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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-K

(Mark One)

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

For the Fiscal Year Ended June 30, 2020

OR

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

Commission File Number: 001-36370

APPLIED GENETIC TECHNOLOGIES CORPORATION
(Exact Name of Registrant as Specified in Its Charter)

Delaware	**59-3553710**
(State or Other Jurisdiction of Incorporation or Organization)	**(I.R.S. Employer Identification No.)**

14193 NW 119th Terrace, Suite 10, Alachua, Florida 32615
(Address of Principal Executive Offices, Including Zip Code)

(386) 462-2204
(Registrant's Telephone Number, Including Area Code)

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

Title of class	Trading Symbol(s)	Name of exchange on which registered
Common Stock, $0.001 par value	**AGTC**	**Nasdaq Global 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 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 and posted 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 and post such files). Yes ☒ No ☐

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

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

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

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

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

The aggregate market value of the voting common shares held by non-affiliates of the registrant was approximately $81.9 million, computed by reference to the closing sales price of the common stock as reported by the Nasdaq Global Market on December 31, 2019, the last trading day of the registrant's most recently completed second fiscal quarter. The Company has no non-voting common shares.

The number of shares of the registrant's common stock outstanding as of September 2, 2020 was 25,857,883.

DOCUMENTS INCORPORATED BY REFERENCE
None

APPLIED GENETIC TECHNOLOGIES CORPORATION
ANNUAL REPORT ON FORM 10-K
FOR FISCAL YEAR ENDED JUNE 30, 2020

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K, including the sections entitled "Business," "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations," contains forward-looking statements. These statements may relate to, but are not limited to, expectations of our future results of operations, including results and timing of our clinical trials and planned clinical trials, business strategies and operations, financing plans, potential growth opportunities, potential market opportunities and the effects of competition, as well as assumptions relating to the foregoing. Forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified. These risks and other factors include, but are not limited to, those listed under "Risk Factors." In some cases, you can identify forward-looking statements by terminology such as "may," "will," "should," "could," "expect," "hope," "plan," "anticipate," "project," "believe," "estimate," "predict," "intend," "potential," "might," "would," "continue," "seek" or the negative of these terms or other comparable terminology. These statements are only predictions. Actual events or results may differ materially.

There may be events in the future that we are not able to accurately predict or control and that may cause our actual results to differ materially from the expectations we describe in our forward-looking statements. Except as required by applicable law, including the securities laws of the United States and the rules and regulations of the SEC, we do not plan to publicly update or revise any forward-looking statements contained in this Annual Report on Form 10-K after we file it, whether as a result of any new information, future events or otherwise. Before you invest in our common stock, you should be aware that the occurrence of any of the events described in the "Risk Factors" section and elsewhere in this Annual Report on Form 10-K could harm our business, prospects, operating results and financial condition. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance or achievements.

As used herein, except as otherwise indicated by context, references to "we," "us," "our," "AGTC" or the "Company" refer to Applied Genetic Technologies Corporation.

Item 1. BUSINESS

Applied Genetic Technologies Corporation ("AGTC") is a clinical-stage biotechnology company that uses a proprietary gene therapy platform to develop transformational genetic therapies for patients suffering from rare and debilitating diseases. Our initial focus is in the field of ophthalmology, where we continue to progress clinical programs in X-linked retinitis pigmentosa (XLRP) and achromatopsia (ACHM). In addition, we have an additional partnered clinical-stage program in optogenetics, and pre-clinical programs in central nervous system (CNS), and several other ophthalmology, and otology indications. With several important clinical milestones on the horizon, we believe that we are well positioned to advance multiple programs towards pivotal studies as data develops to support their advancement. In addition to our product pipeline, we have also developed broad technological capabilities in the design, construction and manufacture of viral vectors using adeno-associated virus (AAV) technology. Finally, we have augmented these capabilities through multiple academic and commercial collaborations which provide us with additional expertise.

Our Strategy

Our objective is to become a leader in developing and commercializing gene therapy treatments for patients with severe diseases, with an initial focus in ophthalmology, and thereby provide a better life for patients with these diseases. Our strategy to accomplish this goal is to:

- **Develop and commercialize gene therapies in orphan ophthalmology.** Our lead product candidates are treatments for the severe orphan eye diseases XLRP and ACHM. Given the severity of these diseases and the current lack of treatment options, a one-time-treatment alternative that corrects the underlying genetic defect would provide long-term value for patients, their families and the healthcare system more broadly.

- **Expand our position in ophthalmology.**
  - **Continue our leadership position in orphan ophthalmology.** We have developed significant experience in the orphan ophthalmology space through our ongoing work on XLRP and ACHM, our previous experience in X-linked retinoschisis (XLRS) and Leber's Congenital Amaurosis Type 2 (LCA2) and in our preclinical ophthalmology programs. We are applying this knowledge to additional pre-clinical programs.
  - **Leverage capabilities into larger ocular market opportunities:** the insight and understanding gained in connection with our inherited retinal disease programs enhance the capabilities to apply our technology to larger ophthalmology indications such as our pre-clinical program in dry age-related macular degeneration, or AMD.
  - **Seek opportunities for strategic partnerships and acquisitions in ophthalmology gene therapy.** In February 2017, we entered into a collaboration agreement with Bionic Sight, LLC, or Bionic Sight, to develop an optogenetic product candidate for patients with advanced retinal disease that leverages our deep experience in gene therapy and ophthalmology and Bionic Sight's innovative neuro-prosthetic device and algorithm for retinal coding. We believe there may be additional opportunities for us to partner with companies and academic groups in ophthalmology and more generally. We expect that our breadth of experience in research, manufacturing, clinical and regulatory matters will help us to identify and execute in-licenses, co-development agreements, intellectual property acquisitions or manufacturing agreements that could further extend our leadership position in ophthalmology gene therapy.
- **Extend our expertise in adeno-associated virus, or AAV, vector design, manufacturing and delivery.** We believe that our deep understanding of our target indications and our robust internal expertise in viral vector design gives us a significant competitive advantage. This understanding includes the identification of novel capsids and the optimization of genes and promoters, physical vector delivery, vector manufacturing, clinical trial design and clinical trial conduct. We intend to continue to devote substantial resources both internally and with external research collaborations to identify novel next generation capsids, develop optimized promoters to enhance product performance, and identify opportunities for continuous improvement in our manufacturing process. We are also expanding our research and discovery capabilities to further enhance our ability to develop next generation products, including the delivery of other payloads such as antibodies and RNA.
- **Expand our manufacturing capabilities.** We continue to invest in the development and expansion of our internal manufacturing capabilities with a particular focus on enhancing the productivity, scalability, and purity of our manufacturing platform. Our late stage commercial manufacturing process has demonstrated it can produce thousands of ophthalmology doses from a 50L bioreactor with an approximately 90% full to empty capsid ratio and greater than 97% purity levels as demonstrated in multiple batches completed by our personnel and two batches completed at a CDMO. We have a fully functional process development and pilot manufacturing facility used to manufacture early stage research materials, and as we advance further into clinical development, we plan to further develop our internal manufacturing capabilities. We have decreased our dependence on a single contract manufacturer by qualifying and contracting with multiple backup contractors. Further, we continue to invest in process and analytical improvements that have resulted in a ten-fold increase in manufacturing yields potentially resulting in up to a 90% reduction in cost of goods compared to other approaches as well as robust quality control enhancements that are amenable to characterization of commercial products. We believe that these investments will facilitate the more rapid advancement of our product candidates through regulatory approval while reducing risk and will enhance the therapeutic and commercial potential of our gene therapy platform.

- **Pursue indications outside of ophthalmology with high unmet medical need and strong probability of a streamlined clinical, regulatory and commercial pathway.** We will continue to use a consistent framework to focus on diseases for which:
  - the underlying genetic defect is well characterized,
  - the underlying genetic defect can be addressed by approaches amenable with the use of AAV, such as gene replacement, RNA targeting sequences, or antibody delivery, and
  - predictive animal models exist and for which clinical endpoints are objective and accepted by regulatory authorities.

We believe that focusing on these types of indications will enable us to obtain data more rapidly and accelerate clinical studies and regulatory approval of our product candidates. Given the relatively low prevalence of patients who have these orphan diseases and the strong key opinion leader communities and patient advocacy groups that support them, we also believe that these markets can be served with a small, targeted commercial infrastructure. Our research in the fields of otology and CNS are examples of this strategy.

Our Initial Focus in Ophthalmology

Sight is critical to the human experience. Many people fear blindness more than premature death. Consequently, we have initially decided to focus our expertise in gene therapy on orphan diseases in ophthalmology. These orphan indications have patient populations that are small enough to allow for clinical trials on a manageable scale but have a sufficient prevalence to provide substantial commercial opportunity. By focusing initially on orphan ophthalmology product candidates, we are also able to leverage our experience and develop strong relationships within the relevant scientific and medical communities. Our clinical trials are conducted mainly at academic test centers and by working with the principal investigators and surgeons at these test centers, we have realized a number of important synergies.

Our most advanced product candidates consist of three ophthalmology development programs across two targets: XLRP caused by mutations in the RPGR gene, and ACHM, caused by mutations in either the CNGB3 gene or the CNGA3 gene. These inherited orphan diseases of the eye are caused by mutations in single genes that significantly affect visual function and currently lack effective medical treatments.

- XLRP is a disease of the rod and cone photoreceptors characterized by progressive degeneration of the retina, which can lead to total blindness in adult men. According to a published study, the incidence rate for retinitis pigmentosa is about one in 4,000 people and we estimate that there are about 200,000 patients in the United States and Europe combined. It is estimated that about ten percent, or 20,000, of these people have XLRP. We have completed targeted enrollment of 28 patients in our Phase 1/2 clinical trials for our XLRP product candidate and are currently planning our next phase of clinical trial activities, which is informed by end-of phase 2 feedback received from the FDA.
- ACHM is characterized by the absence of cone photoreceptor function, resulting in extremely poor visual acuity, extreme light sensitivity, day blindness and complete loss of color discrimination. According to a published study, the incidence rate for ACHM is approximately one in 30,000 people, and we therefore estimate that there are about 27,000 patients in the United States and Europe combined**.** Of these patients, about 75% to 85% have the form of disease caused by mutations in the CNGB3 gene or the CNGA3 gene. We have completed planned enrollment of adult patients in our Phase 1/2 clinical trials for both our ACHM CNGB3 product candidate and our ACHM CNGA3 product candidate and continue to enroll pediatric patients in the higher dose groups.

In addition to these two lead ophthalmology programs, we have an additional clinical program in collaboration with Bionic Sight to develop an optogenetic product candidate for patients with advanced retinal disease.

Recent Corporate Milestones

In October 2019, we entered a strategic collaboration with Otonomy, Inc. to develop and commercialize gene therapy for congenital hearing loss.

In January 2020, we reported positive interim six-month data for centrally dosed patients in our Phase 1/2 XLRP clinical trial.

In January 2020, we reported encouraging interim six-month data from the dose-escalation cohorts in on-going ACHM A3 and B3 Phase 1/2 clinical trials.

In February 2020, we announced completion of enrollment in the two highest dose groups of our XLRP Phase 1/2 clinical trial.

In February 2020, we completed a public offering of our common stock for gross proceeds of $37.4 million.

In March 2020, we completed enrollment in all adult dose groups of our ACHM Phase 1/2 clinical trials.

In June 2020, we launched a nationwide mobile vision testing program for patients enrolled in our ongoing Phase 1/2 clinical trials.

In June 2020, we announced significant productivity and quality enhancements in our manufacturing process.

In June 2020, AGTC was added to the Russell 3000® and 2000® Indexes.

In July 2020, we announced the updated development plan for our XLRP clinical program.

Our Strengths

We believe that the combination of our science and technology expertise as well as our product development know-how positions us well to be leaders in the gene therapy field. We believe that our strengths include:

- Product candidates in clinical development, including three ongoing Phase 1/2 clinical trials with enough capital to complete enrollment and initial data analysis on all of these trials and initiate the next phase of development for our XLRP program;
- Topline interim six-month data from our Phase 1/2 clinical trial for our XLRP product candidate that showed an acceptable safety profile and evidence of increase and expansion in central visual sensitivity, encouraging improvements in visual acuity, and preliminary results from quality of life surveys that support meaningful impact on patients' lives;
- Comprehensive End-of-Phase 2 feedback from the FDA that informed our revised framework for moving the XLRP candidate into the next stages of development;
- Significant relationships with key opinion leaders in the fields of ophthalmology, otology, CNS and AAV production;
- Robust preclinical product development pipeline including ophthalmology, otology and CNS disorders;
- A collaboration with Bionic Sight for the development of an optogenetic gene therapy and a neuro-prosthetic device with an algorithm for advanced retinal coding;
- A partnership with Otonomy, a company with deep expertise in neurotology, for the development of our joint otology program;
- Proprietary gene therapy manufacturing system, capable of making significant quantities of high quality viral vectors in accordance with Good Manufacturing Practice, or GMP, standards as

successfully demonstrated in seven different clinical trials, and has demonstrated a 10-fold increase in productivity as a result of our internal development efforts, which means that we can produce thousands of ophthalmology doses from a 50L bioreactor with greater than 90% full capsids and 97% purity at a cost of goods that is potentially as much as a 90-fold improvement over other methods;

- Product candidates, using our AAV vector technology platform, that have demonstrated they are generally safe and well tolerated for the indications we are developing;
- Technical expertise in analytical techniques, synthetic promoter development, engineered and optimized capsids and specialized formulation and delivery techniques; and
- Capabilities in clinical operations and medical affairs to power our multiple clinical programs forward.

Our Gene Therapy Platform

Although the concept of gene therapy is relatively straightforward, the process of developing and manufacturing vectors capable of delivering genetic material safely into a patient's own cells is highly technical and demands significant expertise, experience and know-how. Our approach to gene therapy product development is built on our core competencies in four key areas: vector selection, design, manufacturing and delivery, each of which is described in further detail below. One of our key capabilities is our depth of understanding of the complex interplay between the clinical disease, the cells in the patient's body that need treatment, the selection of a capsid and a promoter, the design of the gene construct and the physical administration method. We have spent more than 19 years conducting research on the best combinations of these elements with the aim of developing safe and effective product candidates.

Vector Selection

The success of a gene therapy platform is highly dependent on the vector selected. Our platform is based on the use of a non-replicating AAV to deliver the correct DNA directly to the nucleus of the cells affected by the disease. As an underlying platform, we believe that AAV vectors are particularly well suited for treating our target diseases and have advantages over other viral vectors, such as adenovirus, herpes virus and lentivirus. These advantages include:

- AAV is a small, simple non-enveloped virus with only two native genes, which makes the virus easy to engineer as an effective vector;
- AAV is inherently stable and resistant to degradation;
- AAV vectors can deliver functional genes in a manner that supports long-term production of protein, leading to sustained therapeutic effect, without altering the patient's native DNA;
- AAV vectors have a demonstrated safety profile across multiple human clinical trials in several indications; and
- AAV vectors are versatile, having the ability to carry therapeutic gene sequences of up to 4,000 base pairs in length into a patient's cell. As more than 90% of human genes have coding sequences less than 3,000 base pairs in length, this allows AAV vectors to be used in a wide variety of indications.

Vector Design

After selection of the vector type, there are other critical factors to be considered to maximize the safety and efficacy of the final gene therapy product:

- Gene of Interest: The first step in vector design is to identify either the therapeutic protein that we want the patient's own cells to produce (which is expressed from a DNA sequence that defines the gene of interest), or other cargo content, such as gene editing components or an RNA targeting sequence. In many cases the DNA sequence must be engineered to be stable during manufacturing and delivery.

- Promoter: Production of the protein in the cell requires a promoter, which is a genetic element that drives expression. Certain promoters function well only in certain cell types, whereas other promoters function well in almost any cell type. We make our selection by comparing different promoters in the specific type of cells that are affected in each disease target, ideally in an animal whose physiology is close to that of humans, to find the promoter that best enables production of therapeutic levels of protein in that cell type. We have on-going internal and external research efforts to design promoters that optimize therapeutic constructs for maximum expression with a smaller size, better expression and increased cell specificity.
- Capsid: after the promoter and gene of interest are selected, these elements must be packaged into an AAV capsid. There are 10^8 to 10^9 variations of AAV capsids with different abilities to bind to and enter varying cell types. Not only do we engineer these capsids in-house, we also collaborate with commercial and academic researchers to develop novel capsids that efficiently enter the type of cells that are affected by each of our targeted diseases.

Vector Manufacturing

We have built an rAAV manufacturing platform agnostic to indication, dosing requirements or market size, with demonstrated ability to generate thousands of ocular doses in modest scale stirred tank bioreactors. This scalable, proprietary, high-yield vector manufacturing process can address the most demanding material needs with minimized scaling needs. The system is turnkey, employing robust cell lines that are well characterized and have been vetted by regulatory authorities in the United States, Canada and Europe. The upstream process is reproducible and generates vectors characterized by greater than 50% of all capsids containing full-length, target gene sequences while the downstream process further enhances this resulting in excess of 90% full capsids. The companion analytical platform for process and material characterization is comprised of over 35 product-specific assays developed and transferred to vendors, consistent with regulatory requirements for clinical development. The full suite of characterization assays has transitioned to validation activities to support regulatory approval of the production and characterization platforms. Large biotech partners have successfully transferred our technology into their facilities, and even transferred the process themselves to third party Contract Manufacturing Organizations (CDMOs), demonstrating an unparalleled robustness. We have employed an aggressive risk mitigation approach to manufacturing activities for our internal pipeline, resulting in technology transfer of our platform to multiple redundant CDMOs. Recent process improvements advance our upstream productivity over ten-fold relative to earlier versions of the process (figure below), and over 50-fold relative to traditional transient transfection, employing all scalable operations which have been optimized to provide a final product substantially free of process residuals, often below the quantification limits of our sensitive assays.

Our manufacturing process has been reviewed by the United States Food and Drug Administration, or the FDA, Health Canada, the Irish Medicines Board and the Israeli Ministry of Health and has been authorized for production of clinical trial material for use in clinical trials in the United States, Canada, Europe and Israel. We have successfully manufactured clinical trial material for seven different indications using three different CDMOs, and believe we are the only AAV gene therapy company with this level of experience. Our staff have utilized our state-of-the-art process development facility to adapt our process across multiple bioreactor platforms and scales, demonstrating a robust and flexible platform capable of integrating into diverse partner, manufacturing and commercial facility environments.

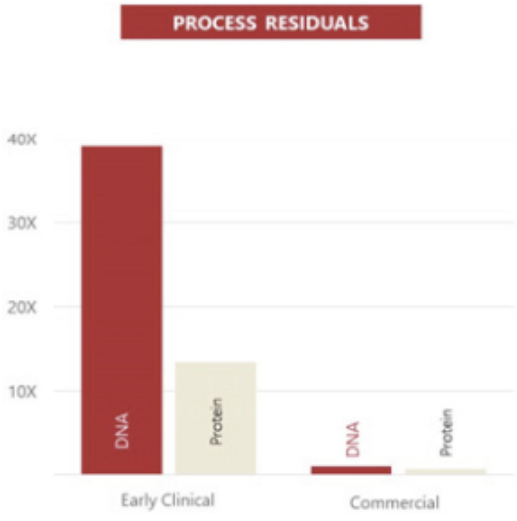
We own or have licensed 37 patents and pending patent applications covering our manufacturing technology. We believe that our core competency and intellectual property estate in vector manufacturing provide a key differentiating element of our gene therapy platform.

The complexity of gene therapy manufacturing and lack of dedicated infrastructure to support it have historically resulted in poor reproducibility and lack of reliability in meeting material needs beyond the early human clinical setting. rAAV vector manufacturing has been limited by inefficient constructs, poor scalability, inadequate yields

and insufficient purity. Our focus is to develop an integrated production and testing platform capable of meeting both clinical and commercial needs and we are committing substantial resources in this area. Important features of our capabilities are set forth below.

- Our propriety platform for AAV production generates high quality rAAV vectors with high packaging fidelity, high infectivity and low empty particles across multiple serotypes.
- Our AAV production system generates high volumetric productivities and has achieved more than 10-fold improvement in productivity compared to other manufacturing formats.
- We have adapted our herpes simplex virus, or HSV, helper manufacturing system to multiple vendors' single use bioreactors, demonstrating robustness and flexibility while removing scale and format limitations attendant with adherent cell culture.
- We have optimized purification and formulation activities to yield multiple rAAV serotypes in a dose-ready form with exceptional purity at previously unattainable genomic concentrations.
- Our integrated testing platform has generated over 35 product-specific characterization assays that have been successfully transferred for the evaluation of HSV helpers and AAV vectors at contract testing organizations.
- The robust cell substrates we employ are well characterized and have been reviewed in several regulatory submissions in the U.S., Canada, Israel and Europe.
- Our ability to successfully transfer the technology to multiple contract manufacturing organizations as well as collaboration partners demonstrates the robustness of our manufacturing process.

