terms of the CIRM grants, the Company is obligated to pay royalties and licensing fees based on a low single digit royalty percentage on net sales of CIRM-funded product candidates or CIRM-funded technology. The Company has the option to decline any and all amounts awarded by CIRM and as an alternative to revenue sharing, the Company has the option to convert the award to a loan. No such election has been made as of the date of the issuance of these Consolidated Financial Statements. In the event that the Company terminates a CIRM-funded clinical trial, it will be obligated to repay the remaining CIRM funds on hand. As of December 31, 2020 and 2019, \$6.4 million and \$5.7 million, respectively, including accrued interest, related to this award is recorded as a loan in other non-current liabilities on the Consolidated Balance Sheets.

#### ***Amended Collaboration and License Agreement with Takeda***

In January 2012, the Company entered into a collaboration and license agreement with Shire International GmbH, a wholly-owned subsidiary of Takeda Pharmaceutical Company Limited ("Takeda"), to research, develop and commercialize a ZFP therapeutic for treating Huntington's disease. The Company received an upfront license fee of \$13.0 million in 2012 and

recognized a \$1.0 million milestone payment in 2014. Takeda does not have any milestone payment obligations, but is required to pay single digit percentage royalties to the Company, up to a specified maximum cap, on the commercial sales of therapeutic products for Huntington's disease. The Company is required to pay single digit percentage royalties to Takeda, up to a specified maximum cap, on commercial sales of therapeutic products from programs returned under the original agreement (which include blood clotting Factors VIII and IX) that use two zinc fingers.

Pursuant to the agreement, the Company granted Takeda an exclusive, world-wide, royalty-bearing license, with the right to grant sublicenses, to use the Company's ZFP technology for the purpose of developing and commercializing human therapeutic and diagnostic products for the huntingtin gene ("HTT gene"). During the term of the agreement, the Company is not permitted to research, develop or commercialize, outside of the agreement, certain products that target the HTT gene. The agreement may be terminated by (i) the Company or Takeda, in whole or in part, for the uncured material breach of the other party, (ii) the Company or Takeda for the bankruptcy or other insolvency proceeding of the other party and (iii) Takeda, in its entirety, effective upon at least 90 days' advance written notice.

The Company assessed the agreement with Takeda in accordance with ASC Topic 606 and concluded that Takeda is a customer. The Company has concluded that the license is not a separate unit of accounting as it does not have stand-alone value to Takeda apart from the research services to be performed pursuant to the Takeda agreement. The Company satisfied the deliverables and research services responsibilities within the amended arrangement which were completed in 2017. As a result, the Company recognized the remaining \$2.3 million of deferred revenue from the upfront payment during the year ended December 31, 2017.

The Company did not recognize any revenues under the Takeda agreement for the years ended December 31, 2020, 2019 and 2018.

#### ***Agreement with Sigma-Aldrich Corporation***

In 2007, Sangamo entered into a license agreement with Sigma-Aldrich Corporation ("Sigma") to provide Sigma with access to Sangamo's proprietary ZFP technology and the exclusive right to use the technology to develop and commercialize research reagent products and services in the research field, excluding certain agricultural research uses that Sangamo previously licensed to Dow AgroSciences LLC ("DAS"), a wholly-owned subsidiary of Dow Chemical Company. Sangamo developed laboratory research reagents using its ZFP technology over a three-year research services period. Sangamo has since transferred the ZFP manufacturing technology to Sigma.

In October 2009, Sangamo expanded its license agreement with Sigma. In addition to the original terms of the license agreement, Sigma received exclusive rights to develop and distribute ZFP-modified cell lines for commercial production of protein pharmaceuticals and certain ZFP-engineered transgenic animals for commercial applications. Under the terms of the agreement, Sigma made an upfront cash payment of \$20.0 million consisting of a \$4.9 million purchase of 636,133 shares of Sangamo common stock, valued at \$4.9 million, and a \$15.1 million upfront license fee. Sangamo is also eligible to receive commercial license fees of \$5.0 million based upon a percentage of net sales and sublicensing revenue and thereafter a reduced royalty rate of 10.5% of net sales and sublicensing revenue. In addition, upon the achievement of certain cumulative commercial milestones, Sigma will make milestone payments to Sangamo up to an aggregate of \$25.0 million. Sangamo does not have additional ongoing performance obligations under the agreement.

Revenues recognized under the agreement with Sigma for the years ended December 31, 2020, 2019 and 2018, were \$0.5 million, \$0.6 million and \$0.5 million, respectively.

#### ***Agreement with DAS***

In 2005, Sangamo entered into an exclusive commercial license with DAS, with an initial three-year research term. Under this agreement, Sangamo is providing DAS with access to its proprietary ZFP technology and the exclusive right to use the technology to modify the genomes or alter the nucleic acid or protein expression of plant cells, plants, or plant cell cultures. Sangamo has retained rights to use plants or plant-derived products to deliver ZFP-TFs or ZFNs into humans or animals for diagnostic, therapeutic or prophylactic purposes. In 2008, DAS exercised its option and obtained a commercial license to sell products incorporating or derived from plant cells generated using the Company's ZFP technology. The exercise of the option triggered a one-time commercial license fee of \$6.0 million, payment of the remaining \$2.3 million of the previously agreed upon \$4.0 million in research milestones, development and commercialization milestone payments for each product, and royalties on sales of products. Furthermore, DAS has the right to sublicense Sangamo's ZFP technology to third parties for use in plant cells, plants, or plant cell cultures, and Sangamo will be entitled to 25% of any cash consideration received by DAS under such sublicenses. In December 2010, the Company amended its agreement with DAS to extend the period of reagent manufacturing services and research services through December 31, 2012.

The agreement with DAS also provides for minimum sublicense fees each year due to Sangamo every October, provided the agreement is not terminated by DAS. Annual fees range from \$0.3 million to \$3.0 million and total \$25.3 million

over 11 years. The Company has identified the performance obligation within this arrangement as a license to the technology. In the event of any termination of the agreement, all rights to use the Company's ZFP technology will revert to Sangamo, and DAS will no longer be permitted to practice Sangamo's ZFP technology or to develop or, except in limited circumstances, commercialize any products derived from the Company's ZFP technology.

Revenues under the agreement with DAS were \$3.0 million, \$3.0 million, and \$3.0 million during 2020, 2019 and 2018, respectively.

#### NOTE 5 – ACQUISITION OF SANGAMO FRANCE

On July 20, 2018, Sangamo entered into various agreements with the goal of eventually acquiring 100% of Sangamo France's share capital. The Company entered into the Sangamo France SPA with certain shareholders of Sangamo France, pursuant to which it acquired 13,519,036 ordinary shares of Sangamo France ("Ordinary Shares") as part of a block transaction that closed on October 1, 2018 (the "Acquisition Date"). Additionally, the Company and Sangamo France entered into a Tender Offer Agreement pursuant to which Sangamo agreed to acquire 11,528,635 Ordinary Shares for the same price per share as the Sangamo France SPA via a cash tender offer that closed on November 23, 2018. Following the block transaction, cash tender offer, and other open market purchases of shares, the Company owned 98.2% of the Ordinary Shares as of December 31, 2018 (or 25,047,671 Ordinary Shares). In addition to the Sangamo France SPA and the tender offer agreement, the Company also entered into arrangements with the holders of approximately 477,000 "free shares" of Sangamo France pursuant to which the Company has the right to purchase such shares from the holders (a call option) and such holders have the right to sell to the Company such shares from time to time through mid-2021 (a put option) (collectively the "Free Shares Options"). During 2019, the Company acquired approximately 111,000 vested free shares, increasing its ownership of the Ordinary Shares from 98.2% to 98.7%. During 2020, the Company acquired approximately 322,000 vested free shares, pursuant to the exercise of the put options for approximately \$0.7 million of cash, increasing its ownership of the Ordinary Shares to 99.8% as of December 31, 2020.

At the Acquisition Date, the fair value of the Free Shares Options was estimated to be a liability of \$0.2 million. See Note 2 – *Fair Value Measurement – Free Shares Asset* for information regarding the valuation method. The fair value of the Free Shares Options will vary based on future changes in the Company's stock price during the option period. The fair value of the Free Shares Options was estimated to be an asset of \$0.1 million as of December 31, 2020.

The acquisition of Sangamo France was accounted for as a business combination in accordance with ASC Topic 805, *Business Combinations*, in exchange for total consideration of approximately \$45.9 million at the Acquisition Date. The operating results of Sangamo France after the Acquisition Date have been included in the Company's Consolidated Statements of Operations.

There was no goodwill impairment during the years ended December 31, 2020, 2019 or 2018 and, as noted below, substantially all of the non-controlling interest on the Acquisition Date was subsequently acquired by the Company and, accordingly, substantially all of the goodwill is allocated to the Company as of December 31, 2020 and 2019.

#### *Non-controlling Interest*

The fair value of the remaining non-controlling interest was determined based on the number of outstanding shares comprising the non-controlling interest and the \$2.99 acquisition price per share as of the Acquisition Date. The non-controlling interest is presented as a component of stockholders' equity on the Company's Consolidated Balance Sheets.

Non-controlling interest as of December 31, 2020 was as follows (in thousands):

	<b>Total</b>
Balance at beginning of year	\$ 185
Fair value of additional shares acquired	(927)
Loss attributable to non-controlling interest	(126)
Balance at end of year	<u>\$ (868)</u>

**NOTE 6 – OTHER BALANCE SHEET DETAILS**
**Property and Equipment, Net**

Property and equipment, net consist of the following (in thousands):

	December 31,	
	2020	2019
Laboratory equipment	\$ 24,737	\$ 17,179
Furniture and fixtures	4,870	4,639
Leasehold improvements	15,953	13,888
Manufacturing equipment	1,089	—
Construction in progress	12,091	5,901
	58,740	41,607
Less: accumulated depreciation and amortization	(17,416)	(11,681)
Property and equipment, net	\$ 41,324	\$ 29,926

Depreciation and amortization expense was \$5.7 million in 2020, \$3.9 million in 2019 and \$2.4 million in 2018.

**Intangible Assets**

The changes in intangible assets were as follows (in thousands):

	December 31,	
	2020	2019
Balance at beginning of year	\$ 53,156	\$ 54,243
Foreign currency translation adjustment	4,972	(1,087)
Balance at end of year	\$ 58,128	\$ 53,156

**Goodwill**

The changes in goodwill were as follows (in thousands):

	December 31,	
	2020	2019
Balance at beginning of year	\$ 39,273	\$ 40,044
Foreign currency translation adjustment	3,525	(771)
Balance at end of year	\$ 42,798	\$ 39,273

**Other Accrued Liabilities**

Other accrued liabilities consist of the following (in thousands):

	December 31,	
	2020	2019
Customer advance	\$ 5,000	\$ —
Accrued research and development expenses	4,257	4,102
Operating lease liabilities – current	3,690	3,214
Accrued professional fees	1,532	1,118
Other	4,133	2,451
Total other accrued liabilities	\$ 18,612	\$ 10,885

**NOTE 7 – COMMITMENTS AND CONTINGENCIES**
**Leases**

Sangamo occupies approximately 87,700 square feet of office and research and development laboratory facilities in Brisbane, California pursuant to a lease that expires in May 2029. Sangamo also occupies approximately 54,200 square feet of research and office space in Richmond, California, pursuant to leases that expire in August 2026. In addition, the Company

leases approximately 20,800 square feet of office, and research and development space in Valbonne, France, subject to leases that expire beginning in June 2025 through March 2028.

In May 2020, the Company entered into an amendment to an existing lease to acquire approximately 8,500 square feet of research and office space in Richmond, California that expires in August 2026. Total lease payments over the life of this amended lease are approximately \$1.6 million. Variable lease payments include the Company's allocated share of costs incurred and expenditures made by the landlord in the operation and management of the building. The amended lease was effective October 1, 2020, and the Company recorded a lease liability and corresponding ROU asset of \$1.3 million upon inception of this amended lease.

Certain of these leases also include renewal options at the election of the Company to renew or extend the lease for an additional five to 10 years. These optional periods have not been considered in the determination of the ROU assets or lease liabilities associated with these leases as the Company did not consider it reasonably certain it would exercise the options.

The Company performed evaluations of its contracts and determined each of its identified leases are operating leases. For the year ended December 31, 2020, the Company incurred \$10.4 million of lease costs included in operating expenses in the Consolidated Statement of Operations in relation to these operating leases. Variable lease expense was \$2.3 million for the year ended December 31, 2020 and was not included in the measurement of the Company's operating ROU assets and lease liabilities. The variable expense consists primarily of the Company's proportionate share of operating expenses, property taxes and insurance and is classified as lease expense due to the Company's election to not separate lease and non-lease components.

Cash paid for amounts included in the measurement of operating lease liabilities for the year ended December 31, 2020 was \$6.4 million and was included in net cash provided by operating activities in the Company's Consolidated Statement of Cash Flows.

Rent expense related to lease agreements was \$10.4 million, \$7.9 million, and \$2.3 million for 2020, 2019 and 2018, respectively. Future minimum payments under lease obligations at December 31, 2020 consist of the following (in thousands):

	<b>Total</b>
2021	\$ 6,191
2022	6,756
2023	6,851
2024	6,995
2025	7,058
Thereafter	19,571
<b>Total lease payments</b>	<b>53,422</b>
Less:	
Imputed interest	(11,336)
<b>Total</b>	<b>\$ 42,086</b>
Reported as of December 31, 2020:	
Operating lease liabilities - current (included in Other accrued liabilities on the Consolidated Balance Sheet)	\$ 3,690
Operating lease liabilities - long-term	38,396
<b>Total</b>	<b>\$ 42,086</b>

As of December 31, 2020, the weighted-average remaining lease term is 7.6 years and the weighted-average incremental borrowing rate used to determine the operating lease liability was 6.1% for the Company's operating leases.

The Company does not have any financing leases.

### **Contractual Commitments**

The following table sets forth the non-cancelable material contractual commitments under manufacturing-related supplier arrangements as of December 31, 2020 (in thousands):

<b>Party</b>	<b>Total commitments</b>	<b>Expiry date</b>
Brammer Bio MA - a Thermo Fisher Scientific Inc. subsidiary	\$ 7,736	December 2022
Lonza Netherlands, B.V.	13,771	December 2022
<b>Total contractual commitments</b>	<b>\$ 21,507</b>	

The Company also had \$1.0 million of license obligations related to its intellectual property as of December 31, 2020.

### **Contingencies**

Sangamo is not party to any material pending legal proceeding. From time to time, Sangamo may be involved in legal proceedings arising in the ordinary course of business.

## **NOTE 8 – STOCKHOLDERS’ EQUITY**

### **Preferred Stock**

The Company’s Certificate of Incorporation authorizes the Company to issue up to 5,000,000 shares of preferred stock, which may be issued at the discretion of the Company’s Board of Directors. As of December 31, 2020, no shares of the Company’s preferred stock have been issued or are outstanding.

### **Common Stock**

In June 2020, the Company’s stockholders approved an amendment to the Company’s Certificate of Incorporation to increase the total number of shares of the Company’s common stock authorized for issuance from 160,000,000 shares to 320,000,000 shares. As of December 31, 2020, 142,063,203 shares of the Company’s common stock are outstanding.

In connection with the collaboration agreement with BIMA described in Note 4 of these Consolidated Financial Statements, the Company entered into a stock purchase agreement with BIMA, pursuant to which BIMA agreed to purchase the Biogen Shares at a price per share of \$9.2137, for an aggregate purchase price of \$225.0 million. The Company closed the sale of the Biogen Shares in April 2020.

In April 2019, the Company completed an underwritten public offering of its common stock, in which the Company sold an aggregate of 12.7 million shares of its common stock at a public offering price of \$11.50 per share. The net proceeds to the Company from the sale of shares in this offering, after deducting underwriting discounts and commissions and other offering expenses, were approximately \$136.3 million.

In April 2018, the Company completed an underwritten public offering of its common stock, in which the Company sold an aggregate of 14.2 million shares of its common stock at a public offering price of \$16.25 per share. The net proceeds to the Company from the sale of shares in this offering, after deducting underwriting discounts and commissions and other offering expenses, were approximately \$215.8 million.

### **At-the-Market Offering Agreement**

In August 2020, the Company entered into an Open Market Sale Agreement<sup>SM</sup> with Jefferies LLC (“Jefferies”) with respect to an at-the-market offering program under which the Company may offer and sell, from time to time at its sole discretion, shares of the Company’s common stock having an aggregate offering price of up to \$150.0 million through Jefferies as the Company’s sales agent or principal. The Company is not obligated to sell any shares under the sales agreement. As of December 31, 2020, no shares had been sold under the sales agreement.

### **2018 Equity Incentive Plan**

In June 2018, the Company’s stockholders approved the Sangamo Therapeutics, Inc. 2018 Equity Incentive Plan (the “2018 Plan”). In connection with the approval of the 2018 Plan, no additional equity awards will be granted under the previous 2013 Plan, however all outstanding equity awards under the 2013 Plan will continue to be subject to the terms and conditions as set forth in the agreements evidencing such awards and the terms of the 2013 Plan.