Taken together, we believe that the efficiency, productivity, scalability, characterization and regulatory definition of our proprietary rAAV manufacturing platform uniquely position us to quickly transition from early phase human clinical trials to late phase, BLA-enabling data in all our clinical programs. We are currently at commercial scale for our orphan ophthalmology programs due to our high productivity that enable us to achieve thousands of doses from a small bioreactor. As such, in the near term, it is more efficient for us to pursue a hybrid strategy where we have developed and optimized the manufacturing process and leverage a CDMO's investment in capital equipment when needed. Productivity (below, left) has increased 55-fold between early clinical and commercial processes at scale, while process residuals (below, right) have decreased nearly 40-fold (DNA) or greater than 10-fold (protein).



Vector Delivery

Our gene therapy platform allows for vector delivery by a variety of methods, and we select the method that is best suited for the disease and cell type that we are targeting.

In ophthalmology, the product candidate can best be delivered to cells in the eye by either injecting the product candidate under the retina, a sub-retinal injection or by injecting the product candidate into the vitreous of the eye, an intravitreal injection. We are using sub-retinal injection as the method of delivery for our XLRP and ACHM product candidates in our ongoing clinical trials and have developed an extensive training program for surgeons in order to assure consistent delivery across patients. Bionic Sight is using an intravitreal injection as the method of delivery for the optogenetic product candidate.

Established surgical techniques used to introduce AAV in otology indications include microinjection into the cochlea via an apical cochleostomy or through the round window membrane. Like the eye, the inner ear sensory organ – the organ of Corti – is bathed by fluid-filled spaces, enabling accessible vector administration.

For each product candidate identified in our CNS pre-clinical programs, we will determine the optimal delivery approach to safely administer the product candidate and ensure optimal therapeutic effect.

Our Product Candidate Pipeline

Our most advanced product candidates address ophthalmology indications XLRP, ACHM B3, and ACHM A3, which are orphan diseases of the eye that are caused by mutations in single genes, significantly affect visual function starting at birth and currently lack effective medical treatments. Ophthalmology is attractive to us as a clinical stage company because treatments for diseases affecting vision have clearly defined, objective clinical endpoints with validated measurement tools that are accepted by regulatory authorities. Other orphan drug companies have spent considerable time and resources working with regulatory authorities to identify acceptable clinical endpoints and develop measurement tools in rare diseases with limited epidemiology data available. In ophthalmology there are four accepted endpoints—visual acuity, visual fields, contrast sensitivity and color vision—that are well understood by clinicians. In addition, the FDA consistently applies these endpoints and works with industry to provide guidance on how much improvement is required for clinical relevancy. We believe that these endpoints could help accelerate the process of clinical study and regulatory approval for our ophthalmic product candidates. We have also been encouraged by guidance from FDA for rare and inherited retinal disease that we believe signals the agency's willingness to work collaboratively on novel clinical design and novel endpoints that could help advance products to patients more efficiently.

Our Lead Programs

X-linked Retinitis Pigmentosa (XLRP)

Retinitis pigmentosa is an inherited retinal disease with progressive loss of vision, meaning children are born with defective genes that cause poor visual function that significantly affects daily activities and worsens over time. XLRP is commonly first observed in boys and young men who notice problems with vision under low light conditions, or night blindness, followed by a restriction of peripheral visual fields, or tunnel vision, leading to poor central vision and eventually to total blindness.

The incidence rate for retinitis pigmentosa is about one in 4,000 people, according to a published study, and we estimate that there are about 75,000 people in the United States and 125,000 people in Europe with retinitis pigmentosa, or 200,000 people combined. According to a published study, about 10% of cases of retinitis pigmentosa are XLRP, from which we therefore estimate that there are about 20,000 persons with XLRP in the United States and Europe combined.

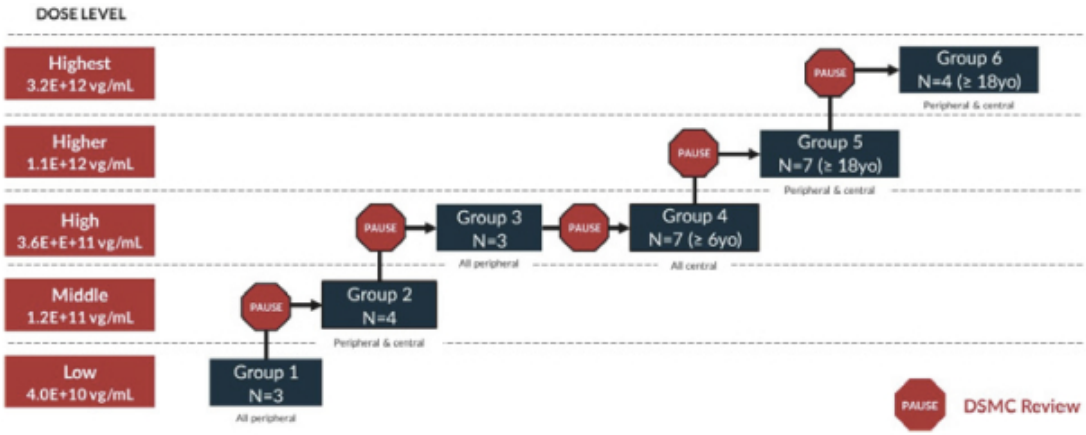
Our XLRP product candidate

Our gene therapy approach to the treatment of XLRP involves using an AAV vector to insert a functional copy of the RPGR gene into the patient's photoreceptor cells. Our XLRP product candidate, AGTC-501, contains an optimized and stabilized RPGR gene and a promoter that have been shown in preclinical studies to drive efficient gene expression in primate rods and cones, as well as maintain photoreceptor function and delay disease progression in dog and mouse models of XLRP. In addition, published non-human primate, or NHP, studies have demonstrated that AGTC's proprietary AAV capsid has as much as twice the transfection efficiency in photoreceptors as is shown by capsids used in competing programs.

Clinical development

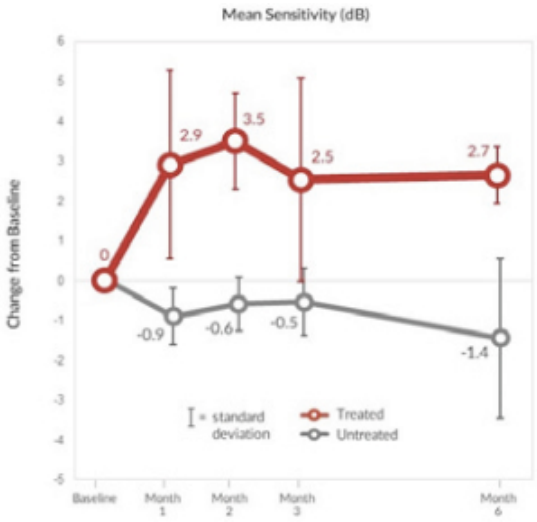
On January 9, 2020, AGTC released interim data from its on-going XLRP Phase 1/2 dose escalation trial, conducted at multiple clinical sites in the U.S. that specialize in inherited retinal diseases. The clinical protocol was designed as a dose escalation trial to evaluate our product candidate in XLRP patients at five dose levels, spanning a 100-fold range, and included targeted enrollment of 28 patients in the dose escalation and expansion portions of the trial. This design is intended to provide us with a robust set of safety and biologic activity data with which to inform our next stage of clinical development. The primary endpoint of this clinical trial is safety, and interim data have shown that the XLRP product candidate thus far continues to show a favorable safety profile and is well tolerated. In addition to safety, this trial is measuring biologic activity by assessing changes in several measures of visual function and quality of life. Durable and meaningful improvements in central visual sensitivity and encouraging improvements in visual acuity were reported through Month 6.

XLRP Phase 1/2 Trial Design and Dosing Schedule

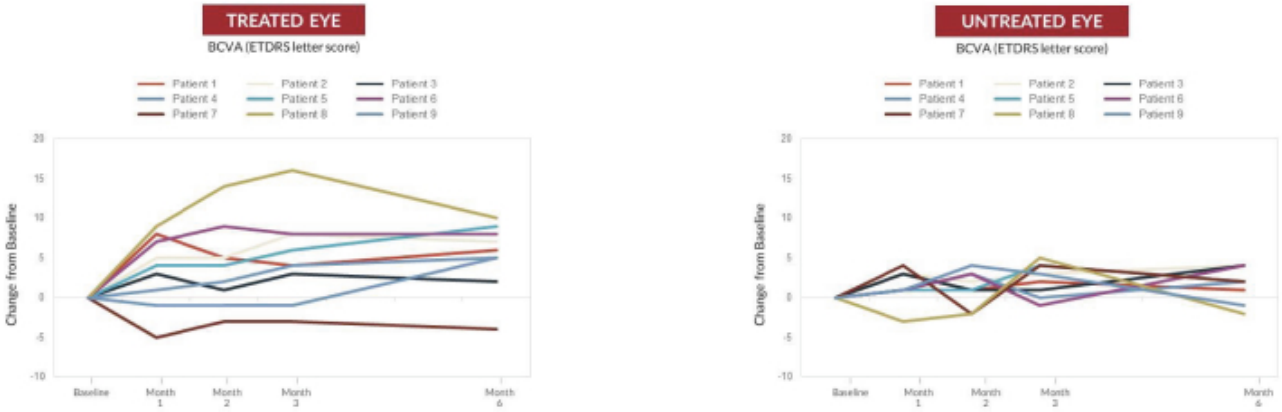


On January 28, 2020 at our Corporate Research and Development Day, we provided more data from the XLRP Phase 1/2 trial to include topline six-month data in 10 patients from the dose escalation Groups 1 through 3, as well as six-month data from 7 patients in the dose expansion Group 4. This data showed a favorable safety profile for all patients and stability of visual function in peripherally dosed patients. The presentation then focused on the nine centrally dosed patients, of which 4 of 8 evaluable patients showed an improvement of visual sensitivity relative to baseline, and 7 of 9 showed positive trend towards improvement in BCVA. Additionally, to date, none of the patients now enrolled in our trial have needed re-administration of steroids to treat secondary inflammation as reported in other XLRP trials. Summaries of the mean sensitivity and BCVA data are provided below.

Mean sensitivity change from baseline in responder sub-group (N=4) from Groups 2 & 4 at Month 6

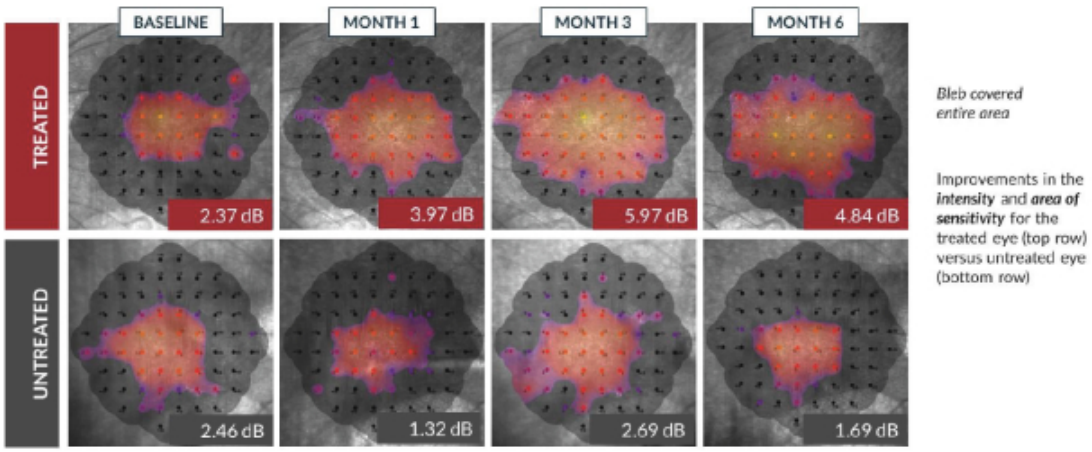


BCVA Change from Baseline in Groups 2 & 4 (N=9) at Month 6

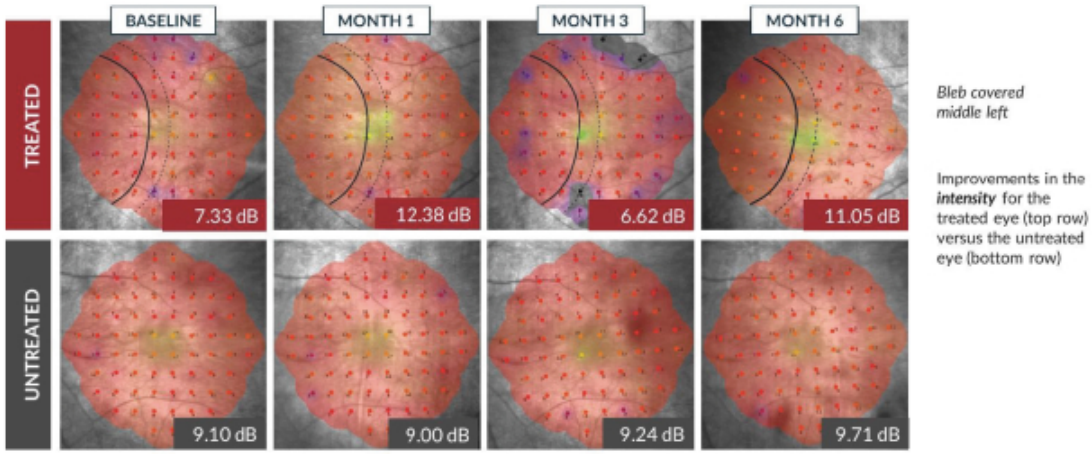


In addition to presenting the summary data for mean sensitivity, the company also reviewed individual heatmaps of all patients, which showed that in many cases, not only did patients experience an increase in mean sensitivity, but also, as illustrated below, the area of the retina responding to light expanded, which is an important component to the patient's quality of life.

MAIA Microperimetry Heatmap from Group 2 Representative Example



MAIA Microperimetry Heatmap from Group 4 Representative Example



Estimated bleb boundary is delineated with solid black line, with dashed line representing 2-degree extension.

On February 19, 2020, AGTC announced that it had met its enrollment target, including dosing of patients in the two highest dose groups, in its Phase 1/2 clinical trial for its XLRP product candidate.

On July 22, 2020, based on comprehensive written feedback received from the FDA on its End-of-Phase 2 submission, AGTC announced its updated clinical development plan for the XLRP product candidate, which includes immediate randomized, masked expansion of the current Phase 1/2 trial, and a planned start of a Phase 2/3 clinical trial during the first quarter of calendar year 2021.

The Company received FDA feedback across three major areas: CMC, pre-clinical and clinical.

Based on feedback on the CMC section, the Company is adjusting the timing of its clinical trial material comparability study to complete this important analysis prior to the initiation of our Phase 2/3 trial. This study will compare the clinical trial material produced under the new manufacturing process to material used in the Phase 1/2 trial. In the Phase 2/3 trial, the Company plans to utilize clinical trial material prepared with its

proprietary manufacturing process, updated since the Phase 1/2 trial, that achieves significant productivity and quality enhancements. The Company is also incorporating the FDA's recommendations into our starting material and lot release testing plans, as well as our approach to process validation. None of these CMC changes, we believe, will affect our overall timeline.

Based on feedback on the pre-clinical section, AGTC has no plans at this time to conduct any additional nonclinical studies before initiation of the Phase 2/3 clinical trial.

Based on feedback on the clinical section, the Company has made several changes, adjusting the clinical endpoint for visual sensitivity, including two masked active arms in the Phase 2/3 study and extending analysis time prior to dosing the contralateral eye. In addition, in order to collect the strongest data set possible, the Company has decided to add a functional mobility test as one of several supplemental endpoints. The Company expects to begin this trial during the first quarter of calendar year 2021 and provide results from a six-month interim analysis during the third quarter of calendar year 2022. The data from this analysis will also be reviewed with the FDA to guide forward clinical development.

AGTC previously presented its microperimetry data as a change, relative to baseline, in mean sensitivity within the treated bleb region, that exceeded the inherent statistical variability of the microperimetry test itself (approximately 1.9 decibels (dB)) and believes this is an important measure of clinical benefit since it captures changes in the full treatment area. As illustrated by the mean sensitivity change from baseline in the responder sub-group figure above, an early and sustained increase in mean sensitivity was observed among responders through Month 6. The mean difference between responders' treated and untreated eyes was 4.1 dB at Month 6, including a 2.7 dB mean increase in treated eyes which exceeded the 1.9 dB threshold. For context, a change of 3 dB represents a doubling in sensitivity to light.

A second way to analyze microperimetry data is to use pointwise analysis i.e., identify points with a given increase or decrease in sensitivity at each locus. To be consistent with communications from the FDA and mirror how other research groups in the XLRP gene therapy space are analyzing visual sensitivity data, we have adjusted our definition of a responder for the visual sensitivity endpoint to a change from baseline in visual sensitivity of at least 7 dB in at least 5 loci at Month 12, which is intended to represent a clinically meaningful benefit. Responder rates from each active arm will be compared to responder rates in the control arm.

We present below our re-analysis of our existing MAIA microperimetry data from centrally dosed Group 2 and Group 4 patients at Month 6. In addition, we are also reporting for the first time the same analysis from the Group 5 dose group at Month 6.

When the patients are analyzed using our new definition of responder, 7 of the 15 centrally treated patients from all three dose groups combined had at least 5 loci that increased by at least 7 dB – 6 patients at Month 6 and 1 patient at Month 3. Focusing on the Group 5 dose group, a dose level that we are planning to move forward, in this analysis 4 of 7 patients met the response criterion. Of note, if we apply the planned inclusion/exclusion criteria for baseline visual sensitivity in the upcoming Phase 2/3 trial to the Group 5 patients, one patient would be removed, such that the responder rate in this case would be 4 of 6, or 67%. We do not currently have a complete set of Month 12 data available nor do we have a control arm in the Phase 1/2, both of which will be part of the planned Phase 2/3 trial and necessary to evaluate efficacy.

Dose Group	C36 Change ³7dB @ ³5 Loci At Month 6	Number of responders
2	**Yes**	1/1
4	No	2/7
	Yes	
	No	
	No	
	Yes	
	No	
	No	
5	**Yes***	4/7
	Yes	
	Yes	
	No	
	No	
	No	
	Yes	

* Month 3 data

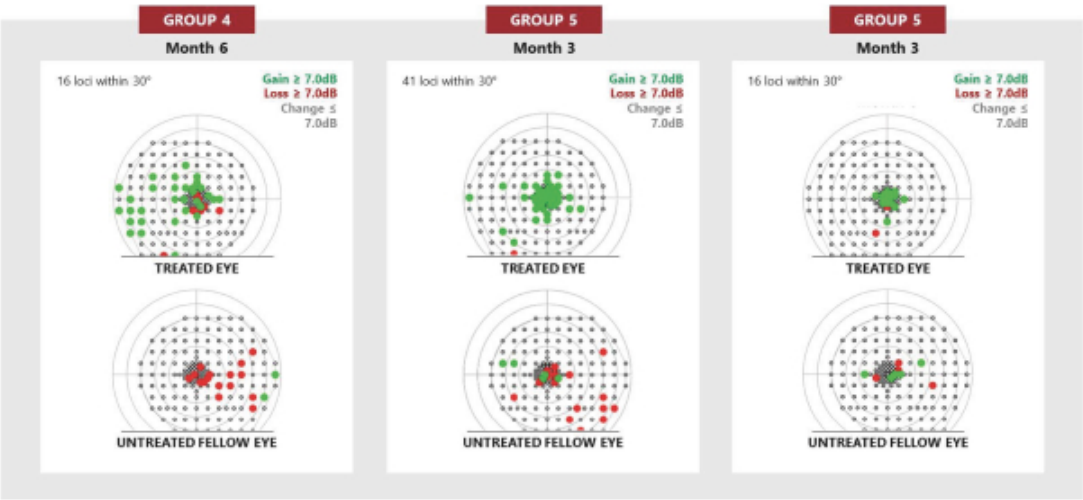
Others have reported visual sensitivity using a static full field perimeter called the Octopus perimeter. This device measures visual sensitivity across a wider area of the retina, as compared to the microperimeter, to include areas outside the macula and into the periphery. As with the MAIA microperimeter, the Octopus perimetry data can be analyzed two ways. First as the mean change over the entire grid, or over a specific portion of the grid. Second using pointwise analysis i.e., plotting the points on the perimetry grid and indicating an increase or decrease in sensitivity for a given locus. We provide our Octopus data analyzed both ways below.

While challenging to compare across research groups due to variations in grid patterns used, and bleb placements achieved, we believe our analysis of mean change in sensitivity, reported below, shows our XLRP product produces comparable results to those reported by others using the Octopus perimeter. We believe that since the injection of the product is in the central macular region microperimetry provides a higher resolution measurement of retinal sensitivity in the target area.

Dose Group	N	Treated Eye - Untreated Eye Within Bleb Mean Sensitivity at Month 6 (dB)	p Value
2	2	+0.87 (-1.24, +2.97)	—
4	6	+1.84 (-0.5, + 4.19)	0.175
All Central	8	+1.60 (-0.04, +3.25)	0.110

We also analyzed our Octopus perimetry data using pointwise analysis i.e., plotting the points on the perimetry grid and indicating an increase or decrease in at least 7 dB for a given locus. We provide representative data from three patients comparing the treated and untreated eyes, and which represent those with at least 5 loci increasing by at least 7 dB at the timepoints indicated.