In May 2020, the Company’s stockholders approved an amendment and restatement of the 2018 Plan, to, among other things, increase the aggregate number of shares of the Company’s common stock reserved for issuance under the 2018 Plan by 9,900,000 shares.

The exercise price of a stock option granted under the 2018 Plan may not be less than 100% of the fair market value of the Company’s common stock subject to the stock option on the date of grant, and the option term will not exceed 10 years. If the person to whom the stock option is granted is a 10% stockholder of the Company, and the stock option granted qualifies as an incentive stock option, then the exercise price per share will not be less than 110% of the fair market value of the Company’s common stock on the date of grant, and the option term will not exceed five years. Generally, stock options granted under the 2018 Plan vest over four years at a rate of 25% on the one-year anniversary of the date of grant and 1/48 per month thereafter and expire 10 years after the date of grant, or earlier upon termination of employment or services to the Company.

The number of shares of common stock reserved for issuance under the 2018 Plan will be reduced: (i) on a 1-for-1 basis for each share of common stock subject to a stock option or stock appreciation right granted under the plan, (ii) by a fixed

ratio of 1.33 shares of common stock for each share of common stock issued pursuant to a full-value award granted under the plan.

Shares subject to any outstanding stock options or other awards under the 2018 Plan that expire or otherwise terminate prior to the issuance of the shares subject to those stock options or awards will be available for subsequent issuance under the 2018 Plan. Any unvested shares issued under the 2018 Plan that the Company subsequently purchases, pursuant to repurchase rights under the 2018 Plan, will be added back to the number of shares reserved for issuance under the 2018 Plan on a 1-for-1 basis or a 1.33-for-1 basis (depending on the ratio at which the share reserve was debited for the original award) and will accordingly be available for subsequent issuance in accordance with the terms of the 2018 Plan.

As of December 31, 2020, there were 10,942,576 shares of the Company's common stock reserved for future awards under the Company's 2018 Plan.

### 2010 Employee Stock Purchase Plan

On June 2018, the Company's stockholders approved an amendment and restatement of the Company's 2010 Employee Stock Purchase Plan ("the ESPP"). As amended, the ESPP provides for a total of 4.6 million shares of common stock reserved for issuance thereunder. Eligible employees may purchase common stock at 85% of the lesser of the fair market value of the Company's common stock on the first day of the applicable two-year offering period or the last day of the applicable six-month purchase period. As of December 31, 2020, there were 2,483,218 shares of the Company's common stock reserved for future issuance under the ESPP. The ESPP expired on April 30, 2020. The ongoing offering will continue through the end of its 24 months offering period ending on October 29, 2021 at which point the ESPP will be fully terminated.

### Stock Option Activity

A summary of the Company's stock option activity is as follows:

	Number of Shares	Weighted- Average Exercise per Share Price	Weighted-Average Remaining Contractual Term (In years)	Aggregate Intrinsic Value (In thousands)
Options outstanding at December 31, 2019	9,829,287	\$ 10.71		
Options granted	4,563,425	\$ 8.09		
Options exercised	(1,162,268)	\$ 8.02		
Options canceled	(1,751,746)	\$ 10.19		
Options outstanding at December 31, 2020	<u>11,478,698</u>	\$ 10.02	7.80	\$ 69,616
Options vested and expected to vest at December 31, 2020	11,478,698	\$ 10.02	7.80	\$ 69,616
Options exercisable at December 31, 2020	5,127,517	\$ 10.71	6.48	\$ 29,025

The intrinsic value of options exercised was \$5.4 million, \$4.7 million and \$27.0 million during 2020, 2019 and 2018, respectively.

At December 31, 2020, the aggregate intrinsic values of outstanding and exercisable options were \$69.6 million and \$29.0 million, respectively. The aggregate intrinsic value of options vested and expected to vest as of December 31, 2020, 2019 and 2018 was \$69.6 million, \$7.5 million and \$24.5 million, respectively.

### Restricted Stock Units

During 2020, 2019 and 2018, the Company awarded 2,517,101, 834,745, and 346,055 RSUs, respectively. The RSUs awarded in 2020, 2019 and 2018 had an average grant date fair value per award of \$8.06, \$9.49 and \$17.87, respectively. These awards generally vest in a series of three successive equal annual installments. The aggregate fair value of RSUs vested during 2020, 2019 and 2018 was \$3.7 million, \$2.0 million and \$0.6 million, respectively.

A summary of the Company's RSU activity is as follows:

	Number of Shares	Weighted-Average Remaining Contractual Term (In years)	Aggregate Intrinsic Value (In thousands)
RSUs outstanding at December 31, 2019	862,850		
RSUs awarded	2,517,101		
RSUs released	(324,305)		
RSUs forfeited	(384,118)		
RSUs outstanding at December 31, 2020	<u>2,671,528</u>	1.19	\$ 41,689
RSUs vested and expected to vest at December 31, 2020	<u>2,671,528</u>	1.19	\$ 41,689

RSUs that vested in 2020, 2019 and 2018 were net-share settled such that the Company withheld shares with value equivalent to the employees' minimum statutory obligation for the applicable income and other employment taxes and remitted the cash to the appropriate taxing authorities. The total shares withheld were approximately 90,617, 39,160, and 20,193 for 2020, 2019 and 2018, respectively, and were based on the value of the RSUs on their respective issuance dates as determined by the Company's closing stock price. Total payments for the employees' tax obligations to taxing authorities were \$0.8 million, \$0.4 million and \$0.3 million in 2020, 2019 and 2018, respectively and are reflected as a financing activity within the accompanying Consolidated Statements of Cash Flows. These net-share settlements had the effect of share repurchases by the Company as they reduced and retired the number of shares that would have otherwise been issued as a result of the vesting and did not represent an expense to the Company.

#### NOTE 9 – STOCK-BASED COMPENSATION

The following table shows total stock-based compensation expense recognized in the accompanying Consolidated Statements of Operations (in thousands):

	Year Ended December 31,		
	2020	2019	2018
Research and development	\$ 13,523	\$ 10,135	\$ 8,249
General and administrative	12,185	9,195	6,428
Total stock-based compensation expense	<u>\$ 25,708</u>	<u>\$ 19,330</u>	<u>\$ 14,677</u>

As of December 31, 2020, total stock-based compensation expense to be recognized in future periods related to unvested stock options was \$35.5 million, which is expected to be expensed over a weighted-average period of 2.72 years. As of December 31, 2020, total stock-based compensation expense to be recognized in future periods related to unvested RSUs was \$17.1 million, which is expected to be expensed over a weighted-average period of 2.07 years. There was no capitalized stock-based employee compensation expense as of December 31, 2020, 2019 or 2018.

#### Valuation Assumptions

Employee stock-based compensation expense was determined using the Black-Scholes option valuation model for stock options and employee share purchases under the ESPP. Option valuation models require the input of subjective assumptions and these assumptions can vary over time. The fair value of RSUs was based on the closing price of the underlying common stock on the date of grant.

The Company bases its determination of expected volatility through its assessment of the historical volatility of its common stock. The Company relied on its historical exercise and post-vested termination activity for estimating its expected term for use in determining the fair value of these options.



The weighted-average estimated fair value per share of options granted during 2020, 2019 and 2018 was \$5.25, \$6.37, and \$11.39, respectively, based upon the assumptions used in the Black-Scholes valuation model. The assumptions used for estimating the fair value of the employee stock options were as follows:

	Year Ended December 31,		
	2020	2019	2018
Risk-free interest rate	0.34-0.61%	1.68-2.25%	2.53-2.96%
Expected term (in years)	5.51-5.57	5.50-5.62	5.59-5.61
Expected dividend yield of stock	—	—	—
Expected volatility	77.61-80.32%	76.46-78.39%	72.33-75.49%

Employees purchased 274,382, 249,364 and 328,710 shares of common stock through the ESPP at an average exercise price of \$7.34, \$8.53, and \$4.51 per share during 2020, 2019 and 2018, respectively. The weighted-average estimated fair values of shares purchased under the Company's ESPP during 2020, 2019 and 2018 were \$8.02, \$4.70 and \$7.07, respectively, based upon the assumptions used in the Black-Scholes valuation model.

The assumptions used for estimating the fair value of the ESPP purchase rights are as follows:

	Year Ended December 31,		
	2020	2019	2018
Risk-free interest rate	1.53-2.80%	1.53-2.42%	2.16-2.80%
Expected term (in years)	0.5-2.0	0.5-2.0	0.5-2.0
Expected dividend yield of stock	—	—	—
Expected volatility	51.02-91.96%	51.02-91.96%	73.21-83.25%

#### NOTE 10 – EMPLOYEE BENEFIT PLAN

The Company sponsors a defined-contribution savings plan under Section 401(k) of the Internal Revenue Code covering all full-time employees ("Sangamo 401(k) Plan"). The Sangamo 401(k) Plan is intended to qualify under Section 401 of the Internal Revenue Code.