Examples 1-3

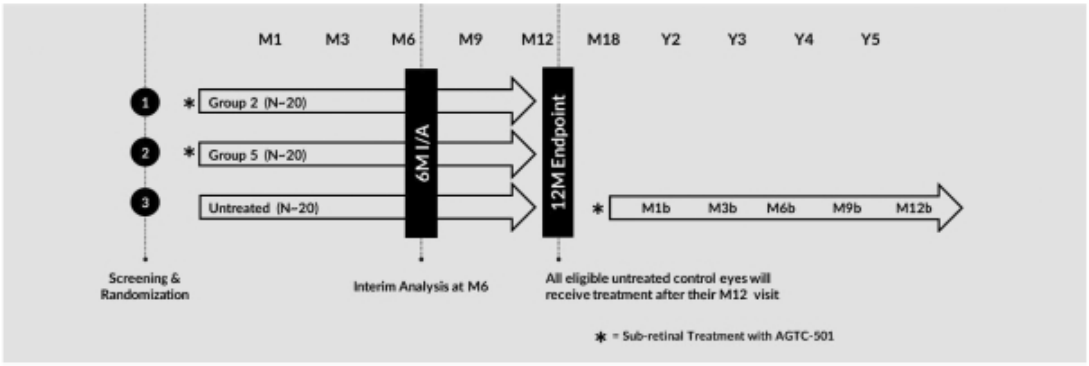


This information, taken as a whole, reaffirms our conclusion that our data are at least comparable to that of other disclosed data from XRLP gene therapy trials. AGTC remains on track to provide the next interim analysis data readout in the second half of 2020, which will include Month 12 data from Groups 2 & 4, and a more complete month six dataset from the two higher dose groups five and six.

For ophthalmology studies in general, and especially with low sample sizes, there is often a concern of possible patient bias that could influence endpoints due to the psychometric nature of the test protocols, and that to the extent possible, this should be minimized. The Company received feedback on this issue of bias from the FDA and has revised its proposed Phase 2/3 trial protocol to minimize this risk. The revised protocol provides for enrollment of approximately 60 patients, and will include two masked active arms, in addition to an untreated control arm. The dose concentrations used will be 1.2E+11 vg/mL (Group 2 in the Phase 1/2 trial design) and 1.1E+12 vg/mL (Group 5 in the Phase 1/2 trial design). The Company has added an important secondary endpoint, a standardized functional mobility test, which captures real-world impacts on XLRP patients by objectively assessing changes in patient mobility that are secondary to improvements in vision resulting from therapeutic intervention. In addition, other secondary endpoints will include BCVA, full-field sensitivity threshold (FST) and changes in contrast sensitivity.

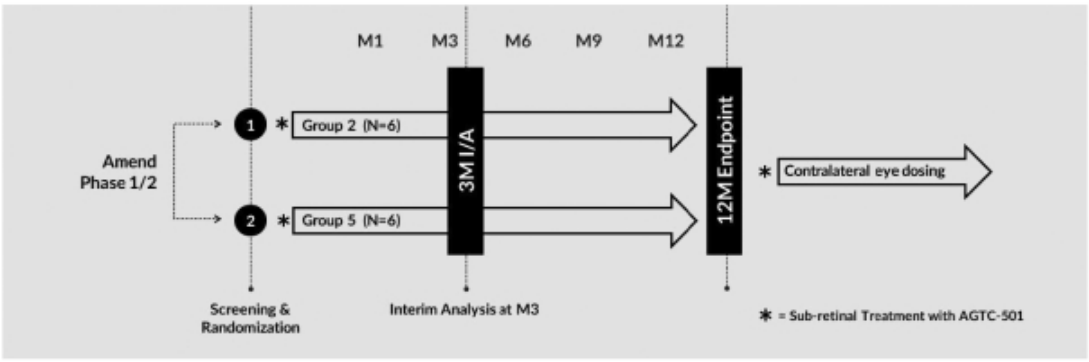
Based on the projected responder rate, using our new primary endpoint analysis described above, from the Phase 1/2 trial, the Phase 2/3 trial is intended to determine a difference between either active arm and the control arm. AGTC expects to begin this trial during the first quarter of calendar year 2021 and provide results from a 6-month interim analysis during the third quarter of calendar year 2022. The Company plans to submit a 6-month interim analysis of the data to the FDA to obtain feedback on the Company's development plan to support approval. Based on this feedback, we may modify the final trial design, enrollment numbers and statistical analysis plan. We also want feedback from the FDA regarding finalization of the dose selection for the treatment of the contralateral eye based on this 6-month interim data. After any adjustments made based on this feedback, if the totality of the data collected at 12 months shows a compelling benefit-risk balance, we believe it may support the submission of a BLA filing. Timing of the reporting of this data may be impacted by future effects of the COVID-19 pandemic on clinical trial enrollment.

Outline of Phase 2/3 Trial



While not required prior to initiating the Phase 2/3 trial, at its discretion, the Company plans to expand its on-going Phase 1/2 trial to include 12 additional patients that will be masked and randomized to doses of 1.2E+11 vg/mL (Group 2 in original trial plan) and 1.1E+12 vg/mL (Group 5 in original trial plan). The Company expects to begin dosing in the fourth quarter of calendar year 2020 and provide results from the three-month interim analysis in the fourth quarter of calendar year 2021, subject to potential effects of the COVID-19 pandemic on clinical trial enrollment. We expect these data to provide a near-term assessment of the correlation between changes in visual sensitivity and the patients' ability to navigate the functional mobility course, the additional secondary endpoint we plan to measure in the Phase 2/3 trial.

Outline of Expanded Phase 1/2 Trial



Achromatopsia (ACHM)

ACHM is an inherited retinal disease, meaning that children are born with the defective gene that causes poor visual function, which significantly affects daily activities. ACHM is present from birth and throughout life and is characterized by a lack of cone photoreceptor function. Cone photoreceptors which are concentrated in the macula and the fovea, respond to moderate or bright intensity light and mediate fine visual acuity. Individuals with ACHM have markedly reduced visual acuity, photophobia or light sensitivity, and complete loss of color discrimination. Their only functioning photoreceptors are rod photoreceptors, which respond to low intensity light conditions and mediate night vision but cannot achieve fine visual acuity. Best-corrected visual acuity in persons affected by ACHM, even under subdued light conditions, is usually about 20/200, a level at which people are considered legally blind. They also experience extreme light sensitivity resulting in even worse visual acuity under normal daylight conditions, or day blindness.

ACHM can be caused by mutations in any of at least five genes that are required for normal cone photoreceptor function. The most common causes are mutations in the CNGB3 gene (about half of all cases) or CNGA3 gene (about one-fourth of all cases). These genes encode the CNGB3 and CNGA3 proteins, which combine to form a channel in the photoreceptor membrane that is required for photo-transduction, the process of converting light into electrical signals that the brain can understand. According to published reports, the incidence rate for ACHM is approximately one in 30,000 people, and we therefore estimate that there are about 10,000 people in the United States and about 17,000 people in Europe with ACHM. Of these, more than 75% have disease caused by mutations in the CNGA3 or CNGB3 gene.

Our ACHM product candidates

Our gene therapy approach to treatment of ACHM involves using an AAV vector to insert a functional copy of the CNGB3 or CNGA3 gene into the patient's photoreceptor cells. Our ACHM product candidates contain either the CNGB3 or the CNGA3 gene and a proprietary cone specific promoter that has been shown in preclinical studies to drive efficient gene expression in all three types of primate cone photoreceptors and restores cone photoreceptor function in dog, mouse and sheep models of ACHM.

Clinical development of our CNGB3 and CNGA3 related ACHM product candidate

We are currently enrolling patients in two Phase 1/2 clinical trials at multiple clinical sites that specialize in inherited retinal diseases. The primary endpoint of these clinical trials is safety, and while available data thus far have shown that the ACHM CNGB3 and CNGA3 product candidates are generally safe and well tolerated, we did experience initial variability in surgical procedures, which we have now resolved through our extensive surgical training procedures. In addition to safety, these trials will measure biologic activity by assessing changes in visual function and quality of life. The clinical protocols are designed as dose escalating trials to evaluate our product candidates in ACHMA3 and ACHMB3 patients at multiple dose levels.

We have completed enrollment of 26 and 19 patients in the dose escalation portions of the CNGB3 and CNGA3 trials, respectively. The safety profile in both trials remains favorable. We are currently enrolling pediatric patients at the two highest dose groups in each trial, although we have experienced delays in pediatric enrollment in connection with the COVID-19 pandemic. We have also taken multiple steps to address the challenges that we have faced due to the COVID-19 pandemic in assessing patients' outcomes and monitoring their safety. For example, we now offer enrolled subjects the option to have testing performed near their home either by visiting a qualified local ophthalmologist, or by visiting a mobile vision center that drives to their neighborhood. The mobile vision center is equipped and staffed with certified technicians who are trained to perform important test modalities.

We believe that dosing the two highest dose groups, (including pediatric patients) and following patients for an extended period of time will enrich our data and build a robust set of safety and efficacy data to support our BLA filing, maximize the benefit to the greatest range of patients.

On January 28, 2020, we provided early achromatopsia (ACHM) data indicating biologic activity in the dose escalation portions in both the B3 and the A3 trials. At the middle dose level one of three patients in each trial and at the high dose level 2 of 3 patients in the B3 trial have shown clinically meaningful improvements, defined as greater than one log change from baseline, in light discomfort at three months. Anecdotal statements from the patients support these improvements as being meaningful to their daily lives.

We have also completed enrollment in a natural history study in persons affected by ACHM caused by CNGB3 mutations and CNGA3 mutations and results from the studies will be presented in appropriate scientific meetings and publications.

Successful completion of the Phase 1/2 clinical studies and the natural history studies will guide us in finalizing the design of the pivotal clinical trial. If successful, we believe that the results of this pivotal trial could support

our submission of a BLA to the FDA and of a marketing authorization application to the European Medicines Agency, or the EMA, for our ACHM product candidates.

Product Candidate to Treat Advanced Retinal Disease

In partnership with Bionic Sight, AGTC provided pre-clinical and IND (investigational new drug) application support in Bionic Sight's development of an optogenetic candidate treatment for individuals having retinitis pigmentosa (RP) who have lost light sensitivity. RP is a large group of inherited retinal disorders in which progressive degeneration of photoreceptors or retinal pigment epithelium (RPE) leads to vision loss that is independent of a patients' genetic mutation. In Europe and the United States, about 200,000 patients suffer from RP and every year between 15,000 and 20,000 patients with RP suffer vision loss. The clinical manifestations of affected individuals present first as defective dark adaptation or "night blindness," followed by reduction of peripheral visual fields and, eventually, loss of central vision. While the photoreceptor cell layers of these patients degenerate, the ganglion cell layer remains intact.

Optogenetics is a biological technique by which cells are modified to express light-sensitive proteins. When the cells expressing these proteins are activated by light, they produce neural signals, which they can pass on to other neurons. Bionic Sight expresses the optogenetic proteins in the retina's ganglion cells, allowing them to send neural signals down the optic nerve to neurons in the visual areas of the brain.

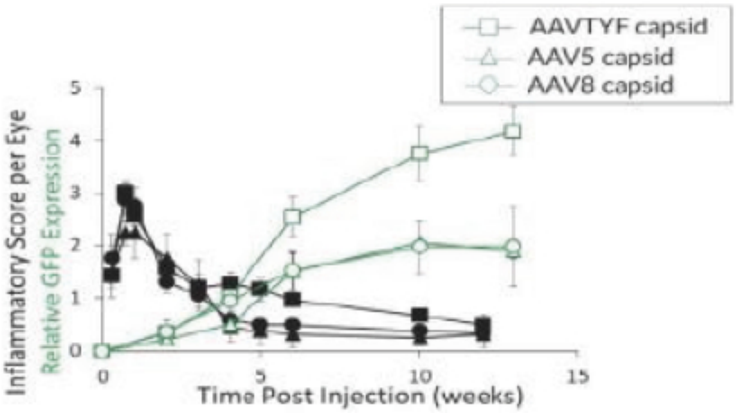
The candidate treatment currently in clinical development by Bionic Sight is an AAV2 vector, injected intravitreally, that expresses a modified optogenetic gene, ChronosFP, in the retinal ganglion cells. The ChronosFP protein that is expressed is believed to have a more dynamic range of sensitivity and responsiveness than other optogenetic proteins being developed. In conjunction with the gene therapy treatment, Bionic Sight is developing a wearable prosthetic device that uses a novel algorithm to provide light signals to the retinal ganglion cells in a pattern similar to that produced by normal retinal cells, which gives the treatment the potential to produce images the brain can recognize and may significantly enhance vision in patients receiving the optogenetic treatment. The IND for the program was successfully cleared by the FDA, and Bionic Sight initiated a Phase 1/2 clinical trial to test safety and potential efficacy of the treatment with the first patient treated in March 2020. Bionic Sight suspended enrollment by the clinical site due to the impact of COVID-19, but reinitiated clinical activities in July 2020 consistent with applicable COVID-19 guidelines.

Ongoing Research in Support for our Current and Future Clinical Programs

In support of our clinical programs described above, we continue to conduct research to fully understand the underlying technology. Additionally, we have initiated or continued several programs that are in early safety and preclinical proof of concept stages that are described below.

XLRP product candidate differentiation

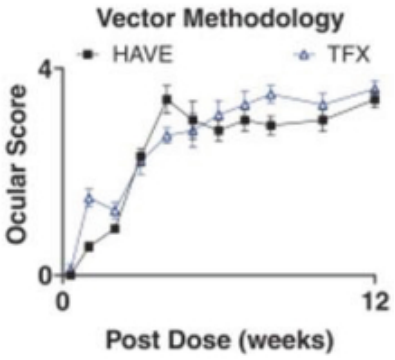
Three subretinal AAV gene therapy vectors are currently in clinical development for treatment of patients with XLRP. To compare the relative attributes of these vectors, a study was conducted which compared the photoreceptor transduction efficiency of subretinally delivered AAVTYF, the AAV capsid used in AGTC product candidates, AAV5 and AAV8 capsids in a head-to-head NHP experiment. Non-human primates were injected with each of the vectors and were followed for 13 weeks. Safety parameters included ocular exams, clinical observations, clinical pathology, and anatomic histopathology. A direct comparison between AAVTYF (n=12), AAV5 (n=4) and AAV8 (n=8) revealed that AAVTYF was comparable to or superior to both AAV5 or AAV8 in transduction of photoreceptors in NHPs when delivered subretinally, while demonstrating a similar, moderate inflammatory response. Therefore, AAVTYF represents, an attractive therapeutic choice for human XLRP gene therapy.

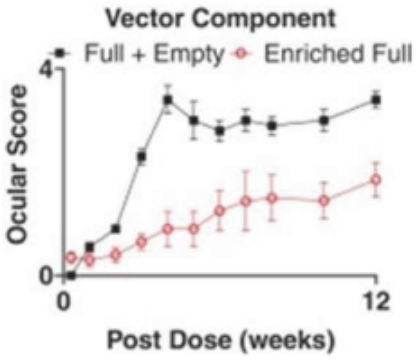


Understanding the ocular inflammatory response to AAV administration

Both intravitreal, and to a lesser extent, subretinal administration elicits an inflammatory response to AAV. In the eye, it remains unclear what specific property of the vector preparation drives inflammation. To this end, through a series of detailed, well-controlled studies in non-human primates, we performed a systematic evaluation of the potential contributory factors involved. A brief summary of the studies conducted is shown in the table below, and the accompanying illustrative figures.

Purpose	Parameters	Observations
Vector Methodology (Transfection (TFX) versus HAVE (Herpes simplex virus system))	Ocular inflammation following intravitreal administration	No difference in ocular inflammatory response between manufacturing methodologies
Vector Components (Full capsids, empty capsids, process residuals)	In-life ocular inflammation, transduction efficiency and cytokine/cellular immune responses following intravitreal administration	Reduction of empty capsids lowers inflammation and enhances transduction Transient minimal response in cytokines or immune cells (local and systemic), with no clear distinction across treatment groups
Capsid serotype (AAVTYF, AAV5, AAV8)	In-life ocular inflammation, transduction efficiency following sub-retinal administration	No difference in ocular inflammatory response between capsid serotypes, two-fold improvement of transduction efficiency with AAVTYF relative to AAV5 and AAV8
Pre-existing Immunity (Low, Medium and High)	In-life ocular inflammation, prevention of vector transduction, and neutralizing antibody correlation between eyes following intravitreal administration	Pre-existing immunity has no impact on ocular inflammation, and is not sufficient in itself to block vector transduction Neutralizing antibody in one eye does not impact neutralizing antibody levels in the contralateral eye





The ocular inflammation is most strongly correlated to total vector dose, and appears to occur in two phases: immediate, surgery/injection related (more explicit with sub-retinal injections) and delayed, in response to vector (processing of capsid and/or transgene expression). The studies outlined above have allowed us to eliminate the following as key drivers of inflammation: production methodology (transfection versus HSV), characteristics of the AAV product (transgene and process residuals) and capsid serotype (AAV2 versus AAV8) or novel engineered variant (AAVTYF). To date, none of the studies indicate that we should make changes in our product candidates, but we continue to work in non-human primates to understand ocular inflammation. The first publication of this work has recently been accepted by Human Gene Therapy (Timmers et al., 2019).

Other opportunities in ophthalmology

We believe that our advanced gene therapy platform will enable us to develop and test new AAV vectors that carry gene sequences both for other inherited diseases in ophthalmology (it is estimated that approximately 290 genes causing inherited retinal disease have been identified), as well as larger ocular indications for which intervention at a specific target gene or pathway has been clearly identified. By leveraging the existing work on our lead programs and further deployment of technology advances, we believe we can reduce the need for early research work. In this way, we anticipate being able to move products efficiently through preclinical studies and into clinical development. We have added two additional ophthalmology programs to our preclinical pipeline:

- **Stargardts Disease:** In November 2019 we selected Stargardts disease as a new orphan ophthalmology indication with a substantial patient population, defined clinical phenotype and available animal models to move forward towards the clinic. Stargardts disease is a macular dystrophy characterized by the progressive loss of photoreceptors leading to blindness and is caused by mutations in the ABCA4 gene. This particular gene at ~6.8 kilobases exceeds the size packaging capacity of AAV, and therefore required the use of a novel dual AAV vector system to deliver the two halves of the gene. This was successfully accomplished, and as a result of extensive studies in a relevant *abca4* knockout mouse model we have shown that the disease phenotype can be corrected with this approach (Dyka et al., 2019). We have also demonstrated that the same dual vector system results in reconstitution of the full length, functional protein in NHP retina when administered by subretinal injection. The program is positioned to proceed to IND-enabling safety and biodistribution studies.
- **Dry AMD:** An estimated 15 million people in North America have age-related macular degeneration (AMD), of which 85-90 percent are diagnosed with the non-exudative dry form. This medical condition may result in blurred or no vision in the center of the visual field, which can make it hard to recognize faces, read, drive and perform daily activities. Progression to geographic atrophy (or wet AMD) leads to serious risk of blindness. Dysregulated complement pathway is considered an important factor in the disease etiology, and a component of the alternative complement pathway, Complement Factor H (CFH), is known to have a strong genetic risk association with AMD. Patients homozygous,
or carrying two copies, of the Y402H mutation have a ~6-fold increased risk of developing AMD

(Sepp et al., 2006). Preclinical data in relevant animal models support the approach of CFH gene augmentation as a potential therapeutic strategy for AMD. The full length CFH gene is too large to fit into a single AAV, and so in this case, we adopted a different approach and designed multiple engineered truncated forms, testing these in combination with different promoters to determine if they retain activity. From these studies, we identified a preferred construct that retains functionality both in vitro and in vivo. For example, we have shown that the construct targets the complement cascade in the retina of a *cfh* knockout mouse model and have performed expression studies in NHP retina. The program is positioned to proceed to IND-enabling safety and biodistribution studies.

Central Nervous System

An additional strategic area of focus for AGTC is in the central nervous system, or CNS, where we see unique opportunities to leverage our comprehensive capabilities in vector design, delivery and manufacturing to address severe unmet medical needs in several diseases. We are actively developing three opportunities and have established a world-class scientific advisory board to assist and guide our efforts as we advance these programs through preclinical development:

- **Frontotemporal dementia (FTD)**: FTD is a degenerative brain disorder, second only to Alzheimer's disease in terms of prevalence and incidence in the dementia spectrum, is on the rise due to the aging population and has no approved treatments. Mutations in the Progranulin (PGRN) gene are one of the three main genetic causes of FTD, representing approximately 20% of familial FTD, and accounting for 3000 to 6000 cases in the US. PGRN is a glycoprotein that undergoes protease enzyme-dependent cleavage into smaller subunits called granulins, and these granulins may play a key role in inflammation, wound repair, tumorigenesis and sexual differentiation. PGRN is critical in neurons for proper trafficking and function of lysosomal enzymes such as ß-glucocerebrosidase and cathepsin D. PGRN haploinsufficiency (the loss of gene expression from one allele, resulting in reduced levels) is causally connected to FTD, and PGRN-deficient neurons are prone to accumulation of the protein TDP-43, which is thought to then lead to neurodegeneration. We are seeking to the augment PGRN levels in order to restore its physiological balance, and this approach is supported by several studies in PGRN-deficient mouse models, where both the pathological and behavioral changes that occur as a result of PGRN loss have been rescued (Arrant et al., 2017, 2018). Following extensive preclinical analysis exploring different capsid, promoter and transgene combinations, we have devised a novel AAV construct to enhance PGRN expression in the brain following direct vector administration into the cerebrospinal fluid, and have data in NHP measuring cerebrospinal fluid levels of PGRN, confirming that projected therapeutic levels are achievable within a defined dose range. The program is now positioned to proceed to IND-enabling safety and biodistribution studies.
- **Amyotrophic lateral sclerosis (ALS)**: ALS (Lou Gehrig's disease) is an autosomal dominant, fatal adult onset disease with no truly effective therapy. It is the most common adult-onset motor neuron disease, with approximately ~30,000 cases in the US, and is characterized by upper and lower motor neuron degeneration. Early symptoms are of muscle weakness that progresses and then ultimately results in respiratory failure, with death usually occurring within 3 to 5 years of first symptoms. There are both sporadic (~90%) and familial (~10%) forms of the disease, with the most common genetic cause linked to the C9orf72 gene, representing 30-40% of cases. C9orf72 mutations are also present in FTD. C9orf72 gene six nucleotide repeat expansions can result in both gain-of-toxicity and loss-of-function, both of which are believed to contribute to the pathogenesis in C9orf72-related disease. Targeting of such repeat sequences with antisense oligonucleotides or artificial microRNAs, for example, has been shown to reduce the accumulation of intra-nuclear transcripts (Martier et al., 2019). We are working on a novel integrated therapeutic approach that we believe has the potential to fully address all of the cellular deficits associated with the mutations and are currently in the process of identifying the optimal combination of components through different library screening strategies.

- **Adrenoleukodystrophy (ALD)**: This disease is an X-linked disorder of fatty acid metabolism that leads to accumulation of very long chain fatty acids in tissues throughout the body, mainly in the central nervous system and the adrenal gland. Patients with ALD cannot break down long-chain fatty acids, leading to their accumulation in cells of the nervous system, brain and adrenal gland. This leads to progressive loss of the membrane that insulates nerves in the brain and spinal cord and may cause damage to the outer layer of the adrenal gland. Clinically, ALD is a heterogeneous disorder with several distinct phenotypes, including rapidly declining neurological function and early death in young boys or progressive muscular weakness leading to lower limb paralysis in adults. There are approximately 14,000 patients with ALD in the United States. Early data from our preclinical studies support a gene therapy-based approach to treating the disease and warrant advancing it to our preclinical pipeline. We have made significant progress on vector design, animal model proof of concept and targeting studies in NHP in order to obtain data to support moving a potential product candidate to IND enabling studies.

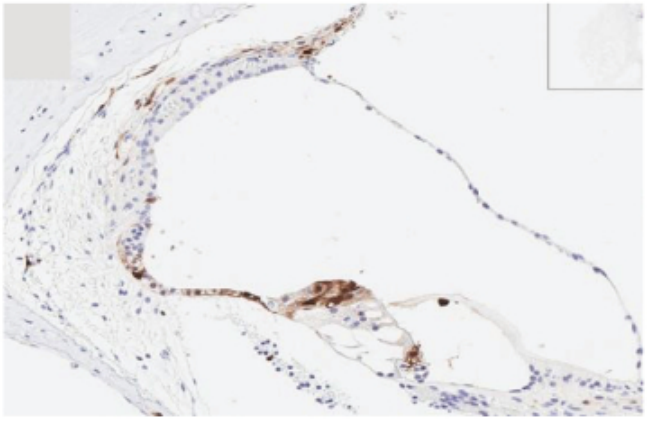
Otology

Hearing loss is one of the most common human sensory deficits and it is estimated that nearly half of the cases have a genetic origin. Of the inherited forms of hearing loss, more than 300 genetic causes have been defined with the specific gene identified for more than 70. Despite the impairment that can be caused by deafness, very little progress has been made in developing therapies that go beyond the temporary and partial solutions provided by hearing aids and cochlear implants. In multiple academic research studies, replacement of defective genes in animal models with normal copies has been shown to improve sound propagation in the auditory hair cells, making this a potentially promising application of AAV gene therapy. Additionally, the inner ear shares many of the characteristics that make ophthalmology attractive: it is anatomically well defined and is a small, well contained space where the target cells to be treated are easily identified. Also, the clinical outcome measures for treatments for hearing loss are well defined.

Developing product candidates for conditions having these characteristics is a natural complement to our ophthalmology and CNS portfolio strategy as we apply our core capabilities and expertise to a new disease field. As part of our efforts in otology, we formed a scientific advisory board and have conducted a detailed evaluation of the development and commercial landscape. From these efforts, we have selected targets which we believe are technically feasible and commercially viable.

- **DFNB1**: In September 2019, we announced that we had entered into a strategic collaboration with Otonomy, a biopharmaceutical company dedicated to the development of innovative therapeutics for neurotology, to co-develop and co-commercialize an AAV-based gene therapy to restore hearing in patients with sensorineural hearing loss caused by a mutation in the gap junction protein beta 2 gene (GJB2). Mutations in GJB2 account for approximately 30% of all genetic hearing loss cases, representing approximately 1000-2000 new cases each year in the US, and patients with this mutation can have severe-to-profound deafness in both ears that is identified in screening tests routinely performed in newborns. The GJB2 gene encodes connexin-26, which is expressed in cochlear support cells, forming gap junctions that control potassium homeostasis which is critical for the survival and function of hair cells and normal hearing. Mutations in GJB2 impair gap junctions and cochlear homeostasis leading to hair cell dysfunction and hearing loss.

  The goal of GJB2 gene therapy is to restore functional gap junctions and preserve hair cells to improve hearing by providing a copy of the wild-type gene to support cells. In order to identify the optimal AAV components to achieve this, we have actively screened novel capsid variants in mouse, guinea-pig and NHP for their ability to transduce the support cells of the cochlear. Below is a representative example from one such study in NHP demonstrating GFP reporter transgene expression by immunohistochemistry (shown as brown staining in the figure) in the target cell types.



The Joint Steering Committee finalized selection of all components for the proposed lead candidate including the identification of a proprietary novel capsid, and a series of confirmatory preclinical efficacy and expression studies in mouse and NHPs will support progress into a planned IND-enabling GLP toxicology and biodistribution study expected to begin in the first half of 2021.

Strategic collaborations

We have formed strategic alliances in which both parties contribute expertise to enable the discovery and development of potential gene therapy product candidates. To access the substantial funding and other resources required to develop and commercialize gene therapy products, we intend to seek additional opportunities to form strategic alliances with collaborators who can augment our industry-leading gene therapy expertise.

On October 1, 2019, we entered into a strategic collaboration with Otonomy, Inc., a San Diego based biopharmaceutical company focused on neurotology. We plan to work with Otonomy to develop and commercialize gene therapy products for genetic forms of hearing loss with an initial focus on GJB2, which is the most common cause of congenital hearing loss. The collaboration leverages our experience and technology in the development and design of optimized AAV gene therapy products with Otonomy's expertise in preclinical and clinical development for neurotology products.

Under the terms of the collaboration agreement, we and Otonomy share the expenses and any revenue or other proceeds equally for jointly developed products. Thus far, the focus of our efforts has been to design and test the optimal product construct, including the identification of a novel capsid with tropism for the target tissues. These results supported selection of the product candidate for further development.

On February 2, 2017, we entered into a strategic research and development collaboration agreement with Bionic Sight to develop therapies for patients with visual deficits and blindness due to retinal disease. Through the AGTC-Bionic Sight collaboration, the companies seek to develop a new optogenetic therapy that leverages AGTC's deep experience in gene therapy and ophthalmology and Bionic Sight's innovative neuro-prosthetic device and algorithm for retinal coding.

Under the terms of the agreement, AGTC provided in-kind support aggregating $2.2 million to Bionic Sight in the form of ongoing research and development efforts focused activities required to file an IND for a selected product candidate and to achieve successful clearance by a relevant Institutional Review Board (IRB), both of which are required before a clinical trial can proceed. In addition to the in-kind activities, AGTC made two payments to Bionic Sight in exchange for equity based on a predetermined valuation, an initial $2.0 million payment upon signing the collaboration agreement and a second payment of $4.0 million upon receipt of an IND clearance from the FDA and receipt of written approval from an IRB to conduct clinical trials from at least one clinical site for that product candidate. Our aggregate equity ownership in Bionic Sight currently is 15.5%.

Bionic Sight is responsible for conducting the Phase 1/2 clinical trial, which enrolled its first patient in March 2020, and we have no further obligations under the agreement. Upon completion of the trial or the achievement of certain pre-agreed definitions of potential efficacy, we have an exclusive option to negotiate to license or partner the program. If we and Bionic Sight are not able to reach agreement after a defined timeframe, Bionic Sight may negotiate with third parties but only on terms that are no more favorable to the third party than last offered by us without our consent.

We will continue to seek to partner with other gene therapy companies and academic institutions to leverage our expertise in vector design, research, manufacturing and the regulatory process. The goal of these collaborations would be to forge strategic partnerships around technologies and programs that would fit with our current and future development pipeline. In general, we would seek new intellectual property, development programs in rare diseases, pipeline products where the regulatory pathway is understood, partners with strong scientific, clinical, commercialization and management expertise, and programs that have synergy with our current knowledge base and product pipeline that would add to our industry leadership. We would also be looking at programs where the disease being treated has a large enough patient population that there would be adequate financial returns for the investment of resources.

Our relationship with the University of Florida

All of our scientific founders spent part of their careers at the University of Florida, or UF, and two are still UF faculty members. Since our inception we have licensed significant technology from and funded research at multiple labs at UF. Pursuant to four agreements, we have licensed three U.S. patents and multiple pending applications covering inventions made at UF. UF has multiple capabilities in genetic cloning, gene therapy manufacturing, novel capsid identification, animal model development and facilities for both small and large animal testing and, in certain instances, we have benefited from the ability to conduct important research at UF without having to expand in-house facilities and personnel.

In May 2013, we and UF were jointly awarded an $8.3 million grant from the National Eye Institute to support development of our ACHM CNGB3 product candidate, with Dr. William Hauswirth, one of our scientific founders, a Professor and holder of the Rybaczki-Bullard Chair in the Department of Ophthalmology at UF, as principal investigator. As of June 30, 2020, we have received payments in the aggregate amount of $3.4 million under this grant.

Our relationships with patient advocacy groups and academic centers

We have long believed that when developing product candidates to treat orphan indications it is important to form strong relationships with patient advocacy groups, and we have done this successfully with both the Foundation Fighting Blindness, or FFB (U.S.), FFB (Canada) and other organizations that are well known for their advocacy of patients' interests in obtaining diagnosis, developing treatments and providing for reimbursement. Many actively support research into treatment, and we have been awarded three research grants totaling $1.6 million from the FFB (U.S.) More importantly, both organizations have been instrumental in assisting us in forming ties with disease experts, recruiting patients into clinical trials and helping us to understand the needs, wants and concerns of patients. We also have relationships with other advocacy organizations such as Achroma Corp, the BCM Family Foundation, MOMS For Sight, Curing Retinal Blindness Foundation, Sofia Sees Hope, National Organization for Rare Disorders,), Italian Achromatopsia Association (IAA), and Alliance for Regenerative Medicine.

In order to gain further patient specific insight to support our later stage ophthalmology clinical development, we have formed a Patient Advisory Council comprised of individuals with inherited retinal diseases (IRDs) and members from the global community of organizations that represent them. AGTC's Patient Advisory Council will initially focus on providing input on XLRP clinical trial patient experiences, processes, recruitment, and enrollment. In addition, the council will serve as consultants on ongoing and future clinical trial activities,

including protocol design, patient registries, development of patient outreach and education materials, and will liaise with AGTC's Scientific Advisory Board and the broader healthcare providing community. In addition, we have formed strong relationships with key academic centers across the United States that have core competencies in gene therapy, orphan ophthalmology and other disease areas, such as CNS and otology, that we have an interest in. These centers conduct sponsored research, act as advisors and collaborate with us on grant proposals. Since our inception, we have been awarded a variety of grant funding, either independently or with our collaborators. This funding has provided peer-reviewed scientific validation of our programs and has facilitated critical early stage research for our lead product candidates.

Intellectual property

We strive to protect and enhance the proprietary technology, inventions and improvements that are commercially important to the development of our business, including seeking, maintaining and defending patent rights, whether developed internally or licensed from third parties and seeking patent term extensions where available. We also rely on trade secrets relating to our proprietary technology platform and on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position in the field of gene therapy that may be important for the development of our business. In addition to intellectual property and trade secrets, we also will rely on regulatory protection afforded through orphan drug designations, data exclusivity and market exclusivity for our product candidates, when possible.

Our commercial success may depend in part on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business; defend and enforce our patents; preserve the confidentiality of our trade secrets; and operate without infringing the valid enforceable patents and proprietary rights of third parties. Our ability to stop third parties from making, using, selling, offering to sell or importing our product candidates may depend on the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities. With respect to both licensed and company-owned intellectual property, we cannot be sure that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our commercial product candidates and methods of manufacturing the same.

We have developed or in-licensed numerous patents and patent applications and possess substantial know-how and trade secrets relating to the development, commercialization and manufacture of gene therapy product candidates. Our proprietary intellectual property, including patent and non-patent intellectual property, is generally directed to, for example, certain genes and promoters, methods of transferring genetic material into cells, processes to manufacture our AAV-based product candidates and other proprietary technologies and processes related to our lead product candidates.

As of July 28, 2020, our patent portfolio included approximately 100 patents and patent applications that we own and approximately 50 patents and patent applications that we have licensed. More specifically, we own 6 U.S. patents, 10 pending U.S. applications, 64 foreign patents and 20 foreign patent applications. We have licensed 6 U.S. patents, 1 pending U.S. application, 40 foreign patents and 1 pending foreign patent application. Of the patents and patent applications that we own or license, 37 cover methods to manufacture AAV vectors, the longest lived and most significant of which is expected to expire in 2029. In October 2017, we were awarded US Patent Number 9,783,826 directed to methods of producing recombinant AAV viral particles using suspension BHK cells. This patent extends the protection on our AAV manufacturing platform from 2025 to 2029.

Our objective is to continue to expand our portfolio of patents and patent applications in order to protect our gene therapy product candidates and AAV manufacturing process. Our owned and licensed patent portfolio includes patents and patent applications directed to our XLRS, ACHM, and XLRP programs, as well as our foundational AAV production platform. See also "License agreements."

In addition to the above, we have established expertise and development capabilities focused in the areas of preclinical research and development, manufacturing and manufacturing process scale-up, quality control, quality assurance, regulatory affairs and clinical trial design and implementation. We believe that our focus and expertise will help us develop product candidates based on our proprietary intellectual property and to expand our intellectual property portfolio.

The term of individual patents depends on the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the date of filing the non-provisional application. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the United States Patent and Trademark Office in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent. The issued patents that are material to our business are expected to expire on various dates from 2022 to 2029.

The term of a patent that covers an FDA-approved drug may also be eligible for patent term extension, which permits patent term restoration of a U.S. patent as compensation for the patent term lost during the FDA regulatory review process. The Hatch-Waxman Act permits a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug is under regulatory review. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent per approved drug may be extended. Moreover, a patent can only be extended once, and thus, if a single patent is applicable to multiple product candidates, it can only be extended based on one product candidate. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug. When possible, depending upon the length of clinical trials and other factors involved in the filing of a BLA, we expect to apply for patent term extensions for patents covering our product candidates and their methods of use.

We may rely, in some circumstances, on trade secrets to protect our technology. 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 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 may be breached and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

License agreements

We have rights to use and exploit multiple technologies disclosed in issued and pending patents under licenses from other entities. We consider the commercial terms of these licenses, which provide for modest milestone and royalty payments, and their provisions regarding diligence, insurance, indemnification and other similar matters, to be reasonable and customary for our industry.

Information about our principal licenses is set forth below.

The University of Florida

We currently have four agreements with the University of Florida Research Foundation, or UFRF, an affiliate of UF, of which the principal licenses are as follows:

- A joint license from UFRF and Johns Hopkins University, or JHU, signed in October 2003 relates to a particular HSV construct and various compositions thereof. We have an exclusive license in all fields of use.

Under the terms of this license, we made cash and stock-based up-front payments to UFRF and JHU and are required to make payments ranging from the mid-five figures to the low-six figures based upon development, regulatory and commercial milestones for any product candidates covered by the in-licensed intellectual property. Assuming that we meet each of the specified development, regulatory and commercial milestones not more than once for each product candidate, which we expect will be the case, the maximum aggregate milestone payments payable under this license with respect to any individual product candidate that we commercialize will be $0.5 million. We will also be required to pay a royalty on net sales of product candidates covered by the in-licensed intellectual property in the low-single digits. We have the right to sublicense our rights under this agreement, and we will be required to pay a percentage of such license income in the low-double digits. We are required to make annual maintenance payments in the low four figures under this license, which payments are creditable against royalty payments on a year-by-year basis.

This license will terminate upon the earlier to occur of the expiration of all of the patents subject to the license and the date on which royalty payments, once commenced, cease for more than three calendar quarters. Additionally, UFRF and JHU may terminate this license upon certain breaches by us of the terms of the license and we may terminate the license at any time by submitting written notice to UFRF.

The longest-lived patent covered by this license is expected to expire in 2022.

- Two licenses from UFRF, signed in September and November 2012, respectively, relate to the use of engineered AAV capsids. We have an exclusive license to the patents covered by the November 2012 license in the fields of ACHM, XLRS and XLRP and a semi-exclusive license in all other fields of orphan ophthalmology. We have a non-exclusive license in all fields of use with respect to the patents covered by the September 2012 license. Currently these patents are most useful for ACHM, XLRS and XLRP but could be important for treating a wide variety of diseases as the engineered capsids have been shown to be able to enter cells more effectively than standard AAV capsids.

  Under the terms of these licenses, we made cash up-front payments to UFRF and are required to make payments ranging from the mid-five figures to the low-six figures based upon development, regulatory and commercial milestones for any product candidates covered by the in-licensed intellectual property. Assuming that we meet each of the specified development, regulatory and commercial milestones not more than once for each product candidate, which we expect will be the case, the maximum aggregate milestone payments payable under these licenses with respect to any individual product that we commercialize will be $0.6 million. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low-single digits. We have the right to sublicense our rights under these agreements, and we will be required to pay a percentage of such license income in the mid-single digits. We are required to make annual maintenance payments in the mid four figures under these licenses, which payments are creditable against royalty payments on a year-by-year basis.

  On April 20, 2020, we amended the capsid license to add further rights to these licenses in exchange for a non-exclusive grant-back of the intellectual property for use by UF investigators to enable research on four gene targets that are not of strategic interest to AGTC. In exchange, we will receive pre-defined portion of any proceeds received any UFRF if they enter into a license agreement for these gene targets with a third party. Further, UFRF agrees that it will notify us of potential license opportunities any of the four gene targets, so long as not otherwise restricted by confidentiality agreements with third parties.

  These licenses will continue until the expiration of all of the patents subject to the licenses, provided or, if later, a date specified in the license. Additionally, UFRF may terminate this license upon certain breaches by us of the terms of the licenses and we may terminate the licenses at any time by submitting written notice to UFRF.

  The longest-lived patent covered by these licenses is expected to expire in 2029. There are also patent applications pending under these licenses.

- An Evaluation and License Agreement from UFRF, signed in May 2019, relates to the use of engineered AAV capsids in the field of otology. Under the terms of the agreement, we undertook the evaluation of multiple promising capsid candidates for potential application in otology. In December 2019, we entered into a non-exclusive license agreement for three capsids that showed the most potential to support therapeutic development. Under the terms of the license agreement, we made a cash up-front payments to UFRF and will be required to make payments ranging from the mid-five figures to the low-six figures based upon development, regulatory and commercial milestones for any product candidates covered by the in-licensed intellectual property. Assuming that we meet each of the specified development, regulatory and commercial milestones not more than once for each product candidate, which we expect will be the case, the maximum aggregate milestone payments payable under these licenses with respect to any individual product that we commercialize will be $0.25 million. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low-single digits. We have the right to sublicense our rights under these agreements, and we will be required to pay a percentage of such license income in the mid-single digits. We are required to make annual maintenance payments in the low four figures under these licenses until such time as royalties on commercial sales exceeds these amounts.

The University of Alabama at Birmingham

A license agreement from the UAB Research Foundation affiliated with The University of Alabama at Birmingham, signed in 2006, relates to one U.S. patent with claims covering the use of HSV helpers to produce AAV vectors. The patent is expected to expire in 2025. Effective in July 2015, we modified the license from co-exclusive to exclusive.

Under the terms of this license, we made a cash up-front payment to the UAB Research Foundation, and we will be required to make payments ranging from the mid-five figures to the low-six figures based upon development and regulatory milestones for any products covered by the in-licensed intellectual property. Assuming that we meet each of these development and regulatory milestones not more than once for each product, which we expect will be the case, the maximum aggregate milestone payments payable under this license with respect to any individual product that we commercialize will be $0.5 million. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low-single digits. We have the right to sublicense our rights under this agreement, and we will be required to pay a percentage of such license income in the mid-single digits. We are required to make annual maintenance payments in the mid-four figures under this license, which payments are creditable against royalty payments on a year-by-year basis.