The Company matched employee contributions equal to 50% for the first 8% in 2020, 2019 and 2018, up to a limit of \$4,000 in 2020, 2019 and 2018. Matching funds are fully vested when contributed. Contributions to the Sangamo 401(k) Plan by the Company were \$1.2 million, \$0.9 million, and \$0.8 million for the years ended December 31, 2020, 2019 and 2018, respectively.

#### NOTE 11 – INCOME TAXES

The domestic and foreign components of loss before income taxes were as follows (in thousands):

	Year Ended December 31,		
	2020	2019	2018
Domestic	\$ (126,624)	\$ (77,354)	\$ (65,695)
Foreign	5,847	(18,065)	(3,194)
Loss before income taxes	\$ (120,777)	\$ (95,419)	\$ (68,889)

The income tax expense consisted of the following (in thousands):

	Year Ended December 31,		
	2020	2019	2018
Income tax expense:			
Current:			
Federal	\$ —	\$ —	\$ —
State	133	—	—
Foreign	686	—	—
Subtotal	819	—	—
Deferred:			
Federal	—	—	—
State	—	—	—
Foreign	(474)	—	—
Subtotal	(474)	—	—
Income tax expense	\$ 345	\$ —	\$ —

The difference between the income tax expense and the amount computed by applying the federal statutory income tax rate to loss before income taxes is explained as follows (in thousands):

	Year Ended December 31,		
	2020	2019	2018
Tax at federal statutory rate	\$ (25,363)	\$ (20,038)	\$ (14,467)
State taxes, net	(3,168)	(9,597)	(2,849)
Foreign rate differential	376	(665)	(177)
Global Intangible Low-Taxed Income	1,335	—	—
Non-deductible stock-based compensation	4,232	2,817	(2,729)
Research credits	(3,657)	(3,429)	(1,005)
Change in valuation allowance	26,537	29,655	20,271
Other	53	1,257	956
Income tax expense	\$ 345	\$ —	\$ —

In March and December 2020, in response to the COVID-19 pandemic, the CARES Act and the Consolidated Appropriations Act, 2021 were passed into law and provide additional economic stimulus to address the impact of the COVID-19 pandemic. The Company does not expect any significant benefit to its income tax provision as a result of this legislation.

Deferred income taxes reflect the net tax effects of 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 deferred tax assets and liabilities are as follows (in thousands):

	December 31,	
	2020	2019
<b>Assets:</b>		
Deferred tax assets:		
Net operating loss carryforwards	\$ 164,276	\$ 133,765
Research and development tax credit carryforwards	27,679	21,459
Stock-based compensation	5,321	4,194
Deferred revenue	20,681	32,171
Fixed assets	10,525	11,282
Lease liability	10,251	11,722
Accruals and reserves	430	675
Other	308	151
<b>Total deferred tax asset</b>	<b>239,471</b>	<b>215,419</b>
Valuation allowance	214,351	187,724
<b>Deferred tax assets</b>	<b>25,120</b>	<b>27,695</b>
<b>Liabilities:</b>		
Intangible assets	(14,321)	(13,609)
Operating lease right-of-use assets	(17,510)	(20,656)
<b>Deferred tax liabilities</b>	<b>(31,831)</b>	<b>(34,265)</b>
<b>Total net deferred tax liabilities</b>	<b>\$ (6,711)</b>	<b>\$ (6,570)</b>

The deferred tax assets and liabilities based on tax jurisdictions are presented on the Consolidated Balance Sheet as follows (in thousands):

	December 31,	
	2020	2019
Deferred tax assets (included in Other non-current assets on the Consolidated Balance Sheet)	\$ 474	\$ —
Deferred tax liabilities	(7,185)	(6,570)
<b>Net deferred tax liabilities</b>	<b>\$ (6,711)</b>	<b>\$ (6,570)</b>

In October 2018, the Company acquired Sangamo France. The Company recorded goodwill and intangible assets as part of accounting for the acquisition of Sangamo France. There is no corresponding tax basis for the goodwill or intangible assets. A portion of the intangible assets acquired were for the use in a particular research and development project and are considered indefinite-lived assets with no tax basis.

The changes in the fair value of the unrealized gain (loss) on marketable securities are recorded as a component of accumulated other comprehensive income (loss), net of a provision for income taxes.

A valuation allowance is recorded when it is more likely than not that all or some portion of the deferred income tax assets will not be realized. The Company regularly assesses the need for a valuation allowance against its deferred income tax assets by considering both positive and negative evidence related to whether it is more likely than not that the Company's deferred income tax assets will be realized. In evaluating the Company's ability to recover its deferred income tax assets within the jurisdiction from which they arise, the Company considers all available positive and negative evidence, including scheduled reversals of deferred income tax liabilities, projected future taxable income, tax-planning strategies, and results of recent operations. Accordingly, based upon the Company's analysis of these factors the net deferred tax assets have been substantially offset by a valuation allowance. The valuation allowance increased by \$26.6 million, \$29.6 million and \$45.3 million for the years ended December 31, 2020, 2019 and 2018, respectively.

As of December 31, 2020, Sangamo had net operating loss carryforwards for federal and state income tax purposes of approximately \$622.6 million and \$261.7 million, respectively. The federal net operating loss generated before 2018 will begin to expire in 2024 and will keep expiring through 2037, if not utilized. Federal net operating loss generated in 2018 will carry forward indefinitely. If not utilized, the state net operating loss carryforwards will begin to expire in 2029, respectively. The Company's French net operating loss carryforward balance is \$145.9 million, which carries over indefinitely. The Company

also has federal and state research tax credit carryforwards of \$21.9 million and \$18.3 million, respectively. The federal research credits will begin to expire in 2021, while the state research credits have no expiration date. Utilization of the Company's net operating loss carryforwards and research tax credit carryforwards may be subject to substantial annual limitations due to the ownership change limitations provided by the Internal Revenue Code and similar state provisions. The annual limitation could result in the expiration of the net operating loss carryforwards and research tax credit carryforwards before utilization.

The Company's policy is to reinvest the earnings of its non-U.S. subsidiaries in those operations. The Company does not provide for U.S. taxes on the earnings of foreign subsidiaries because the Company intends to reinvest such earnings offshore indefinitely. However, if these funds were repatriated, the Company would be required to accrue and pay applicable U.S. taxes and withholding taxes. Due to the cumulative losses generated in foreign countries there are no earnings to repatriate.

The Company files federal and state income tax returns with varying statutes of limitations. The tax years from 2002 forward remain open to examination due to the carryover of net operating losses or tax credits. The Company also files United Kingdom and French income tax returns, and the tax years from 2008 and thereafter remain open in the United Kingdom and 2016 and thereafter in France are still subject to examination.

The Company's practice is to recognize interest and/or penalties related to income tax matters in income tax expense. As of December 31, 2020, the Company had no accrued interest and/or penalties. The unrecognized tax benefits may change during the next year for items that arise in the ordinary course of business. In the event that any unrecognized tax benefits are recognized, the effective tax rate will not be affected.

The following table summarizes the activity related to the Company's unrecognized tax benefits (in thousands):

	December 31,		
	2020	2019	2018
Beginning balance	\$ 11,630	\$ 6,288	\$ 5,659
Additions based on tax positions related to the current year	2,834	5,393	636
Additions for tax positions of prior years	1,982	—	—
Reductions for tax positions of prior years	(3,554)	(51)	(7)
Ending balance	<u>\$ 12,892</u>	<u>\$ 11,630</u>	<u>\$ 6,288</u>

#### NOTE 12 – RELATED PARTY TRANSACTION

The Company acquired 185,400 and 52,700 vested free shares from a former executive of Sangamo, pursuant to the exercise of the Free Shares Options for approximately \$0.4 million and \$0.1 million of cash, during the years ended December 31, 2020 and 2019, respectively.

#### NOTE 13 – QUARTERLY FINANCIAL DATA (UNAUDITED)

The following table sets forth certain unaudited quarterly financial data for the eight quarters ended December 31, 2020. The unaudited information set forth below has been prepared on the same basis as the audited information contained herein and includes all adjustments necessary to present fairly the information set forth. The operating results for any quarter are not indicative of results for any future period. All amounts are in thousands except per share amounts.