This license will terminate upon the expiration of all of the patents subject to the license. Additionally, the UAB Research Foundation may terminate this license upon certain breaches by us of the terms of the license and we may terminate the license at any time by submitting written notice to the UAB Research Foundation.

Collaborations with 4DMT and Synpromics

In April 2015, we entered into a collaboration and option agreement with 4D to discover and develop optimized AAV vectors to treat specific ophthalmic disease indications. The AGTC Agreement expired in October 2018 when AGTC chose to not exercise its option to license during the option period. We continue to collaborate with Synpromics, acquired by AskBio in 2019, a company focused on synthetic promoter technology, bioinformatics and data-driven design that enables more precise cell targeting and gene expression. AGTC and Synpromics are engaged in the research and development of novel synthetic promoters that could be potentially useful in the product candidates under development by AGTC.

Competition

The biotechnology and pharmaceutical industries, including the gene therapy field, are characterized by intense and rapidly changing competition to develop new technologies and proprietary products, and any product candidates that we successfully develop and commercialize will have to compete with existing therapies and new therapies that may become available in the future. While we believe that our proprietary technology estate and scientific expertise in the gene therapy field provide us with competitive advantages, we face potential competition from many different sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions and governmental agencies and public and private research institutions that may develop potentially competitive products or technologies.

Currently there are no approved products for any of our lead orphan ophthalmology indications of ACHM and XLRP. We are aware of a number of companies focused on developing gene therapies in various indications, including Adverum Biotechnologies Inc., Akous, Allergan plc, Apic Bio, Inc., Axovant, Biogen Inc., bluebird bio, Inc., Decibel Therapeutics, Editas Medicine, Inc., 4D Molecular Therapeutics, GenSight Biologics S.A., Gyroscope Therapeutics Limited, Hemera Biosciences, Limelight Bio, Inc., MeiraGTx Limited (partnered with Janssen Pharmaceuticals), IVERIC bio, Oxford Biomedica plc, Passage Bio, Prevail Therapeutics, ProQR Therapeutics N.V., REGENXBIO Inc., the Roche Group (acquiring Spark Therapeutics), Ultragenyx Pharmaceuticals, Inc. and uniQure N.V., as well as several companies addressing other methods for modifying genes and regulating gene expression. Any advances in gene therapy technology made by a competitor may be used to develop therapies that could compete against any of our product candidates. For XLRP, 4D Molecular Therapeutics, MeiraGTx and Biogen are developing AAV-based gene therapies and MeiraGTx also has competing programs in ACHM-B3 and ACHM-A3. We believe that these companies and others could be planning to initiate clinical trials in the future that have the potential to be competitive with AGTC's programs. We further believe that the key competitive factors that will affect the success of our product candidates, if approved, are likely to be their efficacy, safety, convenience of administration and delivery, price, the level of generic competition, market exclusivity and the availability of reimbursement from government and other third-party payors.

Government Regulation

Biological products, including gene therapy products, are subject to regulation under the Federal Food, Drug, and Cosmetic Act, or FD&C Act, and the Public Health Service Act, or PHS Act, and other federal, state, local and foreign statutes and regulations. Both the FD&C Act and the PHS Act and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, recordkeeping, distribution, reporting, advertising and other promotional practices involving biological products. Before clinical testing of biological products may begin, we must submit an IND which must go into effect, and each clinical trial protocol for a gene therapy product candidate is reviewed by the FDA. FDA approval of a BLA also must be obtained before marketing of biological products in the United States. 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.

Within the FDA, the Center for Biologics Evaluation and Research, or CBER, regulates gene therapy products. CBER works closely with the NIH, both of which may engage in a public discussion of scientific, safety, ethical and societal issues related to proposed and ongoing gene therapy protocols. The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols. The FDA also has published guidance documents related to, among other things, gene therapy products in general, their preclinical assessment, observing subjects involved in gene therapy studies for delayed adverse events, potency testing, and chemistry, manufacturing and control information in gene therapy INDs, and gene therapy products for rare diseases and retinal disorders.

Ethical, social and legal concerns about gene therapy, genetic testing and genetic research have led to the enactment of legislation such as the Genetic Information Nondiscrimination Act of 2008 and could result in additional regulations restricting or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed interest in further regulating biotechnology. More restrictive regulations or claims that our product candidates are unsafe or pose a hazard could prevent us from commercializing any product candidates. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

Recent developments in regulation of gene therapy

In August 2017, Kymriah (tisagenlecleucel) became the first gene therapy product approved by the FDA. It was followed by four additional gene therapy product approvals, including Luxturna (voretigene neparvovec-rzyl) in December 2017. The Luxturna approval is of relevance to AGTC because it is a subretinally administered AAV vector that treats patients with a rare form of inherited vision loss. It is also the first FDA approved gene therapy that targets a disease caused by mutations in a specific gene.

FDA's acknowledged recognition of the promise of gene therapy and their expectation that the field will continue to expand has led it to take additional steps to support the advancement of gene therapy products. In January 2020, the FDA finalized six gene therapy guidance documents, which address manufacturing and clinical development. One guidance document provides FDA's recommendations for gene therapy product development and clinical trial design specifically for retinal disorders. AGTC's review of the FDA's recommendations found we are aligned with the agency's approach to product development and we see opportunities to advance our programs as anticipated following the collection of appropriate safety and efficacy data.

In Europe, seven gene therapy products have been approved. In 2012, the EMA approved a gene therapy product called Glybera, which is the first gene therapy product approved by regulatory authorities anywhere in the Western world. Most recently, Zolgensma became the seventh gene therapy product approved by the EMA.

United States biological products development process

The process required by the FDA before a biological product candidate may be marketed in the United States generally involves the following:

- completion of nonclinical laboratory tests and animal studies according to applicable good laboratory practices, or GLP, requirements and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an IND, which must become effective before human clinical trials may begin;
- performance of adequate and well-controlled human clinical trials according to good clinical practice, or GCP, standards and IND and human subject protection regulations, and requirements to ensure the privacy and confidentiality of human research subjects and their health information, to establish the safety and efficacy of the proposed biological product candidate for its intended use;
- validation of the biological product candidate manufacturing and control processes;
- submission to the FDA of a Biologics License Application, or BLA, for marketing approval that includes substantial evidence of safety, purity and potency from results of nonclinical testing and clinical trials;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biological product candidate is produced to assess compliance with GMP requirements, to ensure that the facilities, methods and controls are adequate to preserve the biological product candidate's identity, strength, quality and purity;

- potential FDA audit of the nonclinical and clinical trial sites that generated the data in support of the BLA; and
- FDA review and approval, or licensure, of the BLA prior to any commercial marketing or sale of the product candidate in the United States.

Before testing any biological product candidate, including a gene therapy product candidate, in humans, the product candidate enters the preclinical testing stage. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the product candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including applicable GLP requirements.

Sponsors or institutions receiving NIH funding are responsible for compliance with the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules, or the NIH Guidelines. However, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. The oversight bodies at the clinical site(s) (Institutional Review Board (IRB) and Institutional Biosafety Committee (IBC)) are responsible for determining whether or not the clinical study may be conducted there.

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, although IND sponsors generally wait until the FDA affirmatively provides notice that the agency has no issues with the IND. Under the FD&C Act, the FDA has the authority to prohibit a sponsor from conducting a clinical trial, referred to as a clinical hold, if the investigational product poses an unreasonable risk to the safety of trial subjects, taking into account the qualifications of the clinical investigators, information about the drug, the design of the clinical investigation, the condition for which the drug is to be investigated, and the health status of the subjects involved, or for any other reason the FDA has established by regulation, which are numerous and include, for example, deficiencies in study design or insufficient information to assess the risks to the subjects of a proposed study. If the FDA places the clinical trial on clinical hold within the 30-day time period after submission of an IND, the IND sponsor must address the FDA's concerns and the FDA must life the clinical hold before the clinical trial can begin. With gene therapy protocols, the FDA may seek advice from the FDA's Cellular, Tissue, and Gene Therapies Advisory Committee at any time on data, including preclinical data, related to the safety, effectiveness, and appropriate use of gene transfer therapies. This Advisory Committee consists of members selected by the FDA Commissioner considered to be authorities knowledgeable in the fields of gene therapies and related specialties. If the FDA recommends public review of the protocol or seeks comments from the Cellular, Tissue, and Gene Therapies Advisory Committee, initiation of the clinical trial could be delayed. The FDA may also impose a clinical hold at any time during the conduct of a clinical trial due to, for example, new safety concerns, another drug under investigation or approved for the same indication and available to the same patient population has demonstrated a better potential benefit/risk balance, or one or more adequate and well-controlled studies strongly suggest the lack of effectiveness. A trial on clinical hold may be initiated or continued when the FDA lifts the hold in writing and then only under terms specified by the FDA. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that suspend or terminate such trials.

Clinical trials involve the administration of the biological product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control. Clinical trials are conducted under 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 rules that ensure a clinical trial will be stopped if certain adverse events 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 GCP standards, human subject protection

requirements, and FDA's investigational new drug requirements, including the requirement that all research subjects provide informed consent. Further, each clinical trial must be reviewed and approved by an independent institutional review board, or IRB, at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. Clinical trials also must be reviewed by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees basic and clinical research conducted at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

Human clinical trials are typically conducted in three sequential phases that may overlap, be combined, or be bifurcated into two parts:

- *Phase 1*. The biological product candidate is initially introduced into healthy human subjects and tested for safety. In the case of some product candidates for severe or life-threatening diseases, especially when the product candidate may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.
- *Phase 2*. The biological 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*. Clinical trials are undertaken to further evaluate dosage, clinical efficacy, potency and safety in an expanded patient population at geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the product candidate and provide an adequate basis for product approval and labeling.

Post-approval clinical trials, sometimes referred to as Phase 4 clinical trials, may be required as a condition of approval or may be recommended after initial marketing approval if required. 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. Depending on the type of product and mechanism of action, the FDA may recommend that sponsors observe subjects for potential gene therapy-related delayed adverse events as part of a long-term follow up before licensure, which may include annual examinations and/or annual queries, either in person or by questionnaire, of trial subjects. After licensure, the FDA may recommend the establishment of a patient registry specifically to collect adverse event data from gene therapy patients.

During all phases of clinical development, 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 suspected adverse reactions, any findings from other trials, tests in laboratory animals 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. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. 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 health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the biological product candidate has been associated with unexpected serious harm to patients.

Human gene therapy products are a new category of therapeutics. Although the FDA has approved five gene therapy products in the United States, gene therapy remains a relatively new and expanding area of novel therapeutic interventions. Consequently, there can be no assurance as to the length of the trial period, the number of patients the FDA will require to be enrolled in the trials in order to establish the safety, efficacy, purity and potency of human gene therapy products, or that the data generated in these trials will be acceptable to the FDA to support marketing approval. Over the last several years the FDA has issued helpful guidance on development of gene therapy products and has recently finalized a guidance for gene therapy products for rare diseases in which the FDA shows an increased willingness to work closely with developers and encourages ongoing interactions with sponsors throughout the development process.

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

United States review and approval processes

After the completion of clinical trials of a biological product candidate, FDA approval of a BLA must be obtained before commercial marketing of the biological product candidate. The BLA must include results of laboratory, animal and human studies, information on the manufacture and composition of the product candidate, proposed labeling and other relevant information. In addition, under the Pediatric Research Equity Act, or PREA, a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the biological product candidate for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product candidate is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any biological product candidate for an indication for which orphan designation has been granted. The testing and approval processes require substantial time and effort and there can be no assurance that the FDA will accept the BLA for filing and, even if filed, that any approval will be granted on a timely basis, if at all.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a user fee. The FDA adjusts the PDUFA user fees on an annual basis. According to the FDA's fee schedule for fiscal year 2021, which becomes effective October 1, 2020, the user fee for an application requiring clinical data, such as a BLA, is $2,875,842. PDUFA also imposes an annual prescription drug program fee ($336,432) for certain approved products. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on BLAs for product candidates designated as orphan drugs, unless the product candidate also includes a non-orphan indication.

Within 60 days following submission of the application, the FDA reviews a BLA submitted 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 this 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 potent, or effective, for its intended use, and has an acceptable purity

profile, and whether the product candidate is being manufactured in accordance with GMP regulations to ensure and preserve the product candidate's identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, such as the Cellular, Tissue, and Gene Therapy 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 biological product approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy, or REMS, is necessary to ensure the safe use of the biological product candidate. A REMS may be imposed to ensure safe use of the drug, and could include medication guides, physician communication plans, or elements to ensure 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 GMP requirements and adequate to ensure consistent production of the product candidate within required specifications. Additionally, before approving a BLA, the FDA may inspect one or more clinical sites to ensure that the clinical trials were conducted in compliance with IND trial requirements and GCP requirements. If information from such an inspection, or from any other source, raises a significant question about the integrity of the clinical data, the FDA may suspend review of the BLA under its Application Integrity Policy, or AIP. After AIP has been invoked, FDA will not resume substantive review of any pending application unless and until the data have been validated. To ensure data integrity and GMP and GCP compliance, an applicant must incur significant expenditure of time, money and effort in the areas of training, recordkeeping, production, and quality control.

Notwithstanding the submission of relevant data and information, the FDA may ultimately decide that the BLA does not satisfy its regulatory criteria for approval and deny approval. Data obtained from clinical trials are not always conclusive and the FDA may interpret data differently than we interpret the same data. If the agency decides not to approve the BLA in its present form, the FDA will issue a complete response letter that usually describes all of the specific deficiencies in the BLA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical trials. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application.

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, which could restrict the commercial value of the product candidate. 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 biological product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

One of the performance goals agreed to by the FDA under the PDUFA is to review 90% of standard BLAs in 10 months and 90% of 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. If the FDA issues a complete response letter at the end of the initial review cycle and the sponsor resubmits its BLA addressing all deficiencies, a new two or six-month review cycle will begin, depending on the extent of the deficiencies to be addressed.

Orphan-drug designation

Under the Orphan Drug Act, the FDA may grant orphan-drug designation to a drug or biological product candidate intended to treat a rare disease or condition, which is defined as a disease or condition that affects fewer than 200,000 individuals in the United States, or 200,000 or more individuals in the United States if there is no reasonable expectation that the cost of developing and making a drug or biological product available in the United States for this type of disease or condition will be recovered from sales of the product candidate. Orphan-drug designation must be requested before submitting a New Drug Application, or NDA, or BLA. After the FDA grants orphan-drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan-drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process. Orphan-drug designation may also be rescinded if the product candidate no longer meets the criteria for designation.

If a product candidate that has orphan-drug designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the "same" drug or biological product for the same indication for seven years; however, the FDA has not yet established what characteristics of a gene therapy product are relevant to determining whether two gene therapy products would be considered the same for purposes of orphan drug market exclusivity. In January 2020, the FDA issued a draft guidance in which the FDA proposed its interpretation of the "sameness" criterion for determining whether a gene therapy product will be eligible for orphan drug market exclusivity. The FDA proposed to consider two gene therapy products for the same indication to be different, thus each eligible for market exclusivity, if they have different transgenes and different vectors, different transgenes regardless of whether they have the same vectors, or different vectors from a different viral class. The public docket to submit comments on the draft guidance closed on July 28, 2020. There is no required timeframe within which the FDA must complete its review of comments and decide whether to revise the guidance, finalize it as proposed, or withdraw it.

The FDA may approve a second drug or biological product during an exclusivity period in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product does not have exclusivity. Orphan product exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same biological product as determined by the FDA or if our product candidate is determined to be contained within the competitor's product for the same indication or disease. If a drug or biological product designated as an orphan product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan product exclusivity. Orphan drug status in the European Union has similar, but not identical, benefits.

Expedited development and review programs

The FDA has a Fast Track program that is intended to expedite and facilitate the process for reviewing new drugs and biological products that meet certain criteria. Specifically, new drugs and biological products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition and shown to have the potential to address unmet medical needs for the disease or condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. The sponsor of a new drug or biologic may request the FDA to designate the drug or biologic as a Fast Track product candidate at any time during the clinical development of the product candidate. Unique to a Fast Track product candidate, the FDA may consider for review sections of the marketing application on a rolling basis before the complete application is submitted if: the sponsor provides a schedule for the submission of the sections of the application; the FDA agrees to accept sections of the application and determines that the schedule is acceptable; and the sponsor pays any required user fees upon submission of the first section of the application.

In addition to the Fast Track program, the FDA provides other expedited programs for qualifying product candidates, such as priority review and accelerated approval. Any product candidate is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a disease compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biological product candidate designated for priority review in an effort to facilitate the review, and aims to review such applications within six months as opposed to ten months for standard review. Additionally, a product candidate may be eligible for accelerated approval. A drug or biological product will qualify for accelerated approval if the product treats a serious or life-threatening illness and, based on adequate and well-controlled clinical trials, is shown generally to provide a meaningful therapeutic benefit over existing treatments, and demonstrates an effect on a surrogate endpoint, or an intermediate clinical endpoint (i.e., an endpoint that can be measured earlier than irreversible morbidity or mortality) that is reasonably likely to predict a clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of accelerated approval, the FDA may require that a sponsor of a drug or biological product candidate perform adequate and well-controlled post-marketing clinical trials. In addition, the FDA currently requires, as a condition for accelerated approval, the pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

Under the provisions of the Food and Drug Administration Safety and Innovation Act, or FDASIA, enacted in 2012, a sponsor can request designation of a product candidate as a "breakthrough therapy." A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Drugs designated as breakthrough therapies are also eligible for accelerated approval and receive the same benefits as drugs with Fast Track designation. The FDA must take certain actions, such as holding timely meetings and providing advice, intended to expedite the development and review of an application for approval of a breakthrough therapy.

Following the establishment of the breakthrough therapy designation, FDA established the regenerative medicine advanced therapy (RMAT) designation in conjunction with the 2016 21st Century Cures Act. Like the breakthrough designation, the RMAT designation requires preliminary clinical evidence indicating that the therapy has the potential to address unmet medical needs. However, the RMAT designation does not require evidence to indicate that the drug may offer a substantial improvement over the available therapies, which the breakthrough therapy designation does. Fast Track, breakthrough therapy, and RMAT designations may also be rescinded if the product candidate does not continue to meet the designation criteria.

Fast Track designation, priority review, accelerated approval, breakthrough therapy designation and RMAT designation do not change the standards for approval but may expedite the development or approval process.

Post-approval requirements

Maintaining compliance with applicable federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Rigorous and extensive FDA regulation of biological products continues after approval, particularly with respect to GMP requirements. We will rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of any products that we may commercialize. Manufacturers of our products are required to comply with applicable requirements in the GMP regulations, including quality control and quality assurance and maintenance of records and documentation. Other post-approval requirements applicable to biological products include reporting of GMP 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 may also be subject to official lot release. In this case, as part of the manufacturing process, the manufacturer is required to perform certain tests on

each lot of the product before it is released for distribution. 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 of the manufacturer's tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products, such as viral vaccines, before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products.

FDA regulations prohibit the promotion of an investigational product for an unapproved use, which may include certain company-sponsored scientific and educational activities if the content of those activities is not independent of a company's influence or control. The FDA distinguishes impermissible promotion of an investigational product from the permissible exchange of scientific and medical information among healthcare professionals, which may include company-sponsored scientific and educational activities if the content of those activities is free of company influence or control. The FDA has issued Warning Letters and untitled letters to sponsors and clinical investigators who have claimed, directly or indirectly, that an investigational product is safe and effective for its intended use.

FDA regulations also impose requirements and limitations on advertising and promotional activities specifically for approved biological products. While a BLA is still under review, the BLA applicant must submit to the FDA copies of all promotional materials intended for use within 120 days following BLA approval, after which time the applicant must submit promotional materials at least 30 days prior to the intended time of use, unless otherwise directed by the FDA. For all drug and biological products, the FD&C Act prohibits false or misleading labeling, which includes statements in promotional materials about the product's safety, effectiveness, and indications for use. The FDA's regulations require advertising and promotional materials and activities to provide, among other things, adequate safety and risk information and fair balance, and prohibit the promotion of products for uses or in-patient populations that are not described in the product's approved labeling (known as "off-label use"). In addition, FDA has published guidelines, which include limitations on direct-to-consumer advertising and promotional activities via the Internet and social media. The failure to comply with the applicable regulatory requirements may result in Warning Letters to come into compliance and the FDA further requesting the cessation or revision of marketing materials and activities or the dissemination of corrective marketing materials. The FDA has the authority to seek an injunction to stop the dissemination of violative marketing materials and activities if adequate corrective actions are not taken voluntarily.

Biological product manufacturers and other entities involved in the manufacture and distribution of approved biological products are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with GMPs and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain GMP compliance. 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.

The FDA has the authority to take a variety of actions to address violations, including invoking the AIP and suspending the review of a pending application; refusing to approve or withdrawing approval of a marketing application; placing a study on clinical hold; issuing warning or untitled letters; ordering a biological product recall; seizing product in distribution; seeking an injunction to stop manufacture and distribution of a product; seeking restitution, disgorgement of profits, and fines; and debarring a company and its executives individually from participation in any capacity in the drug approval process. The U.S. Department of Justice has the authority to criminally prosecute companies and company executives for violations of the FD&C Act and the PHS Act.

United States patent term restoration and marketing exclusivity

Depending on the timing, duration and specifics of product development and the FDA review of a BLA, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved biological product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The United States Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for one or more of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant BLA.

A biological product can obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or Affordable Care Act, signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCI Act, which created an abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA-licensed reference biological product. This amendment to the PHS Act attempts to minimize duplicative testing. Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical trial or trials. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product and, for products administered multiple times, the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. However, complexities associated with the larger, and often more complex, structure of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation that are still being worked out by the FDA.

The BPCI Act provides a reference biologic for 12 years of exclusivity from the time of first licensure of the reference product. On June 23, 2016, the Price Relief, Innovation, and Competition for Essential Drugs (PRICED) Act (H.R. 5573) was introduced, which would have reduced exclusivity for reference biological drugs from 12 to seven years. The PRICED Act was reintroduced on June 20, 2019 (H.R. 3379) and, if passed into law, would reduce exclusivity for reference biological drugs from 12 to five years. The BPCI Act also provides that the first biologic product submitted under the biosimilar abbreviated approval pathway that is determined to be interchangeable with the reference product has exclusivity against other biologics submitting under the abbreviated approval pathway for the lesser of (i) one year after the first commercial marketing, (ii) 18 months after approval if there is no legal challenge, (iii) 18 months after the resolution in the applicant's favor of a lawsuit challenging the biologics' patents if an application has been submitted, or (iv) 42 months after the application has been approved if a lawsuit is ongoing within the 42-month period.

Pharmaceutical coverage, pricing and reimbursement

Sales of our products, when and if approved for marketing, will depend, in part, on the extent to which our products will be covered by third-party payors, such as federal, state, and foreign government healthcare programs, commercial insurance and managed healthcare organizations. These third-party payors are increasingly reducing reimbursements for medical products, drugs and services. In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. Adoption of price controls and cost containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our net revenue and results. Decreases in third-party reimbursement for our product candidates or a decision by a third-party payor not to cover our product candidates could reduce physician usage of our products once approved and have a material adverse effect on our sales, results of operations and financial condition. Given the potential for long term durable therapeutic benefit from the single administration of a gene therapy product, the question of appropriate pricing and method of payment, including annuity payments and "pay for performance" schemes, is currently an active discussion and, depending on outcome, could affect the use of our products and our financial performance.

Other healthcare laws

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, state and federal anti-kickback, fraud and abuse, false claims, privacy and security and physician sunshine laws and regulations, many of which may become more applicable to us if our product candidates are approved and we begin commercialization. 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 penalties, including, without limitation, civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability to operate our business and our financial results.

Research and Development

Our research and development expenses were $35.8 million and $33.2 million for the years ended June 30, 2020 and 2019, respectively.

Employees

As of June 30, 2020, we had 83 full-time employees, 48 of whom have Ph.D., M.D. or other post-graduate degrees. Of these full-time employees, 60 were engaged in research and development activities and 23 were engaged in finance, human resources, facilities and general management.

Effective July 1, 2019 and through June 30, 2020, all of our personnel were co-employees of AGTC and a professional human resource service organization, Insperity PEO Services, L.P., or Insperity. Insperity replaced TriNet HR Corporation as our professional human resource service organization on July 1, 2019. Under our agreement with Insperity, Insperity is a co-employer of our personnel and is responsible for administering all payroll functions, including tax withholding, and providing health insurance and other benefits to those individuals. We reimburse Insperity for these costs and pay Insperity an administrative fee for its services. We are responsible for, and control, all aspects of the hiring, retention, compensation, management and supervision of our personnel. We consider the terms of our contract with Insperity to be reasonable and customary and believe that this arrangement provides substantial benefit to us in the form of lower costs for employee benefits and a reduced administrative burden on us.

We have no collective bargaining agreements with our employees, and we have not experienced any work stoppages. We consider our relations with our employees to be good.

Corporate information

We were incorporated in Florida in January 1999 and reincorporated in Delaware in October 2003. On April 1, 2014, we completed our initial public offering of our common stock, which is traded on the Nasdaq Global Market under the symbol "AGTC." Our principal executive offices are located at 14193 NW 119th Terrace, Suite 10, Alachua, Florida 32615, and our telephone number is (386) 462-2204. Our corporate website address is www.agtc.com. Through our website, we make available, free of charge, our annual report on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and any amendments to those reports, as well as proxy statements, and, from time to time, other documents as soon as reasonably practicable after we electronically file such materials with, or furnish it to, the Securities and Exchange Commission. Information contained on or accessible through our website is not a part of this annual report.

We use "AGTC" and the double helix logo as trademarks in the United States and other countries. As of June 30, 2020, these trademarks have been registered in the United States, the European Union and Japan.

This annual report contains references to our trademarks and to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to in this annual report, including logos, artwork and other visual displays, may appear without the ® or ™ symbols, but such references are not intended to indicate that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to those trademarks and trade names. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any such companies.

Item 1A. RISK FACTORS

You should carefully consider the risks and uncertainties described below, together with the information included elsewhere in this Annual Report on Form 10-K and other documents we file with the Securities and Exchange Commission (the SEC). The risks and uncertainties described below are those that we have identified as material, but are not the only risks and uncertainties facing us. Our business is also subject to general risks and uncertainties that affect many other companies, such as overall U.S. and non-U.S. economic and industry conditions, including a global economic slowdown, geopolitical events, changes in laws or accounting rules, fluctuations in interest and exchange rates, terrorism, international conflicts, major health concerns, natural disasters or other disruptions of expected economic and business conditions. Additional risks and uncertainties not currently known to us or that we currently believe are immaterial also may impair our business operations and liquidity.

Risks related to our financial condition and capital requirements

We have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future.

We are a clinical-stage biotechnology company, and we have not yet generated revenue from product sales. With the exception of the fiscal year ended June 30, 2017, in which we reported net income of $0.4 million due in part to the amortization associated with a former collaboration agreement with Biogen MA, Inc., a wholly owned subsidiary of Biogen Inc., or Biogen, we have incurred losses from operations in each year since our inception in 1999. For the fiscal years ended June 30, 2020 and 2019, we reported net losses of $45.9 million and $2.0 million, respectively. As of June 30, 2020, we had an accumulated deficit of $181.4 million. Our prior losses, combined with expected future losses, have had and may continue to have an adverse effect on our stockholders' equity and working capital.

We have devoted most of our financial resources to research and development, including our clinical and preclinical development activities. To date, we have financed our operations primarily through the sale of equity securities and, to a lesser extent, through debt financing, research grants from third parties or milestone payments from a collaborator. The amount of our future net losses will depend, in part, on the rate of our future expenditures and our ability to obtain funding through equity or debt financings, strategic collaborations or additional grants. We anticipate that it will be several years, if ever, before we have a product candidate ready for commercialization. Even if we obtain regulatory approval to market a product candidate, our future revenue will depend upon the size of any markets in which our product candidates have received approval, and our ability to achieve sufficient market acceptance, reimbursement from third-party payors and adequate market share for our product candidates in those markets.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- continue our research and preclinical and clinical development of our product candidates;
- expand the scope of our current clinical trials for our product candidates;
- initiate additional preclinical studies, clinical trials or other studies for our product candidates;
- further develop our gene therapy platform, including the process for design, delivery and manufacturing of our vectors for our product candidates;
- change or add additional manufacturers or suppliers;
- seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials;
- establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval;
- seek to identify and validate additional product candidates;
- acquire or in-license other product candidates and technologies;
- make milestone or other payments under any in-license agreements;
- maintain, protect and expand our intellectual property portfolio;
- attract and retain skilled personnel;
- create additional infrastructure to support our operations as a public company and our product development and planned future commercialization efforts; and
- experience any delays or encounter issues with any of the above.

The net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance. In any particular quarter or quarters, our operating results could be below the expectations of securities analysts or investors, which could cause our stock price to decline.

Our ability to generate revenue from product sales is highly uncertain and we may never achieve or sustain profitability, which could depress the market price of our common stock, and could cause you to lose part or all of your investment.

All of our revenue generated to date has come from research grants from third parties or license fees or milestone payments from collaborations. Our ability to generate substantial revenue and achieve profitability depends on

our ability, alone or with strategic collaboration partners such as Bionic Sight and Otonomy, Inc., to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our product candidates. We do not anticipate generating revenue from product sales for at least the next several years, if ever. If any of our product candidates fail in clinical trials or do not gain regulatory approval, or if any of our product candidates, if approved, fail to achieve market acceptance, we may never become profitable. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our ability to generate future revenue from product sales depends heavily on our success in:

- completing research and preclinical and clinical development of our product candidates;
- seeking and obtaining regulatory and marketing approvals for product candidates for which we complete clinical trials;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate (in amount and quality) products and services to support clinical development and the market demand for our product candidates, if approved;
- launching and commercializing product candidates for which we obtain regulatory and marketing approval, either by collaborating with a partner or, if launched independently, by establishing a sales, marketing and distribution infrastructure;
- obtaining and maintaining adequate coverage and reimbursement from third-party payors for our product candidates;
- obtaining market acceptance of our product candidates and gene therapy as a viable treatment option;
- addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- identifying and validating new gene therapy product candidates;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- attracting, hiring and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate, particularly to the extent that we seek to commercialize any product for an indication that has a patient population significantly larger than those addressed by our current lead product candidates. Our expenses could increase beyond expectations if we are required by the FDA, the EMA or other regulatory agencies, domestic or foreign, to perform clinical trials and other studies in addition to those that we currently anticipate. Even if we are able to generate revenue from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations. Our failure to become and remain profitable would depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations.

We are party to a loan and security agreement that contains operating and financial covenants that may restrict our business and financing activities and we may be required to repay the outstanding indebtedness in an event of default, which could have a materially adverse effect on our business.

In June 2020, we entered into a Loan and Security Agreement with the several banks and other financial institutions or entities that are from time to time parties to the Loan Agreement, referred to herein as the Lenders, and Hercules Capital, Inc., in its capacity as administrative agent and collateral agent for itself and Lenders,

providing for a term loan to us with an aggregate principal amount of up to $25.0 million. This term loan consists of a term loan advance in an aggregate principal amount of $10.0 million on June 30, 2020 and a right to request that the Lenders make, in the Lenders' sole discretion, additional term loan advances to us in an aggregate principal amount of up to $15.0 million. The Loan Agreement contains customary representations, warranties and both affirmative and negative covenants applicable to us. The negative covenants include, among other things, agreements by us limiting additional indebtedness, liens (including a negative pledge on intellectual property and other assets), guaranties, mergers and consolidations, substantial asset sales, investments and loans, certain corporate changes, transactions with affiliates, and fundamental changes.

The covenants, restrictions and obligations in our Loan Agreement, as well as any future financing agreements that we may enter into, may restrict our ability to finance our operations, engage in business activities or expand or fully pursue our business strategies. Our ability to comply with these covenants may be affected by events beyond our control, and we may not be able to meet those covenants. A breach of any of these covenants could result in a default under the Loan Agreement, which could cause all of the outstanding indebtedness under the facility to become immediately due and payable.

If we are unable to generate sufficient cash to repay our debt obligations when they become due and payable, either when they mature, or in the event of a default, we may not be able to obtain additional debt or equity financing on favorable terms, if at all, which may negatively impact our business operations and financial condition.

In order to obtain regulatory approval for and commercialize our product candidates, we will need to raise additional funding in the future, which may not be available on acceptable terms, or at all. Failure to obtain necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.

Other than our product candidates for the treatment of XLRP, ACHM CNGB3 and ACHM CNGA3, all of our lead programs in orphan ophthalmology and otology are currently in preclinical development. Developing gene therapy products is expensive, and we expect our research and development expenses to increase substantially as we advance our current product candidates in clinical trials and as we undertake preclinical studies of new product candidates.

Our operations have consumed substantial amounts of cash since inception. As of June 30, 2020 and 2019, our cash and cash equivalents and investments amounted to $80.5 million and $82.0 million, respectively. Our research and development expenses were $35.8 million and $33.2 million for the fiscal years ended June 30, 2020 and 2019, respectively. We believe that our existing cash and cash equivalents at June 30, 2020 will be sufficient to enable us to advance planned preclinical studies and clinical trials for our lead product candidates into the fourth quarter of calendar year 2021. In order to complete the process of obtaining regulatory approval for our lead product candidates and to build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize our lead product candidates, if approved, we will require substantial additional funding. Also, our current operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements, or a combination of these approaches.

Any such fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, financing may not be available to us in the future in sufficient amounts or on terms acceptable to us, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all of our stockholders. The incurrence of additional indebtedness would result in increased fixed payment obligations and a portion of our

operating cash flows, if any, being dedicated to the payment of principal and interest on such indebtedness, and we may be required to agree to additional restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available, and we may be required to relinquish or license on unfavorable terms rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, financial condition, results of operations and prospects and cause the price of our common stock to decline.

Effective March 2019, Biogen terminated its Collaboration Agreement with us relating to developing, seeking regulatory approval for and commercializing gene therapy products to treat XLRS and XLRP based on our AAV vector technologies, and certain discovery programs using our AAV technology. Consequently, we are not entitled to receive any future milestone-based or royalty payments under that agreement, which makes it more likely that we will need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements, or a combination of these approaches. If we are unable to obtain needed funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition, results of operations and prospects and cause the price of our common stock to decline.

Our ability to raise capital may be materially adversely impacted by the COVID-19 pandemic.

We have funded our operations and capital spending, in part, through proceeds from the sale of our capital stock, debt financings and collaboration agreements. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we anticipate that we will require additional capital to fund our operations, which we may raise through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements. Any sustained disruption in the capital markets from the COVID-19 pandemic could negatively impact our ability to raise capital from the offering of equity or debt securities. In addition, the safety measures that have been implemented, and may continue to be implemented, by national, state and local governments, including quarantines, border closures, travel restrictions, shelter-in-place orders and shutdowns, are disrupting normal business operations both in and outside of affected areas and have had significant negative impacts on businesses and financial markets worldwide that could negatively impact our ability to secure funding through collaborations, strategic alliances and licensing arrangements.

Risks related to the discovery and development of our product candidates

All of our product candidates are in preclinical or clinical development. Clinical drug development is expensive, time consuming and uncertain, and we may ultimately not be able to obtain regulatory approvals for the commercialization of some or all of our product candidates.

The research, testing, manufacturing, labeling, approval, selling, marketing and distribution of drug products are subject to extensive regulation by the FDA and other regulatory authorities, which regulations differ from country to country. Our product candidates are in various stages of development and are subject to the risks of failure typical of drug development. The development and approval process is expensive and can take many years to complete, and its outcome is inherently uncertain. We have not submitted an application for or received marketing approval for any of our product candidates. We have limited experience in conducting and managing the later-stage clinical trials necessary to obtain regulatory approvals, including approval by the FDA. To receive regulatory approval, we must, among other things, demonstrate with substantial evidence from clinical trials that

the product candidate is safe, pure and effective for each indication for which approval is sought, and failure can occur in any stage of development. Satisfaction of the approval requirements typically takes several years and the time needed to satisfy them may vary substantially, based on the type, complexity and novelty of the pharmaceutical product. We cannot predict if or when we might receive regulatory approvals for any of our product candidates currently under development.

The FDA and foreign regulatory authorities also have substantial discretion in the drug approval process. The number and types of preclinical studies and clinical trials that will be required for regulatory approval varies depending on the product candidate, the disease or condition that the product candidate is designed to address, and the regulations applicable to any particular product candidate. Approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions, and there may be varying interpretations of data obtained from preclinical studies or clinical trials, either of which may cause delays or limitations in the approval or the decision not to approve an application. Regulatory agencies can delay, limit or deny approval of a product candidate for many reasons, including:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical or clinical significance required by the FDA or comparable foreign regulatory authorities for approval;
- the patients recruited for a particular clinical program may not be sufficiently broad or representative to establish the safety of the product candidate in the full population of patients with the condition for which we seek approval;
- the clinical trial results may not confirm the positive results from earlier preclinical studies or clinical trials;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to the satisfaction of FDA or comparable foreign regulatory authorities to support the submission of a biologics license application, or BLA, or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;
- regulatory agencies might not approve or might require changes to our manufacturing processes or facilities; or
- regulatory agencies may change their approval policies, clinical development guidelines and recommendations, or adopt new regulations in a manner rendering our clinical data insufficient for approval.

Any delay in obtaining or failure to obtain required approvals could materially adversely affect our ability to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact our stock price. Furthermore, any regulatory approval to market a product candidate may be subject to limitations on the indicated uses for which we may market the product candidate. These limitations may limit the size of the market for the product candidate.

We are not permitted to market our product candidates in the United States or in other countries until we receive approval of a BLA from the FDA or marketing authorization from applicable regulatory authorities outside the

United States. We are also not permitted to promote our product candidates as safe and effective therapies until after receiving approval. Obtaining approval of a BLA can be a lengthy, expensive and uncertain process. If we fail to obtain FDA approval to market our product candidates, we will be unable to sell our product candidates in the United States, which will significantly impair our ability to generate any revenue. In addition, failure to comply with FDA and non-U.S. regulatory requirements may, either before or after product approval, if any, subject our company to administrative or judicially imposed sanctions, including:

- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
- restrictions on the products, distribution, or manufacturing processes;
- warning letters or untitled letters alleging violations;
- civil and criminal penalties;
- injunctions;
- suspension or withdrawal of regulatory approvals;
- product seizures, detentions or import bans;
- voluntary or mandatory product recalls and publicity requirements;
- total or partial suspension of production or distribution or both;
- imposition of restrictions on operations, including costly new manufacturing requirements; and
- refusal to approve pending BLAs or supplements to approved BLAs.

Even if we do receive regulatory approval to market a product candidate, any such approval may be subject to limitations on the indicated uses for which we may market the product. It is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain the appropriate regulatory approvals necessary for us or our collaborators to commence product sales. Any delay in obtaining, or an inability to obtain, applicable regulatory approvals would prevent us from commercializing our product candidates, generating revenue and achieving and sustaining profitability.

Our gene therapy product candidates are based on a novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. To date, five gene therapy products have been approved in the United States and seven such products have been approved in Europe.

We have concentrated our product research and development efforts on our gene therapy platform, and our future success depends on the successful development of this approach. There can be no assurance that any development problems we experience in the future related to our gene therapy platform will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience unanticipated problems or delays in expanding our manufacturing capacity or transferring our manufacturing process to commercial partners, which may prevent us from completing our clinical trials or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical trial requirements of the FDA, the EMA and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidates. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates.

The regulatory framework for evaluating and approving gene and cell therapy products has changed frequently and may continue to change in the future. For example, the FDA established the Office of Tissues and Advanced

Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue, and Gene Therapies Advisory Committee to advise CBER on its review. Before a clinical trial can begin at a study site, that institution's Institutional Review Board, or IRB, and its Institutional Biosafety Committee, or IBC, have to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other regulatory bodies to change their standards for the quantity and quality of data needed to support approval of any 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 studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these 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 for orphan ophthalmology product candidates. 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 to maintain our business.

Interim data and ad hoc analyses are preliminary in nature. Success in animal studies or early clinical trials may not be indicative of results obtained in later trials.

Trial designs and results from animal studies or early clinical trials are not necessarily predictive of our future clinical trial designs or results, and interim results of a clinical trial are not necessarily indicative of final results. Our product candidates may also fail to show the desired safety and efficacy in clinical development despite demonstrating positive results in animal studies or having successfully advanced through initial clinical trials. There can be no assurance that the success we achieved in the animal studies or interim data for our lead product candidates will result in success in our clinical trials of those product candidates. For example, we have reported interim results, from our XLRP Phase 1/2 trial, that 7 of the 15 centrally treated patients from all three dose groups combined had at least 5 loci that increased by at least 7 dB– 6 patients at Month 6 and 1 patient at Month 3; however, we do not have a set of Month 12 data available and there is no assurance that the data obtained at Month 12 or from our Phase 2/3 study will indicate a clinically meaningful benefit or support the submission of a BLA.

There is a high failure rate for drugs and biological products proceeding through clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in later stage clinical trials even after achieving promising results in earlier stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our product candidate development.

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on the speed at which we can recruit patients to participate in testing our product candidates as well as completion of required follow-up periods. If patients are unwilling to participate in our gene therapy studies because of negative publicity from adverse events in the biotechnology or gene therapy industries or for other reasons, including competitive clinical trials for similar patient populations, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of our product candidates may be delayed. For example, trials using a high-dose AAV therapy have led to several well-publicized adverse events, including reported deaths related to sepsis. If there are delays in accumulating the

required number of clinical events in trials for our product candidates where clinical events are a primary endpoint, there may be delays in completing the trial. These delays could result in increased costs, delays in advancing our product candidates, delays in testing the effectiveness of our technology or termination of the clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with the required or desired characteristics to achieve diversity in a trial, to complete our clinical trials in a timely manner. For example, enrolling eligible patients in novel orphan-drug trials can be challenging. We have experienced delays in enrollment of our pediatric patients in the dose escalation portions of our CNGB3 and CNGA3 trials for ACHM in connection with the COVID-19 pandemic and we previously encountered slower-than-expected enrollment in our Phase 1/2 clinical trial for our XLRS product candidate as a result of patients not meeting one or more study eligibility criteria. Challenges such as these in enrolling a sufficient number of patients to conduct our clinical trials as planned, may cause us to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business. We could also encounter delays if physicians encounter unresolved ethical issues associated with enrolling patients in clinical trials of our product candidates.