	2020				2019			
	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4
Revenues	\$ 13,076	\$ 21,553	\$ 57,763	\$ 25,800	\$ 8,071	\$ 17,548	\$ 21,958	\$ 54,851
Operating expenses	\$ 57,598	\$ 59,450	\$ 61,464	\$ 69,232	\$ 51,968	\$ 51,052	\$ 51,206	\$ 53,382
Net (loss) income	\$ (42,974)	\$ (35,965)	\$ (1,508)	\$ (40,675)	\$ (42,203)	\$ (30,356)	\$ (27,361)	\$ 4,501
Net (loss) income attributable to non-controlling interest	\$ (61)	\$ (36)	\$ 42	\$ (71)	\$ (53)	\$ (72)	\$ (54)	\$ (54)
Net (loss) income attributable to Sangamo Therapeutics, Inc.	\$ (42,913)	\$ (35,929)	\$ (1,550)	\$ (40,604)	\$ (42,150)	\$ (30,284)	\$ (27,307)	\$ 4,555
Basic and diluted net (loss) income per share attributable to Sangamo Therapeutics, Inc.	\$ (0.37)	\$ (0.26)	\$ (0.01)	\$ (0.29)	\$ (0.41)	\$ (0.26)	\$ (0.24)	\$ 0.04

## **NOTE 14 – SUBSEQUENT EVENTS**

In the first quarter of 2021 through February 19, 2021, the Company sold 1,034,762 shares of its common stock for gross proceeds of approximately \$16.1 million from an at-the-market offering program under an Open Market Sale Agreement<sup>SM</sup> with Jefferies dated August 5, 2020. After deducting sales commissions and expenses, net cash proceeds through February 19, 2021 under the at-the-market offering are approximately \$15.7 million.

## **ITEM 9 – CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE**

None.

## **ITEM 9A – CONTROLS AND PROCEDURES**

### **Evaluation of Disclosure Controls and Procedures**

We maintain disclosure controls and procedures that are designed to provide reasonable assurance that information required to be disclosed in our Exchange Act reports is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms and that such information is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

Under the supervision of our principal executive officer and acting principal financial officer, we evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Exchange Act) as of December 31, 2020. Based on that evaluation, as of December 31, 2020, our principal executive officer and acting principal financial officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level.

### **Inherent Limitations on Controls and Procedures**

Our management, including the principal executive officer and acting principal financial officer, does not expect that our disclosure controls and procedures and our internal control over financial reporting will prevent all error and all fraud. A control system, no matter how well designed and operated, can only provide reasonable assurances that the objectives of the control system are met. The design of a control system reflects resource constraints; the benefits of controls must be considered relative to their costs. Because there are inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, for our company have been or will be detected. As these inherent limitations are known features of the disclosure and financial reporting processes, it is possible to design into the processes safeguards to reduce, though not eliminate, these risks. These inherent limitations include the realities that judgments in decision-making can be faulty and that breakdowns occur because of simple error or mistake. Controls can also be circumvented by the individual acts of some persons, by collusion of two or more people, or by management override of the control. The design of any system of controls is based in part upon certain assumptions about the likelihood of future events. While our disclosure controls and procedures and our internal control over financial reporting are designed to provide reasonable assurance of achieving their objectives, there can be no assurance that any design will succeed in achieving its stated goals under all future conditions. Over time, controls may become inadequate because of changes in conditions or deterioration in the degree of compliance with the policies or procedures. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

### **Management's Report on Internal Control over Financial Reporting**

Our management is responsible for establishing and maintaining an adequate internal control over financial reporting (as such term is defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act) for our company. Our management, including our principal executive officer and acting principal financial officer, conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework set forth in the "Internal Control—Integrated Framework" issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework). Based on an evaluation under that framework, our management concluded that our internal control over financial reporting was effective at the reasonable assurance level as of December 31, 2020.

The effectiveness of our internal control over financial reporting as of December 31, 2020 has been audited by Ernst & Young LLP, an independent registered public accounting firm, as stated in their report, which is included herein.

**Changes in Internal Control over Financial Reporting**

There have been no changes in our internal control over financial reporting identified in connection with the evaluation required by Rules 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the three months ended December 31, 2020 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

## Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Sangamo Therapeutics, Inc.

### Opinion on Internal Control over Financial Reporting

We have audited Sangamo Therapeutics, Inc.'s internal control over financial reporting as of December 31, 2020, based on criteria established in Internal Control – Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) (the COSO criteria). In our opinion, Sangamo Therapeutics, Inc. (the Company) maintained, in all material respects, effective internal control over financial reporting as of December 31, 2020, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the 2020 consolidated financial statements of the Company and our report dated February 24, 2021 expressed an unqualified opinion thereon.

### Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management's Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the 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 audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects.

Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

### Definition and Limitations of Internal Control Over Financial Reporting

A company's 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. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

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.

/s/ ERNST & YOUNG LLP

Redwood City, California  
February 24, 2021

## **ITEM 9B – OTHER INFORMATION**

None.

## **PART III**

Certain information required by Part III is omitted from this Report on Form 10-K because we intend to file our definitive Proxy Statement for our next Annual Meeting of Stockholders, pursuant to Regulation 14A of the Securities Exchange Act of 1934, as amended, or the 2021 Proxy Statement, no later than April 30, 2021, and certain information to be included in the 2021 Proxy Statement is incorporated herein by reference.

## **ITEM 10 – DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE**

The information required by this item is to be included in our 2021 Proxy Statement as follows:

- The information relating to our directors and nominees for director is to be included in the section entitled “Election of Directors;”
- The information relating to our executive officers is to be included in the section entitled “Executive Officers;”
- The information relating to our audit committee and audit committee financial expert is to be included in the section entitled “Election of Directors – Audit Committee;”
- The information relating to the procedures by which stockholders may recommend nominees to our Board of Directors is to be included in the section entitled “Questions and Answers About These Proxy Materials and Voting;” and
- The information regarding compliance with Section 16(a) of the Exchange Act is to be included in the section entitled “Delinquent Section 16(a) Reporting.”

Such information is incorporated herein by reference to our 2021 Proxy Statement, provided that if the 2021 Proxy Statement is not filed within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, the omitted information will be included in an amendment to this Annual Report on Form 10-K filed not later than the end of such 120-day period.

## **ITEM 11 – EXECUTIVE COMPENSATION**

The information required by this item is to be included in our 2021 Proxy Statement under the sections entitled “Executive Compensation,” “Director Compensation,” “Election of Directors—Compensation Committee Interlocks and Insider Participation” and “Report of the Compensation Committee of the Board of Directors” and is incorporated herein by reference, provided that if the 2021 Proxy Statement is not filed within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, the omitted information will be included in an amendment to this Annual Report on Form 10-K filed not later than the end of such 120-day period.

## **ITEM 12 – SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS**

The information required by this item with respect to equity compensation plans is to be included in our 2021 Proxy Statement under the section entitled “Equity Compensation Plan Information” and the information required by this item with respect to security ownership of certain beneficial owners and management is to be included in our 2021 Proxy Statement under the section entitled “Security Ownership of Certain Beneficial Owners and Management” and in each case is incorporated herein by reference, provided that if the 2021 Proxy Statement is not filed within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, the omitted information will be included in an amendment to this Annual Report on Form 10-K filed not later than the end of such 120-day period.

## **ITEM 13 – CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE**

The information required by this item is to be included in our 2021 Proxy Statement under the sections entitled “Certain Relationships and Related Transactions” and “Election of Directors—Board Independence” and is incorporated herein by reference, provided that if the 2021 Proxy Statement is not filed within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, the omitted information will be included in an amendment to this Annual Report on Form 10-K filed not later than the end of such 120-day period.



**ITEM 14 – PRINCIPAL ACCOUNTING FEES AND SERVICES**

The information required by this item is to be included in our 2021 Proxy Statement under the section entitled “Ratification of Independent Registered Public Accounting Firm” and is incorporated herein by reference, provided that if the 2021 Proxy Statement is not filed within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, the omitted information will be included in an amendment to this Annual Report on Form 10-K filed not later than the end of such 120-day period.