In particular, most of the conditions for which we plan to evaluate our product candidates are rare genetic disorders with limited patient pools from which to draw for clinical trials. The eligibility criteria of our clinical trials will further limit the pool of available trial participants.

Patient enrollment is affected by factors including:

- severity of the disease under investigation;
- design of the clinical trial protocol;
- size and nature of the patient population;
- eligibility criteria for the trial in question;
- perceived risks and benefits of the product candidate under trial;
- proximity and availability of clinical trial sites for prospective patients;
- availability of competing therapies and clinical trials;
- clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians; and
- our ability to monitor patients adequately during and after treatment.

We plan to seek initial marketing approval for our product candidates in the United States and the European Economic Area, or EEA. We may not be able to initiate or continue clinical trials if we cannot enroll a sufficient number of eligible patients to participate in the clinical trials required by the FDA, the EMA or other foreign regulatory authorities. Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with contract research organizations, or CROs, and physicians;
- different standards for conducting clinical trials;
- our inability to locate qualified local consultants, physicians and partners; and

- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatments.

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

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of such product candidates in humans. Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. Our clinical trials have and may continue to be delayed by the necessity to re-test study agent, the decision to use a single surgeon to treat patients and protocol amendments that require approval by institutional review boards at the clinical sites. A failure of one or more clinical trials can occur at any stage of testing.

Events that may prevent successful or timely completion of clinical development include:

- delays in raising, or inability to raise, sufficient capital to fund the planned clinical trials;
- inability to generate sufficient preclinical, toxicology, or other data to support the initiation of human clinical trials;
- delays in reaching a consensus with regulatory agencies on trial design;
- identifying, recruiting and training suitable clinical investigators;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- delays in obtaining required IRB and IBC approval at each clinical trial site;
- delays in recruiting suitable patients to participate in our clinical trials;
- delays due to changing standard of care for the diseases we are targeting;
- adding new clinical trial sites;
- imposition of a clinical hold by regulatory agencies, after review of an IND application or equivalent application or an inspection of our clinical trial operations or trial sites;
- failure by our CROs, other third parties or us to adhere to clinical trial requirements;
- loss of product due to shipping delays or delays in customs in connection with delivery to foreign countries for use in clinical trials;
- failure to perform in accordance with the FDA's good clinical practice, or GCP requirements and guidelines or similar regulatory requirements and guidelines in other countries;
- delays in the manufacture, testing, release, import or export for the use of sufficient quantities of our product candidates for the use in clinical trials by our vendors, such as the vendor testing errors previously experienced in our ongoing clinical trials; failure by us or our vendors to manufacture our product candidate in accordance with FDA's current good manufacturing practice, or GMP, requirements or similar regulatory requirements and guidelines in other countries;
- delays by us or our contract vendors in the testing, validation and delivery of our product candidates to the clinical trial sites;
- delays in having patients' complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites deviating from trial protocol or clinical trial sites or patients dropping out of a trial;

- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- changes in regulatory requirements, FDA policy, and guidance that require amending or submitting new clinical protocols;
- the costs of clinical trials of our product candidates may be greater than we anticipate; or
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon drug development programs.

For example, in connection with the clinical development of our XLRP product candidate, we received comprehensive written feedback regarding the design and execution of our proposed registration trial and future regulatory submissions. Based on this feedback, we have revised our development plan to include expansion of the current trial in parallel with the planned Phase 2/3 trial, which is designed to evaluate sustained efficacy across multiple measures of potential benefit in patients with XLRP. While the Company continues to move forward as planned with manufacturing, clinical site preparation and other activities to enable initiation of the studies as quickly as possible, such expansion will result in additional costs and may delay the completion of the clinical development of our XLRP product candidate.

Further, a clinical trial may be suspended or terminated by us, our collaborators, the IRBs, in the institutions in which such trials are being conducted, the Data Safety Monitoring Board, or DSMB, for such trial, or by the FDA or other regulatory authorities due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, information from an inspection or other source that raises significant concerns about the integrity of the clinical trial data, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. In appropriate circumstances, we may also elect to temporarily suspend an ongoing clinical trial to further study unexpected results, even if those results would not require us to formally suspend the trial under the applicable regulatory requirements or clinical protocols. Such temporary suspension could include further testing of trial materials and the need to review subject responses to ensure safety. If we experience termination of, or delays in the completion of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenue from any of these product candidates will be delayed. Furthermore, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenue from product sales, regulatory and commercialization milestones and royalties. In addition, if we or our third-party collaborators make manufacturing or formulation changes to product candidates, we or they may need to conduct additional trials to bridge the modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

If the results of our clinical trials are inconclusive or if there are safety concerns or adverse events associated with our product candidates, we may:

- be delayed in obtaining marketing approval for our product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;

- be subject to changes with the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and impair our ability to commercialize our product candidates.

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

As with many pharmaceutical and biological products, treatment with our product candidates may produce undesirable side effects or adverse reactions. These adverse reactions may occur despite our belief that our AAV vectors have a generally acceptable safety profile.

Known adverse reactions that could occur with treatment with AAV vectors include an immunologic reaction to the capsid protein or gene at early time points after administration. In previous clinical trials involving AAV viral vectors for gene therapy, some subjects experienced serious adverse events, including the development of T-cell response due to immune response against the vector capsid proteins. If our vectors demonstrate a similar effect, or other adverse events, we may be required to halt or delay further clinical development of our product candidates. In addition, theoretical adverse reactions of AAV vectors include replication and spread of the virus to other parts of the body and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, also known as cancer, which could potentially enhance the risk of malignant transformation. Potential procedure-related adverse reactions, including inflammation, can also occur and have, in fact, been observed in our XLRS, XLRP and ACHM CNGB3 trials. There is also the potential risk of delayed adverse reactions following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. If any such adverse reactions occur, our clinical trials could be suspended or terminated and the FDA, the EMA or other foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. The product-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial. If we elect or are required to delay, suspend or terminate any clinical trial of any of our product candidates, the commercial prospects of such product candidates will be harmed and our ability to generate product revenue from any of these product candidates will be delayed or eliminated. Any of these occurrences may harm our business, financial condition and prospects significantly.

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

- regulatory authorities may withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;

- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent or delay us from achieving or maintaining market acceptance of our product candidates and could significantly harm our business, prospects, financial condition and results of operations.

We may be unable to obtain orphan-drug designation or exclusivity for some of our product candidates. If our competitors are able to obtain approval and orphan-drug exclusivity for their products that are considered to be the same as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals diagnosed annually in the United States, or a patient population equal to or greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, the EMA's Committee for Orphan Medicinal Products, or COMP, grants orphan-drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union Community. Additionally, designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biological product. Our product candidates for the treatment of XLRS, ACHM (in the form caused by mutations in the CNGB3 and CNGA3 genes) and XLRP (in the form caused by mutations in the RPGR gene) have been granted orphan medicinal product designation by the FDA and the European Commission. We may request orphan-drug designation for our other product candidates in the future but there can be no assurances that the FDA will grant any of our product candidates such designation. Additionally, the designation by the FDA of any of our product candidates as an orphan drug does not guarantee that the FDA will accelerate regulatory review of or ultimately approve that product candidate. Orphan-drug designation may also be rescinded before approval if the FDA concludes that the product candidate no longer meets the criteria for designation.

Generally, if a product candidate with an orphan-drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the EMA or the FDA from approving another marketing application for the same drug and the same indication during the exclusivity period, except in limited circumstances. The FDA has not defined the meaning of "same drug" specifically for gene therapy products in a regulation. In January 2020, the FDA issued draft guidance in which the FDA proposed its interpretation of the "sameness" criterion for determining whether a gene therapy product would be eligible for orphan-drug exclusivity. The FDA proposed to consider two gene therapy products for the same indication to be different, thus each eligible for market exclusivity, if they have different transgenes and different vectors, different transgenes regardless of whether they have the same vectors, or different vectors from a different viral class. The public docket to submit comments on the draft guidance closed on July 28, 2020. There is no required timeframe within which the FDA must complete its review of comments. The FDA could decide to revise the sameness guidance, finalize it as proposed, or withdraw it. It is possible that the FDA could conclude that no two gene therapy products could ever

be considered the same, thus precluding any gene therapy product from obtaining orphan product exclusivity. The applicable orphan exclusivity period is seven years in the United States and 10 years in Europe. The European exclusivity period can be reduced to six years if a product no longer meets the criteria for orphan-drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure the regulatory body that it can provide sufficient quantity of the product to meet the needs of patients with the rare disease or condition, or if a gene therapy product considered to be the same as our product candidate is superior in certain respects.

Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different drugs can be approved for the same condition. In the United States, even after an orphan drug is approved, the FDA can subsequently approve another drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

Even if we complete the necessary clinical trials, we cannot predict when or if we will obtain regulatory approval to commercialize a product candidate or the approval may be for a narrower indication than we expect.

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates demonstrate safety and efficacy in clinical trials, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA advisory committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical trials and the review process. Regulatory agencies also may approve a product candidate for fewer or more limited indications than requested, may not approve the price we intend to charge for our product candidate, may impose significant limitations in the form of narrow indications, warnings, precautions or contra-indications with respect to conditions of use or may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

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

Even if we obtain regulatory approval in a jurisdiction for our product candidates, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, recordkeeping, and submission of safety and other post-market information. Any regulatory approvals that we receive for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. FDA guidance advises that patients treated with some types of gene therapy undergo follow-up observations for potential adverse events and this follow-up may extend for many years. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials and claims must be consistent with approved labeling and be in compliance with FDA regulations as well as other potentially applicable federal and state laws. In addition, biological product advertising and promotional materials intended to be used during the first 120 days after approval must be submitted to the FDA during the BLA review period. After approval, advertising and promotional materials must be submitted to the FDA 30 days prior to their intended use.

In addition, product manufacturers are subject to payment of program fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with GMP requirements and adherence to commitments made in the BLA or foreign marketing application. 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 or with the integrity or sufficiency of data, records, or documentation, or disagrees with the promotion, marketing or labeling of that product, a regulatory agency may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, a regulatory agency may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners;
- restrict the marketing or manufacturing of the product;
- seize or detain product or otherwise require the withdrawal of product from the market;
- refuse to permit the import or export of products; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law 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 revenue.

In addition, the FDA's policies may change and additional government laws may be enacted and implementing regulations promulgated, which could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations.

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

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries must also approve the manufacturing and marketing of the product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States,

including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our products, if approved, is also subject to approval. We intend to submit a marketing authorization application to the EMA for approval in the EEA, but obtaining such approval is a lengthy and expensive process and the EMA has its own procedures for approval of product candidates. Even if a product candidate is approved, the FDA or the EMA, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the EEA also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries and regulatory approval of a product candidate in one country does not ensure approval in any other country, while a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in others. Also, regulatory approval for any of our product candidates may be withdrawn. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business will be adversely affected.

The recent outbreak of COVID-19 could materially and adversely affect our ability to conduct clinical trials and engage with our third-party vendors and thereby have a material adverse effect on our financial results.

The FDA has indicated that the staff of the Center for Biologics Evaluation and Research continue to operate according to historical timelines despite the allocation of substantial resources to address the COVID-19 pandemic. The FDA has noted, however, that its current levels of performance may be impacted by the workload created by COVID-19 activities. It is possible that COVID-19 activities could be prioritized in the future, which could delay future meetings or preclude in-person meetings with the FDA regarding next-phase clinical study design for our product candidates, including XLRP, and thus could delay their development programs. Any decision by the FDA to delay or refuse meeting with us or to limit communications with us in light of COVID-19 could have a material adverse effect on our scheduled clinical trials, which could increase our operating expenses and have a material adverse effect on our financial results.

We have experienced delays in enrollment of our pediatric patients in the dose escalation portions of our CNGB3 and CNGA3 trials for ACHM in connection with the COVID-19 pandemic. In addition, we could experience delays in critical follow-up visits required under clinical trial protocols, which could increase the cost of those trials and also impact their expected timelines. Our ability to fully interpret the trial outcomes and the ability of certain lab-based employees to perform their jobs due to stay-at-home orders or other restrictions related to COVID-19 could also result in delays and increase our operating expenses. We have engaged a mobile vision center as an alternative method of data acquisition in an effort to maintain existing timelines for our programs. However, this method of data acquisition and/or other methods to respond to the impact of COVID-19 has, and may continue to, increase our operating costs.

Furthermore, third-party vendors, such as raw material suppliers and contracted manufacturing, testing or research organizations, could also be impacted by COVID-19, which could result in unavoidable delays and/or increases in our operating costs.

The extent to which COVID-19 may impact our clinical trials and our dealings with vendors will depend on future developments, which are highly uncertain and cannot be predicted with confidence, such as the duration of the outbreak, the severity of COVID-19, and the effectiveness of actions to contain and treat those who have contracted COVID-19.

Risks related to our reliance on third parties

We expect to rely on third parties to conduct aspects of our product manufacturing and protocol development, and these third parties may not perform satisfactorily.

We do not expect to independently conduct all aspects of our vector production, product manufacturing, protocol development, and monitoring and management of our ongoing and planned preclinical and clinical programs. We have expanded our internal capabilities to include a full-scale pilot facility to facilitate continued improvement in our manufacturing process. We currently rely, and expect to continue to rely, to a significant degree, on third parties for the production of our clinical trial materials. In such cases, we expect to control only certain aspects of their activities.

Under certain circumstances, these third parties may be entitled to terminate their engagements with us or we may seek to terminate our engagement with them. Because of the complexities inherent in gene therapy manufacturing, we expect that any engagement by us of a new third-party manufacturer for our product candidates would take a substantial amount of time to establish. Accordingly, if we need to enter into alternative arrangements, it could delay our product development activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study and trial protocols. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study and trial plans and protocols, or if there are disagreements between us and these third parties, we will not be able to complete, or may be delayed in completing, the preclinical studies and clinical trials required to support future IND submissions and approval of our product candidates. In some such cases, we may need to locate an appropriate replacement third-party relationship, which may not be readily available or on acceptable terms, which would cause additional delay with respect to the approval of our product candidates and would thereby have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- delays in the production of our product candidates associated with transitioning to a new third-party manufacturer;
- reduced control as a result of using third-party manufacturers for all aspects of manufacturing activities;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier.

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

We and our contract manufacturers are subject to significant regulatory oversight with respect to manufacturing our products. The manufacturing facilities on which we rely may not continue to meet regulatory requirements and may have limited capacity.

All parties involved in the preparation of therapeutics for clinical trial or commercial sale are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage

clinical trials must be manufactured in accordance with GMP requirements. These regulations govern manufacturing processes and procedures (including recordkeeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA on a timely basis and must adhere to the FDA's GMP requirements enforced by the FDA through its facilities inspection program. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

The regulatory authorities also may, at any time following approval of a product for sale, audit our manufacturing facilities or those of our third-party manufacturers. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or our third-party manufacturers to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a manufacturing facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

If we or any of our third-party manufacturers or testing contractors fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new product candidate, or revocation of a pre-existing approval. Such an occurrence may cause our business, financial condition and results of operations to be materially harmed.

Additionally, if supply from an approved manufacturer is interrupted, there could be a significant disruption in commercial supply of our products. Because of the complexities inherent in our gene therapy manufacturing, we expect that there will be a significant period of time following our engagement of an alternative third-party manufacturer before that manufacturer will be in a position to provide an adequate supply of our product candidates for our clinical trials. In addition, any alternative manufacturer will also need to be qualified through a BLA supplement which could result in further delay. The regulatory agencies may also require additional trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed, or we could lose potential revenue.

We expect to rely on third parties to conduct and supervise our clinical trials, and if these third parties perform in an unsatisfactory manner, it may harm our business.

We expect to continue to rely on academic research institutions and CROs along with clinical trial sites to ensure our clinical trials are conducted properly and on time. While we will have agreements governing their activities, we will have limited influence over their actual performance and will control only certain aspects of our CROs' activities. Nevertheless, we will be responsible for ensuring that each of our clinical trials is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities.

We and our CROs are required to comply with the FDA's and other regulatory authorities' GCP, GMP and good laboratory practice, or GLP, requirements for conducting, recording and reporting the results of our preclinical studies and clinical trials to assure that the data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical trial participants are protected. The FDA enforces these requirements through periodic inspections of study sponsors, principal investigators and clinical trial sites. If we or our CROs fail to comply with applicable GCP requirements, the clinical data generated in our future clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving any marketing applications. Upon inspection, the FDA may determine that our clinical trials did not comply with GCP requirements, which may render the data generated in those trials unreliable. In addition, our future clinical trials will require a sufficient number of test subjects to evaluate the safety and effectiveness of our product candidates. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of patients, we may be required to repeat such clinical trials, which would delay the regulatory approval process.

Our CROs are not our employees, and, except for remedies available to us under our agreements with such CROs, we are therefore unable to directly monitor whether or not they devote sufficient time and resources to our clinical and nonclinical programs. Our CROs also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenue could be delayed.

Switching or adding CROs involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, prospects, financial condition and results of operations.

We also expect to rely on other third parties to store and distribute our vectors and products for any clinical trials that we may conduct. Any performance failure on the part of our distributors could delay clinical development, regulatory review or marketing approval of our product candidates or commercialization of our products, if approved, producing additional losses and depriving us of potential product revenue.

Collaborations with third parties may be important to our business. If these collaborations are not successful, our business could be adversely affected.

In addition to our current collaborations, we may in the future seek third-party collaborators for the development and commercialization of product candidates based on our gene therapy platform. If we enter into such collaborations, we will have limited control over the amount and timing of resources that our collaborators will dedicate to the development or commercialization of our product candidates. Our ability to generate revenue from any future collaboration or license agreement will depend on the collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, any collaborators may have the right to abandon research or development projects and terminate applicable agreements, including any funding obligations, prior to or upon the expiration of the agreed upon terms. For example, on December 7, 2018, we received notice from Biogen that the Biogen collaboration agreement would be terminated effective March 8, 2019. As a result of the termination, we will not receive any future milestone-based or royalty payments under the Biogen collaboration agreement.

Our current collaborations or any collaboration that we enter into in the future may pose a number of risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- exclusivity rights we negotiate with our collaborators may be unenforceable in certain jurisdictions;
- collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators may decide not to continue the development of collaboration products and could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- take-over or step-in rights granted to a collaborator with respect to one or more of our product candidates may cause us to have limited control over future development activities and/or realize diminished economic or other benefits upon the ultimate commercialization of that product candidate;
- a collaborator with marketing, distribution and commercialization rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;
- if we fail to obtain orphan product designation for a partnered product, we may realize diminished economic benefit upon the ultimate commercialization of that product candidate;
- restrictions and commitments contained in collaborations may have the effect of preventing us from independently undertaking development and other efforts that may appear to be attractive to us;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development of any product candidates, might cause delays or termination of the research, development or commercialization of such product candidates, might lead to additional responsibilities for us with respect to such product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- collaborations may be terminated at the convenience of the collaborator or for a material breach by either party, and, if a collaboration is terminated, we could be required to make payments to the collaborator or have our potential payments under the collaboration reduced; and

- in the event of the termination of a collaboration, like the termination of the Biogen collaboration agreement, we could be required to raise additional capital to pursue further development or commercialization of the product candidates returned to us by our former collaborator.

If our collaborations do not result in the successful development and commercialization of products or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of our gene therapy platform and product candidates could be delayed and we may need additional resources to develop product candidates and gene therapy platform. As a result of these or other factors, we may not receive the benefits that we expect from our collaborations.

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

We may in the future determine to collaborate with other pharmaceutical and biotechnology companies for development and potential commercialization of product candidates. These relationships or those like them may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. For example, in December 2019, as a result of certain milestone triggers outlined in our strategic research and development collaboration agreement with Bionic Sight, we became obligated to purchase additional equity in Bionic Sight for $4.0 million and receive such equity interest based on certain pre-determined valuation criteria. We completed this purchase in March 2020.

In addition, we could face significant competition in seeking appropriate collaborators and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement with any such new party will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for our product candidates because our research and development pipeline may be insufficient, our product candidates may be deemed to be at too early of a stage of development for collaborative effort and third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy. If we license product candidates, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. We cannot be certain that, following a strategic transaction or license, we will achieve the revenue or specific net income that justifies such transaction.

If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to fund and undertake development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates or bring them to market or continue to develop our gene therapy platform and our business may be materially and adversely affected.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we rely on third parties to manufacture our viral vectors and our product candidates, and because we collaborate with various organizations and academic institutions on the advancement of our gene therapy platform, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets.

Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our collaborators, advisors, employees and consultants to publish data potentially relating to our trade secrets. Our academic collaborators typically have rights to publish data, provided that we are notified in advance and may delay publication for a specified time in order to secure our intellectual property rights arising from the collaboration. In other cases, publication rights are controlled exclusively by us, although in some cases we may share these rights with other parties. We also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets in cases where we do not have proprietary or otherwise protected rights at the time of publication. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

Risks related to commercialization of our product candidates

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

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

We face intense competition and rapid technological change and the possibility that our competitors may develop therapies that are more advanced or effective than ours, which may adversely affect our financial condition and our ability to successfully commercialize our product candidates.