**PART IV****ITEM 15 – EXHIBITS, FINANCIAL STATEMENT SCHEDULES**

(a) The following documents are included as part of this Annual Report on Form 10-K:

1. Financial Statements—See Index to Consolidated Financial Statements in Item 8.
2. Financial Statement Schedules—Not Applicable.
3. Exhibits

<u>Exhibit Number</u>	<u>Description of Document</u>
2.1	<a href="#">Share Purchase Agreement dated July 20, 2018 among the Company and the Selling TxCell Shareholders named on the signature page thereto (incorporated by reference to Exhibit 2.1 to the Company’s Current Report on Form 8-K filed July 23, 2018).</a>
2.2	<a href="#">Amendment Agreement to the Share Purchase Agreement dated October 1, 2018 between the Company and TxCell S.A. (incorporated by reference to Exhibit 2.2 to the Company’s Current Report on Form 8-K filed November 6, 2018).</a>
2.3	<a href="#">Tender Offer Agreement dated July 20, 2018 between the Company and TxCell S.A. (incorporated by reference to Exhibit 2.2 to the Company’s Current Report on Form 8-K filed July 23, 2018).</a>
2.4	<a href="#">Amendment No. 1 to the Tender Offer Agreement dated October 1, 2018 between the Company and TxCell S.A. (incorporated by reference to Exhibit 2.4 to the Company’s Current Report on Form 8-K filed November 6, 2018).</a>
3.1	<a href="#">Seventh Amended and Restated Certificate of Incorporation, as amended (incorporated by reference to Exhibit 3.1 to the Company’s Quarterly Report on Form 10-Q filed August 9, 2017).</a>
3.2	<a href="#">Fourth Certificate of Amendment of the Seventh Amended and Restated Certificate of Incorporation (incorporated by reference to Exhibit 3.1 to the Company’s Current Report on Form 8-K filed May 22, 2020).</a>
3.3	<a href="#">Fourth Amended and Restated Bylaws (incorporated by reference to Exhibit 3.1 to the Company’s Current Report on Form 8-K filed December 15, 2020).</a>
4.1	<a href="#">Description of Capital Stock.</a>
4.2	<a href="#">Form of Specimen Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Company’s Current Report on Form 8-K filed January 6, 2017).</a>
10.1(+)	<a href="#">Amended and Restated 2013 Stock Incentive Plan (the “2013 Plan”) (incorporated by reference to Exhibit 10.2 to the Company’s Quarterly Report on Form 10-Q filed May 10, 2018).</a>
10.2(+)	<a href="#">Amended and Restated 2018 Equity Incentive Plan (the “2018 Plan”) (incorporated by reference to Exhibit 10.1 to the Company’s Current Report on Form 8-K filed May 22, 2020).</a>
10.3(+)	<a href="#">2018 Equity Incentive Plan French Stock-Options Sub-Plan (the “French Options Sub-Plan”) (incorporated by reference to Exhibit 10.3 to the Company’s Annual Report on Form 10-K filed March 1, 2019).</a>
10.4(+)	<a href="#">2018 Equity Incentive Plan French Restricted Stock Unit Award Sub-Plan (the “French RSU Sub-Plan”) (incorporated by reference to Exhibit 10.4 to the Company’s Annual Report on Form 10-K (File No. 000-30171), filed with the SEC on March 1, 2019).</a>
10.5(+)	<a href="#">2020 Employee Stock Purchase Plan (incorporated by reference to Exhibit 99.1 to the Company’s Registration Statement on Form S-8, filed October 15, 2020).</a>
10.6(+)	<a href="#">Form of Restricted Stock Unit Award Agreement under the 2013 Plan (incorporated by reference to Exhibit 10.2 to the Company’s Current Report on Form 8-K filed June 14, 2013).</a>
10.7(+)	<a href="#">Form of Notice of Grant of Stock Option under the 2013 Plan (incorporated by reference to Exhibit 10.3 to the Company’s Current Report on Form 8-K filed June 14, 2013).</a>
10.8(+)	<a href="#">Form of Stock Option Agreement under the 2013 Plan (incorporated by reference to Exhibit 10.4 to the Company’s Current Report on Form 8-K filed June 14, 2013).</a>
10.9(+)	<a href="#">Form of Notice of Grant of Stock Option – Director Initial Grant under the 2013 Plan (incorporated by reference to Exhibit 10.5 to the Company’s Current Report on Form 8-K filed June 14, 2013).</a>
10.10(+)	<a href="#">Form of Notice of Grant of Stock Option – Director Annual Grant under the 2013 Plan (incorporated by reference to Exhibit 10.6 to the Company’s Current Report on Form 8-K filed June 14, 2013).</a>
10.11(+)	<a href="#">Form of Automatic Stock Option Agreement under the 2013 Plan (incorporated by reference to Exhibit 10.7 to the Company’s Current Report on Form 8-K filed June 14, 2013).</a>
10.12(+)	<a href="#">Form of Stock Option Grant Notice and Form of Option Agreement (U.S. employees) under the 2018 Plan (incorporated by reference to Exhibit 99.2 to the Company’s Current Report on Form 8-K filed June 15, 2018).</a>

<b><u>Exhibit Number</u></b>	<b><u>Description of Document</u></b>
10.13(+)	<a href="#">Form of Stock Option Grant Notice and Form of Option Agreement (non-employee directors) under the 2018 Plan (incorporated by reference to Exhibit 99.3 to the Company's Current Report on Form 8-K filed June 15, 2018).</a>
10.14(+)	<a href="#">Form of Stock Option Grant Notice and Form of Option Agreement (U.K. employees) under the 2018 Plan (incorporated by reference to Exhibit 99.4 to the Company's Current Report on Form 8-K filed June 15, 2018).</a>
10.15(+)	<a href="#">Form of Stock Option Grant Notice (French employees) under the 2018 Plan and the French Options Sub-Plan (incorporated by reference to Exhibit 10.14 to the Company's Annual Report on Form 10-K filed March 1, 2019).</a>
10.16(+)	<a href="#">Form of Stock Option Agreement (French Employees) under the 2018 Plan and the French Options Sub-Plan (incorporated by reference to Exhibit 10.15 to the Company's Annual Report on Form 10-K filed March 1, 2019).</a>
10.17(+)	<a href="#">Form of Restricted Stock Unit Grant Notice and Form of Restricted Stock Unit Award Agreement (U.S. employees) under the 2018 Plan (incorporated by reference to Exhibit 99.5 to the Company's Current Report on Form 8-K filed June 15, 2018).</a>
10.18(+)	<a href="#">Form of Restricted Stock Unit Grant Notice and Form of Restricted Stock Unit Award Agreement (non-employee directors) under the 2018 Plan (incorporated by reference to Exhibit 99.6 to the Company's Current Report on Form 8-K filed June 15, 2018).</a>
10.19(+)	<a href="#">Form of Restricted Stock Unit Grant Notice and Form of Restricted Stock Unit Award Agreement (U.K. employees) under the 2018 Plan (incorporated by reference to Exhibit 99.7 to the Company's Current Report on Form 8-K filed June 15, 2018).</a>
10.20(+)	<a href="#">Form of Restricted Stock Unit Grant Notice and Form of Restricted Stock Unit Award Agreement (French employees) under the 2018 Plan and the French RSU Sub-Plan. (incorporated by reference to Exhibit 10.19 to the Company's Annual Report on Form 10-K (File No. 000-30171), filed with the SEC on March 1, 2019).</a>
10.21(+)	<a href="#">Amended and Restated Severance Plan (incorporated by reference to Exhibit 10.20 to the Company's Annual Report on Form 10-K (File No. 000-30171), filed with the SEC on March 1, 2019).</a>
10.22(+)	<a href="#">Amended and Restated Incentive Compensation Plan (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed May 10, 2018).</a>
10.23(+)	<a href="#">Form of Indemnity Agreement (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q filed May 11, 2020).</a>
10.24(+)	<a href="#">Employment Agreement between the Company and Alexander (Sandy) Macrae, dated May 17, 2016 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed August 4, 2016).</a>
10.25(+)	<a href="#">Employment Agreement between the Company and Sung Lee effective as of October 31, 2019 (incorporated by reference to Exhibit 10.24 to the Company's Annual Report on Form 10-K filed February 28, 2020).</a>
10.26(+)	<a href="#">Letter Agreement between the Company and Sung Lee dated as of January 22, 2021 (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed January 28, 2021).</a>
10.27(+)	<a href="#">Employment Agreement between the Company and Gary Loeb effective as of June 6, 2019 (incorporated by reference to Exhibit 10.25 to the Company's Annual Report on Form 10-K filed February 28, 2020).</a>
10.28(+)	<a href="#">Employment Agreement between the Company and Rolf Andrew (Andy) Ramelmeier effective as of November 1, 2017 (incorporated by reference to Exhibit 10.26 to the Company's Annual Report on Form 10-K filed February 28, 2020).</a>
10.29(+)	<a href="#">Letter Agreement Regarding Andrew Ramelmeier Special Bonus (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed August 5, 2020).</a>
10.30(+)	<a href="#">Employment Agreement between the Company and Stéphane Boissel, effective October 1, 2018 (incorporated by reference to Exhibit 10.27 to the Company's Annual Report on Form 10-K filed March 1, 2019).</a>
10.31(+)	<a href="#">Employment Agreement between the Company and Adrian Woolfson, effective January 21, 2019 (incorporated by reference to Exhibit 10.28 to the Company's Annual Report on Form 10-K filed March 1, 2019).</a>
10.32(+)	<a href="#">Employment Agreement between the Company and Mark McClung effective as of April 13, 2020 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed August 5, 2020).</a>
10.33(+)	<a href="#">Triple Net Laboratory Lease between the Company and Point Richmond R&amp;D Associates II, LLC, dated May 23, 1997 (incorporated by reference to Exhibit 10.5 to the Company's Registration Statement on Form S-1 (Reg. No. 333-30314), as amended, filed February 24, 2000).</a>

<u>Exhibit Number</u>	<u>Description of Document</u>
10.34	<a href="#">First Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&amp;D Associates II, LLC, dated March 12, 2004 (incorporated by reference to Exhibit 10.20 to the Company's Annual Report on Form 10-K filed February 23, 2005).</a>
10.35	<a href="#">Second Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&amp;D Associates II, LLC, dated March 15, 2007 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed November 4, 2013).</a>
10.36	<a href="#">Third Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&amp;D Associates II, LLC, dated August 1, 2013 (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed November 4, 2013).</a>
10.37	<a href="#">Fourth Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&amp;D Associates II, LLC, dated June 10, 2016 (incorporated by reference to Exhibit 10.33 to the Company's Annual Report on Form 10-K (File No. 000-30171), filed with the SEC on March 1, 2019).</a>
10.38	<a href="#">Fifth Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&amp;D Associates II, LLC, dated July 10, 2017 (incorporated by reference to Exhibit 10.34 to the Company's Annual Report on Form 10-K (File No. 000-30171), filed with the SEC on March 1, 2019).</a>
10.39	<a href="#">Sixth Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&amp;D Associates II, LLC, dated May 11, 2018 (incorporated by reference to Exhibit 10.9 to the Company's Quarterly Report on Form 10-Q filed August 8, 2018).</a>
10.40	<a href="#">Seventh Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&amp;D Associates II, LLC, dated May 20, 2020 (incorporated by reference to Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q filed August 5, 2020).</a>
10.41	<a href="#">Eighth Amendment to Triple Net Laboratory Lease between the Company and Point Richmond R&amp;D Associates II, LLC, dated May 29, 2020 (incorporated by reference to Exhibit 10.5 to the Company's Quarterly Report on Form 10-Q filed August 5, 2020).</a>
10.42	<a href="#">Lease Agreement between the Company and Marina Boulevard Property, LLC dated November 3, 2017 (incorporated by reference to Exhibit 10.21 to the Company's Annual Report on Form 10-K filed March 1, 2018).</a>
10.43	<a href="#">First Amendment to Lease Agreement between the Company and Marina Boulevard Property, LLC dated January 1, 2019 (incorporated by reference to Exhibit 10.37 to the Company's Annual Report on Form 10-K (File No. 000-30171), filed with the SEC on March 1, 2019).</a>
10.44	<a href="#">Open Market Sale Agreement between the Company and Jefferies LLC, dated August 5, 2020 (incorporated by reference to Exhibit 1.1 to the Company's Quarterly Report on Form 10-Q filed August 5, 2020).</a>
10.45†	<a href="#">Amended and Restated Collaboration and License Agreement between the Company and Shire International GmbH, dated September 1, 2015 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed October 30, 2015).</a>
10.46†	<a href="#">Global Research, Development and Commercialization Collaboration and License Agreement between the Company and Biogen MA Inc. (Bioverativ Inc.), dated January 8, 2014 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed May 7, 2014).</a>
10.47†	<a href="#">Letter Amendment to Global Research, Development and Commercialization Collaboration and License Agreement between the Company and Biogen MA Inc. (Bioverativ Inc.), dated December 14, 2015 (incorporated by reference to Exhibit 10.63 to the Company's Annual Report on Form 10-K filed February 18, 2016).</a>
10.48†	<a href="#">Letter Agreement and Waiver between the Company and Biogen MA Inc. (Bioverativ Inc.), dated March 24, 2016 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed May 5, 2016).</a>
10.49†	<a href="#">Collaboration and License Agreement between the Company and Pfizer Inc., dated May 10, 2017 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed August 9, 2017).</a>
10.50*‡	<a href="#">Letter Amendment, dated December 17, 2019, to the Collaboration and License Agreement between the Company and Pfizer Inc., dated May 10, 2017 (incorporated by reference to Exhibit 10.45 to the Company's Annual Report on Form 10-K filed February 28, 2020).</a>
10.51†	<a href="#">Research Collaboration and License Agreement between the Company and Pfizer Inc., dated December 28, 2017 (incorporated by reference to Exhibit 10.40 to the Company's Annual Report on Form 10-K filed March 1, 2018).</a>
10.52†	<a href="#">Amendment No. 1 to Research Collaboration and License Agreement between the Company and Pfizer Inc., dated March 21, 2019 (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q filed May 8, 2019).</a>

<b>Exhibit Number</b>	<b>Description of Document</b>
10.53*	<a href="#">Amendment No. 2 to Research Collaboration and License Agreement between the Company and Pfizer Inc., dated July 31, 2020 (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q filed November 4, 2020).</a>
10.54†	<a href="#">Amended and Restated Collaboration and License Agreement between the Company and Kite Pharma, Inc., dated September 11, 2019 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed November 6, 2019).</a>
10.55*	<a href="#">Collaboration and License Agreement among the Company, Biogen MA, Inc. and Biogen International GmbH, dated February 26, 2020 (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed May 11, 2020).</a>
10.56	<a href="#">Stock Purchase Agreement between the Company and Biogen MA, Inc., dated February 26, 2020 (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed May 11, 2020).</a>
10.57*	<a href="#">Collaboration and License Agreement between the Company and Novartis Institutes for BioMedical Research, Inc., dated July 27, 2020 (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed November 4, 2020).</a>
21.1	<a href="#">Subsidiaries of the Company.</a>
23.1	<a href="#">Consent of Independent Registered Public Accounting Firm.</a>
24.1	<a href="#">Power of Attorney (included on signature page).</a>
31.1	<a href="#">Rule 13a-14(a) Certification of Principal Executive Officer.</a>
31.2	<a href="#">Rule 13a-14(a) Certification of Principal Financial Officer.</a>
32.1*	<a href="#">Certification Pursuant to 18 U.S.C. Section 1350.</a>
101.INS	XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document
104	The cover page from Sangamo's Annual Report on Form 10-K for the year ended December 31, 2020, is formatted in Inline XBRL and it is contained in Exhibit 101

† Confidential treatment has been granted for certain information contained in this document pursuant to an order of the SEC. Such information has been omitted and filed separately with the SEC.

\* Certain portions of this exhibit (indicated by "[\*]") have been omitted in accordance with 17 CFR § 229.601(b).

(+) Indicates management contract or compensatory plan or arrangement.

\* The certifications attached as Exhibit 32.1 accompany this Annual Report on Form 10-K pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, and shall not be deemed "filed" by the Company for purposes of Section 18 of the Securities Exchange Act of 1934, as amended.

#### **ITEM 16 – FORM 10-K SUMMARY**

None.



## DESCRIPTION OF CAPITAL STOCK

References herein to “Sangamo,” “our,” “we,” “us” and the “Company” refer only to Sangamo Therapeutics, Inc. and not to any of our subsidiaries.

### General

Our seventh amended and restated certificate of incorporation, as amended, or the Restated Certificate, authorizes us to issue 320,000,000 shares of common stock, par value \$0.01 per share, and 5,000,000 shares of preferred stock, par value \$0.01 per share.

The following summary description of our capital stock is based on the provisions of the Restated Certificate, our fourth amended and restated bylaws, or the Bylaws, and the applicable provisions of the General Corporation Law of the State of Delaware, or DGCL. This information may not be complete in all respects and is qualified entirely by reference to the provisions of the Restated Certificate, the Bylaws and the DGCL. The Restated Certificate and the Bylaws are filed as exhibits to this Annual Report on Form 10-K to which this Description of Capital Stock is an exhibit.

### Common Stock

Shares of our common stock are the only security of the Company registered pursuant to Section 12 of the Securities Exchange Act of 1934, as amended, or the Exchange Act. The holders of common stock are entitled to one vote per share on all matters to be voted on by the stockholders. Stockholders have no cumulative voting rights. Subject to the preferences of any outstanding shares of preferred stock, the holders of common stock are entitled to receive ratably any dividends our board of directors declares out of funds legally available for the payment of dividends. If we are liquidated, dissolved or wound up, the holders of common stock are entitled to share pro rata all assets remaining after payment of liabilities and liquidation preferences of any outstanding shares of preferred stock. Holders of common stock have no preemptive rights or rights to convert their common stock into any other securities. There are no redemption or sinking fund provisions applicable to the common stock.

### Preferred Stock

Pursuant to the Restated Certificate, our board of directors has the authority, without further action by the stockholders, to issue shares of preferred stock in one or more series. Our board of directors also has the authority to determine or alter the designation, rights, preferences, privileges and restrictions granted to or imposed upon any unissued series of preferred stock, any or all of which may be greater than the rights of the common stock. Our board of directors, without stockholder approval, may issue preferred stock with voting, conversion or other rights that are superior to the voting and other rights of the holders of common stock. The issuance of preferred stock may have the effect of delaying, deferring or preventing a change of control of Sangamo without further action by the stockholders, and may have the effect of delaying or preventing changes in management of Sangamo. In addition, the issuance of preferred stock may have the effect of decreasing the market price of the common stock and may adversely affect the voting power of holders of common stock and reduce the likelihood that common stockholders will receive dividend payments and payments upon liquidation.

### Antitakeover Effects of Provisions of our Restated Certificate, Bylaws and Delaware Law

#### *Our Restated Certificate and Bylaws*

As noted above, our board of directors, without stockholder approval, has the authority under our Restated Certificate to issue preferred stock with rights superior to the rights of the holders of common stock. As a result, the issuance of preferred stock may have the effect of delaying, deferring or preventing a change of control of Sangamo without further action by the stockholders and may adversely affect the voting and other rights of the holders of common stock. Our Restated Certificate also requires that any action required or permitted to be taken by our stockholders must be effected at a duly called annual or special meeting of the stockholders and may not be effected by a consent in writing. Further, our Restated Certificate provides that a special meeting of the stockholders may be called only by our board of directors.

In addition to the provisions noted above, our Bylaws further establish advance notice procedures with respect to stockholder proposals and nomination of candidates for election as directors other than nominations made by or at the direction of the board of directors or a committee of the board of directors. Although our stockholders may amend, repeal or alter our Bylaws by a vote of at least a majority of the outstanding shares of our capital stock entitled to vote, our board of directors may also unilaterally adopt, repeal, alter, amend and rescind our Bylaws by a vote of at least a majority of board of directors. Finally, our board of directors has the ability to elect a director to fill a vacancy created by the expansion of the board of directors or due to the resignation or departure of an existing board member.

These provisions may have the effect of delaying, deferring or preventing a change in control and may also delay or prevent changes in management of Sangamo, which could have an adverse effect on the market price of our stock. These and other provisions are intended to enhance the likelihood of continued stability in the composition of our board of directors and to discourage certain types of transactions that may involve an actual or threatened change of control. However, such provisions could have the effect of discouraging others from making tender offers for our shares and, as a consequence, such provisions also may inhibit fluctuations in the market price of our shares that could result from actual or rumored takeover attempts.

#### *Section 203 of the General Corporation Law of the State of Delaware*

We are subject to Section 203 of the DGCL which regulates acquisitions of some Delaware corporations. In general, Section 203 prohibits, with some exceptions, a publicly held Delaware corporation such as us from engaging in a “business combination” with an “interested stockholder” for a period of three years following the time that the stockholder became an interested stockholder, unless:

- prior to the time the stockholder became an interested stockholder, the board of directors of the corporation approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the number of shares outstanding (but not the outstanding voting stock owned by the interested stockholder) those shares owned by (a) persons who are directors and also officers and (b) employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- at or subsequent to the time the stockholder became an interested stockholder, the business combination is approved by the board of directors and authorized at an annual or special meeting of stockholders, and not by written consent, by the affirmative vote of at least 66 $\frac{2}{3}$ % of the outstanding voting stock which is not owned by the interested stockholder.

Section 203 of the DGCL generally defines a “business combination” to include any of the following:



- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, lease, exchange, mortgage, transfer, pledge or other disposition involving the interested stockholder (in one transaction or a series of transactions) of assets of the corporation having an aggregate market value equal to 10% or more of the aggregate market value of either all of the assets of the corporation or its outstanding stock;
- subject to exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;
- subject to exceptions, any transaction involving the corporation that has the effect, directly or indirectly, of increasing the proportionate share of the stock or any class or series of the corporation beneficially owned by the interested stockholder; and
- the receipt by the interested stockholder of the benefit, directly or indirectly (except proportionately as a stockholder of such corporation), of any loans, advances, guarantees, pledges or other financial benefits, other than certain benefits set forth in Section 203, provided by or through the corporation.

In general, Section 203 defines an “interested stockholder” as an entity or person who, together with the person’s affiliates and associates, beneficially owns, or within three years prior to the time of determination of interested stockholder status did own, 15% or more of the outstanding voting stock of the corporation.

Section 203 of the DGCL could depress our stock price and delay, discourage or prohibit transactions not approved in advance by our board of directors, such as takeover attempts that might otherwise involve the payment to our stockholders of a premium over the market price of our common stock.

### **Forum Selection Bylaw**

Unless we consent in writing to the selection of an alternative forum, the sole and exclusive forum for (1) any derivative action or proceeding brought on behalf of Sangamo, (2) any action asserting a claim of breach of a fiduciary duty owed by any director, officer, other employee or stockholder of Sangamo to Sangamo or to our stockholders, (3) any action asserting a claim arising pursuant to any provision of the DGCL, the Restated Certificate, the Bylaws, or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware, or (4) any action asserting a claim governed by the internal affairs doctrine shall be a state or federal court located within the state of Delaware. However, this provision does not apply to actions arising under the Securities Act of 1933, as amended, or the Securities Act, or the Exchange Act, or any claim for which the federal courts have exclusive jurisdiction.

Unless we consent in writing to the selection of an alternative forum, to the fullest extent permitted by law, the federal district courts of the United States of America shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act.

Any person or entity purchasing or otherwise acquiring any interest in shares of capital stock of Sangamo is deemed to have notice of and consented to the forum selection provisions of the Bylaws.

**Subsidiaries of the Company**

Gendaq Limited (U.K.)

Ceregene Inc. (Delaware)

Sangamo Therapeutics France S.A.S. (France)

**CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

We consent to the incorporation by reference in the following Registration Statements:

- 1 Registration Statements (Forms S-8 No. 333-166220, 333-189621, 333-206173, 333-221827, 333-225552, 333-241033, and 333-249482) pertaining to the Amended and Restated 2013 Stock Incentive Plan, 2010 Employee Stock Purchase Plan, Amended and Restated 2018 Equity Incentive Plan, and 2020 Employee Stock Purchase Plan of Sangamo Therapeutics, Inc., and
- 2 Registration Statements (Form S-3 No. 333-224418) and related prospectuses of Sangamo Therapeutics, Inc.;

of our reports dated February 24, 2021, with respect to the consolidated financial statements of Sangamo Therapeutics, Inc. and the effectiveness of internal control over financial reporting of Sangamo Therapeutics, Inc. included in this Annual Report (Form 10-K) of Sangamo Therapeutics, Inc. for the year ended December 31, 2020.

/s/ Ernst & Young LLP

Redwood City, California  
February 24, 2021

**CERTIFICATION**

I, Alexander D. Macrae, certify that:

1. I have reviewed this annual report on Form 10-K of Sangamo Therapeutics, 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(s) 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(s) 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: February 24, 2021

/s/ ALEXANDER D. MACRAE

Alexander D. Macrae  
President and Chief Executive Officer  
(Principal Executive Officer)

**CERTIFICATION**

I, Sung H. Lee, certify that:

1. I have reviewed this annual report on Form 10-K of Sangamo Therapeutics, 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(s) 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(s) 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: February 24, 2021

/s/ SUNG H. LEE

---

Sung H. Lee  
Acting Principal Financial Officer  
(Principal Financial Officer)

**Certifications Pursuant to 18 U.S.C. §1350, as Adopted  
Pursuant to §906 of the Sarbanes-Oxley Act of 2002**

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, Alexander Macrae, President and Chief Executive Officer of Sangamo Therapeutics, Inc. (the "Company"), and Sung Lee, Acting Principal Financial Officer of the Company, each hereby certifies in such capacity, that, to the best of his knowledge:

- (1) the Company's Annual Report on Form 10-K for the year ended December 31, 2020, to which this Certification is attached as Exhibit 32.1 (the "Report") fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and
- (2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ ALEXANDER D. MACRAE

---

Alexander D. Macrae  
President and Chief Executive Officer  
(Principal Executive Officer)

Date: February 24, 2021

/s/ SUNG H. LEE

---

Sung H. Lee  
Acting Principal Financial Officer  
(Principal Financial Officer)

Date: February 24, 2021

*This certification accompanies the Annual Report on Form 10-K to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of Sangamo Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Form 10-K), irrespective of any general incorporation language contained in such filing. A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sangamo Therapeutics, Inc. and will be retained by Sangamo Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.*