The biotechnology and pharmaceutical industries are characterized by intense and rapidly changing competition to develop new technologies and proprietary products, and any product candidates that we successfully develop and commercialize will have to compete with existing therapies and new therapies that may become available in the future. While we believe that our proprietary technology estate and scientific expertise in the gene therapy field provide us with competitive advantages, we face potential competition from many different sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions and governmental agencies and public and private research institutions that may develop potentially competitive products or technologies.

Currently there are no approved products for any of our lead orphan ophthalmology indications of XLRP and ACHM. We believe that the key competitive factors that will affect the success of our product candidates, if approved, are likely to be their efficacy, safety, convenience of administration and delivery, price, the level of generic competition and the availability of reimbursement from government and other third-party payors.

A number of companies have announced that they are working on AAV-based gene therapy technology and there are companies developing gene therapies in the field of orphan ophthalmology, on which we are currently focused, which have programs that are at the clinical and pre-clinical stages. Other companies could also potentially seek to enter this field.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of treatments and the commercialization of those treatments. To the extent that we develop product candidates for indications with larger patient populations, we expect to experience particularly intense competition from larger and better funded pharmaceutical companies. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, market exclusivity provisions for products with orphan-drug designation could severely limit the sales potential for any of our product candidates that do not gain first-to-market approval.

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

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

- a covered benefit under its health plan;

- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining coverage and reimbursement for a product from governmental and private payors is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data for the use of our products. We may not be able to provide data sufficient to receive a positive coverage determination. If coverage and reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. In the United States, third-party payors, including private and governmental payors, such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. The Medicare and Medicaid programs increasingly are used as models for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs and biologics. Currently, no gene therapy products have been approved for coverage under the Medicare program. The Centers for Medicare & Medicaid Services, or CMS, the agency responsible for administering the Medicare program, covers some items and services nationally through National Coverage Determinations. More frequently, coverage determinations for new products are made by the individual Medicare Administrative Contractors, or MACs, that operate the program on a day-to-day basis in their awarded geographic jurisdictions. It is difficult to predict what CMS or the local MACs will decide with respect to coverage and reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products. Moreover, Medicare reimbursement is determined in part based on where the drug or biologic is administered. Drugs or biologics administered in the inpatient setting are bundled along with other services into Diagnosis Related Groups for payment purposes. In the outpatient setting, drugs and biologics such as our product candidates are generally reimbursed at Average Sales Price (ASP) + 6 %. Outside of the United States, agencies in Europe may be more conservative than CMS with respect to reimbursement. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe that the increasing emphasis on cost-containment initiatives in Europe, Canada, and other countries has and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulations could restrict the amount that we are able to charge for our product candidates and delay their commercial launch. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced or delayed compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product

candidates, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and potential legislative changes on both the federal and state levels. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

Negative public opinion and increased regulatory scrutiny of gene therapy and genetic research may damage public perception of our product candidates or adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

Gene therapy remains a novel technology, with only five gene therapy products approved to date in the United States and only seven gene therapy products approved to date in Europe. 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. In particular, our success will depend upon physicians specializing in the treatment of those diseases that our product candidates target prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments they are already familiar with and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, trials using a high-dose AAV therapy have led to several well-publicized adverse events, including reported deaths related to sepsis. Adverse events in our clinical trials or the clinical trials of other gene therapy companies, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental 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.

Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, or the ACA, was passed, which substantially changes the way healthcare is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The ACA, among other things, subjects biologic products to potential competition by lower-cost biosimilars, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations, establishes annual fees and taxes on manufacturers of certain branded prescription drugs, and subjects additional drugs to lower pricing under the 340B drug pricing program by adding new entities to the program.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. On August 2, 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 up to 2% per fiscal year, which went into effect on April 1, 2013. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

Some of the provisions of the ACA have been subject to judicial and Congressional challenges, and we expect there to be further challenges in the future. For example, with enactment of the Tax Cuts and Jobs Act of 2017, or

the TCJA, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas ruled that the individual mandate is a critical and inseverable feature of the Affordable Care Act and, therefore, because it was repealed as part of the TCJA, the remaining provisions of the ACA are invalid as well. The Trump administration and CMS have both stated that the ruling will have no immediate effect, and on December 18, 2019, the Fifth Circuit U.S. Court of Appeals held that the individual mandate is unconstitutional, and remanded the case to the lower court to reconsider its earlier invalidation of the full ACA. Pending review, the ACA remains in effect, but it is unclear at this time how the latest ruling will impact the Affordable Care Act and our business.

Since January 2017, President Trump has also signed two Executive Orders designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. On January 20, 2017, President Trump signed an Executive Order directing federal agencies with authority and responsibility under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. President Trump also signed an Executive Order terminating the cost-sharing subsidies that reimburse insurers under the ACA. During his term, President Trump has been seeking to repeal or replace all or portions of the ACA, but, to date, the federal government has been unable to agree on any such legislation. In the coming years, additional legislative and regulatory changes could be made to governmental health programs that could significantly impact our business. We cannot predict what legislation, if any, intended to repeal or replace the ACA will become law, or what impact any such legislation may have on us or our partners' business and financial condition, if any.

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

Ethical, social and legal concerns about gene therapy and genetic research could result in additional regulations restricting or prohibiting the products and processes we may use. Even with the requisite approvals from the FDA in the United States and other government bodies internationally, the commercial success of our product candidates will depend in part on the medical community's, patients', and third-party payors' acceptance of gene therapy products in general, and our product candidates in particular, as medically necessary, cost-effective, and safe. Any product that we bring to the market may not gain market acceptance by physicians, patients, third-party payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of product candidates over alternative treatments;
- the clinical indications for which the product candidate is approved;
- the safety of product candidates seen in a broader patient group, including its use outside the approved indications;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- the cost of treatment relative to alternative treatments;
- relative convenience and ease of administration;

- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- label limitations required by regulatory authorities, which could limit size of market;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party insurance coverage and reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched. Our efforts to educate the medical community and third-party payors on the benefits of the product candidates may require significant resources and may never be successful. Such efforts to educate the marketplace may require more resources than are required by the conventional technologies marketed by our competitors. If any of our product candidates is approved but fails to achieve market acceptance among physicians, patients, or healthcare payors, we will not be able to generate significant revenue from such product, which could have a material adverse effect on our business, prospects, financial condition and results of operations.

If we obtain approval to commercialize our product candidates outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.

If any of our product candidates are approved for commercialization, we may enter into agreements with third parties to market them on a worldwide basis or in more limited geographical regions. We expect that we will be subject to additional risks related to entering into international business relationships, including:

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the Foreign Corrupt Practices Act of 1977 or comparable foreign regulations;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

These and other risks associated with our international operations may materially adversely affect our ability to attain or maintain profitable operations.

We may not be successful in our efforts to identify or discover additional product candidates.

The success of our business depends primarily upon our ability to identify, develop and commercialize product candidates based on our gene therapy platform. Although certain of our product candidates are currently in clinical or preclinical development, we may fail to identify other potential product candidates for clinical development for a number of reasons. For example, our research methodology may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

If any of these events occur, we may be forced to abandon our development efforts with respect to a particular product candidate, which would have a material adverse effect on our business and could potentially cause us to cease operations. Research programs to identify new product candidates require substantial technical, financial and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful.

Risks related to our business operations

We previously identified a material weakness in our internal control over financial reporting, which has now been remediated. If we fail to maintain an effective system of internal controls over financial reporting, we may not be able to report our financial results timely and accurately, which could adversely affect investor confidence in the Company, and in turn, our results of operations and our stock price.

Effective internal controls are necessary for us to provide reliable financial reports and operate successfully as a public company. Section 404 of the Sarbanes-Oxley Act of 2002, or SOX, requires that companies evaluate and report on their systems of internal control over financial reporting.

As disclosed in our Form 10-K for the fiscal year ended June 30, 2017, we previously identified a material weakness in our internal controls over financial reporting relating to the design and operation of our closing and financial reporting processes. We completed our remediation efforts related to the material weakness and we have not subsequently identified any material weaknesses in our internal controls over financial reporting.

Although we have remediated this material weakness in our internal controls over financial reporting, any failure to maintain effective internal controls could cause a delay in compliance with our reporting obligations, SEC rules and regulations or SOX Section 404, which could subject us to a variety of administrative sanctions, including, but not limited to, SEC enforcement action, ineligibility for short form registration, the suspension or delisting of our common stock from the stock exchange on which it is listed and the inability of registered broker-dealers to make a market in our common stock, which could adversely affect our business and the trading price of our common stock.

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

If we are successful in executing our business strategy, we will need to continue to expand our managerial, operational, financial and other systems and resources to manage our operations, continue our research and development activities, and, in the longer term, build a sales force and commercial infrastructure to support commercialization of any of our product candidates that are approved for sale. Future growth would impose significant added responsibilities on members of management. It is possible that our management, finance,

development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and products requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

We may enter into or seek to enter into business partnerships, combinations and/or acquisitions which may be difficult to integrate, disrupt our business, divert management attention or dilute stockholder value.

A key element of our strategy is to enter into business partnerships, combinations and/or acquisitions. We have limited experience in making acquisitions, which are typically accompanied by a number of risks, including:

- the difficulty of integrating the operations and personnel of the acquired companies;
- the potential disruption of our ongoing business and distraction of management;
- potential unknown liabilities and expenses;
- the failure to achieve the expected benefits of the combination or acquisition;
- the maintenance of acceptable standards, controls, procedures and policies; and
- the impairment of relationships with employees as a result of any integration of new management and other personnel.

If we are not successful in completing acquisitions that we may pursue in the future, we would be required to reevaluate our business strategy and, in the meantime, we may have incurred substantial expenses and devoted significant management time and resources in seeking to complete the acquisitions. In addition, we could use substantial portions of our available cash as all or a portion of the purchase price, or we could issue additional securities as consideration for these acquisitions, which could cause our stockholders to suffer significant dilution.

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

We are highly dependent on our executive officers, the loss of whose services may adversely impact the achievement of our objectives. Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel, will also be critical to our success. There is currently a shortage of skilled executives and scientific personnel in our industry, which is likely to continue. As a result, competition for skilled personnel is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in preclinical studies or clinical trials may make it more challenging to recruit and retain qualified personnel. The inability to recruit or loss of the services of any executive, key employee, consultant or advisor may impede the progress of our research, development and commercialization objectives.

In order to induce valuable employees to remain at AGTC, in addition to salary and cash incentives, we have provided stock options that vest over time. The value to employees of stock options that vest over time may be significantly affected by movements in our stock price that are beyond our control and may at any time be insufficient to counteract more lucrative offers from other companies.

Despite our efforts to retain valuable employees, members of our management, scientific and development teams have in the past and may in the future terminate their employment with us. The loss of the services of any of our executive officers or other key employees and our inability to find suitable replacements could potentially harm our business, prospects, financial condition or results of operations. We do not maintain “key man” insurance

policies on the lives of these individuals or any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level, and senior managers as well as junior, mid-level, and senior scientific and medical personnel.

Many of the other biotechnology and pharmaceutical companies that we compete against for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They may also provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high quality candidates than what we can offer. If we are unable to continue to attract and retain high quality personnel, the rate and success at which we can discover, develop and commercialize product candidates will be limited.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities.

We are exposed to the risk that our employees, CROs, principal investigators, consultants and commercial partners may engage in fraudulent conduct or other illegal activity or may fail to disclosure unauthorized activities to us. Misconduct by these parties could include intentional, reckless and/or negligent failures to comply with:

- the laws and regulations of the FDA and non-U.S. regulators, including those laws requiring the reporting of true, complete and accurate information to such regulatory bodies;
- manufacturing standards that we have established;
- healthcare fraud and abuse laws and regulations in the United States and similar foreign laws; or
- laws requiring the accurate reporting of financial information or data or the disclosure of unauthorized activities to us.

In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these 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, including the imposition of significant fines or other sanctions.

We may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

Our operations may be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws. If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, many of these laws will become more directly applicable to our operations, including, without limitation, the federal Health Care Program Anti-Kickback Statute, the federal civil and criminal False Claims Acts and Physician Payments Sunshine Act and regulations. These laws may impact, among other things, our proposed sales, marketing and educational programs. In

addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include, but are not limited to:

- the federal Health Care Program Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind in return for, the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;
- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other government payers that are false or fraudulent;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act; Other Modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, healthcare clearinghouses and healthcare providers;
- federal transparency laws, including the federal Physician Payment Sunshine Act that requires disclosure of payments and other transfers of value provided to physicians and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations;
- the ACA and its implementing regulations, which may impact, among other things, reimbursement rates by federal healthcare programs and commercial insurers;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;
- federal government price reporting laws, which require us to calculate and report complex pricing metrics to government programs, where such reported prices may be used in the calculation of reimbursement and/or discounts on our marketed drugs, when and if approved; participation in these programs and compliance with the applicable requirements may subject us to potentially significant discounts on our products, when and if approved, increased infrastructure costs and potentially limit our ability to offer certain marketplace discounts; and
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government, or otherwise restrict certain payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states.

In addition, any sale of our products or product candidates, if commercialized outside of the United States, may also subject us to foreign laws governing prescription drug marketing and fraud and abuse, including laws similar

to the U.S. healthcare laws mentioned above. Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent healthcare reform legislation has strengthened these laws. For example, the ACA, among other things, amends the intent requirements of the federal Anti-Kickback Statute and the criminal statute governing healthcare fraud. A person or entity can now be found guilty of violating the Anti-Kickback Statute and the federal criminal healthcare fraud statute without actual knowledge of the statute or specific intent to violate it. In addition, the ACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act.

If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, imprisonment, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

If the use of our product candidates harms patients, we could be subject to costly and damaging product liability claims.

The use of our product candidates in clinical trials and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. For example, we may be sued if any product candidate we develop allegedly causes injury or is found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability, and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- impairment of our business reputation;
- withdrawal of clinical trial participants;
- initiation of investigations by regulators;
- costs due to related litigation;
- distraction of management’s attention from our primary business;
- substantial monetary awards to trial participants, patients or other claimants;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize our product candidates; and
- decreased demand for our product candidates, if approved for commercial sale.

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop. While we believe that our product liability insurance coverage is sufficient in light of our current clinical programs, the amount of the product liability coverage that we carry varies from time to time, depending on a number of factors, the most significant of which are the nature and scope of the clinical trials in which we are engaged and the number of patients being treated with our product candidates in these trials. This amount may increase or

decrease in the future. We may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. Additionally, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. If and when we obtain marketing approval for product candidates, we intend to expand our insurance coverage to include the commercial sale of our products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business.

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

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment, manufacture and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. Although we believe that our procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses, we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

We rely on our relationship with a professional employer organization for our human relations function and as a co-employer of our personnel, and if that party failed to perform its responsibilities under that relationship, our relations with our employees could be damaged and we could incur liabilities that could have a material adverse effect on our business.

All of our personnel, including our executive officers, are co-employees of AGTC and a professional employer organization, Insperity. Under the terms of our arrangement, Insperity is the formal employer of all of our personnel and is responsible for administering all payroll, including tax withholding, and providing health insurance and other benefits to those individuals. We reimburse Insperity for these costs, and pay Insperity an administrative fee for its services. If Insperity fails to comply with applicable laws or its obligations under this arrangement, our relationship with our employees could be damaged. We could, under certain circumstances, be held liable for a failure by Insperity to appropriately pay, or withhold and remit required taxes from payments to, our employees. In such a case, our potential liability could be significant and could have a material adverse effect on our business.

We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Substantially all of our manufacturing operations and a majority of our research and development operations are conducted from our headquarters located near Gainesville, Florida. Hurricanes or other natural disasters could severely disrupt our operations, damage our research facilities or destroy stored research materials that could be

difficult to replace, and otherwise have a material adverse effect on our business, results of operations, financial condition and prospects. In addition, despite the implementation of security measures, our internal computer systems and those of our current and any future CROs and other contractors and consultants and collaborators are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure or that otherwise disrupted our operations or the operations of our third-party contract manufacturer, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. For example, the loss of clinical trial data from our clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. If our security measures, disaster recovery and business continuity plans are not adequate in the event of such a breach, serious disaster or similar event, we could incur substantial expenses and the further development and commercialization of our product candidates could be delayed, which could have a material adverse effect on our business.

Interruptions in the supply of product or inventory loss may adversely affect our operating results and financial condition.

Our product candidates are manufactured using technically complex processes requiring specialized facilities, highly specific raw materials and other production constraints. The complexity of these processes, as well as strict company and government standards for the manufacture and storage of our products, subjects us to production risks. While product batches released for use in clinical trials or for commercialization undergo sample testing, such testing is subject to human error and some defects may only be identified following product 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. Most of 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 become adversely affected, making them no longer suitable for use. The occurrence or suspected occurrence of production and distribution difficulties can lead to lost inventories, and in some cases product recalls, with consequential reputational damage and the risk of product liability. The investigation and remediation of any identified problems can cause production delays, substantial expense, lost sales and delays of new product launches. Any interruption in the supply of finished products or the loss thereof could hinder our ability to timely distribute our products and satisfy customer demand. Any unforeseen failure in the storage of the product or loss in supply could delay our clinical trials and, if our product candidates are approved, result in a loss of our market share and negatively affect our revenue and operations.

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

Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

Our ability to use our net operating losses may be subject to limitation.

Under Section 382 of the Internal Revenue Code of 1986, as amended, substantial changes in our ownership may limit the amount of net operating loss carryforwards that could be utilized annually in the future to offset our taxable income. Specifically, this limitation may arise in the event of a cumulative change in ownership of our company of more than 50% within a three-year period. Any such annual limitation may significantly reduce the utilization of our net operating loss carryforwards before they expire. We believe that it is likely that transactions that have occurred in the past and other transactions that may occur in the future could trigger an ownership change pursuant to Section 382, which could limit the amount of net operating loss carryforwards that could be utilized annually in the future to offset our taxable income, if any.

Further, net operating losses arising in tax years beginning after December 31, 2017, and before January 1, 2021, generally may be carried back to each of the five tax years preceding the loss and then carried forward indefinitely. To the extent that such losses are carried forward to tax years beginning after December 31, 2020, a taxpayer's ability to utilize such carryforwards is limited to 80% of taxable income. In addition, net operating loss carryforwards arising in tax years ending after December 31, 2020 can be carried forward indefinitely and can be utilized to the extent of 80% of a taxpayer's taxable income in the relevant carryforward tax year; however, carryback of such losses is generally prohibited. Net operating loss carryforwards generated before January 1, 2018 will not be subject to the taxable income limitation and will continue to have a twenty-year carryforward period. Nevertheless, our net operating loss carryforwards and other tax assets could expire before utilization and could be subject to limitations, which could harm our business, revenue and financial results.

Cyber-attacks or other breaches of network or other information technology security could have an adverse effect on our business.

Cyber-attacks or other breaches of network or information technology security may cause equipment failures or disruptions to our operations. While, to date, we have not been subject to cyber-attacks or other cyber incidents which, individually or in the aggregate, have been material to our operations or financial condition, the preventative actions we take to prevent or detect the risk of cyber incidents and protect our information technology and networks may be insufficient to prevent or detect a major cyber-attack in the future. If we fail to prevent the theft of valuable information such as financial data, sensitive information about us, our patients or our intellectual property, or if we fail to protect the privacy of patient and employee confidential data against breaches of network or information technology security, we could incur liability and it would damage our reputation, which could adversely impact the confidence of our partners, investors and employees. Any of these occurrences could result in a material adverse effect on our results of operations and financial condition.

Risks related to our intellectual property

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

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and product candidates.

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. 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. Moreover, in some circumstances, we do not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third

parties. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, if at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in issued patents that protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Moreover, we may be subject to a third-party pre-issuance submission of prior art to the United States Patent and Trademark Office, or become involved in opposition, derivation, reexamination, *inter partes* review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Even if our owned and licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner.

In addition, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. 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.

Third parties may initiate legal proceedings alleging claims of intellectual property infringement, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

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 and *inter partes* reexamination proceedings before the United States Patent and Trademark Office and corresponding foreign patent offices. Numerous United States 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. 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. 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. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of 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 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, methods for manufacture or methods of use, including combination therapy, 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 patent expires. In either case, such a license may not be available on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense 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.

Further, we are aware of third-party patent applications that, if granted with claims as currently drafted, may cover our gene therapy compositions for treating XLRP. These applications include a U.S. patent application with claims that are similar to claims that have been previously allowed in related applications, although the applicant has chosen not to pay the issue fee in these cases. We do not believe that our XLRP product candidate infringes any valid claim in these patent applications. If these patents are issued, however the third party could initiate lawsuits against us for patent infringement and assert that its patents are valid and cover our XLRP product candidate. It could be determined that our XLRP product candidate and/or actions in manufacturing or selling our XLRP product candidate infringe such patents. If these patents were asserted against our XLRP product candidate and we are found to infringe, we could be required to obtain a license from the third party to continue developing and, if approved, marketing our product. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing product. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed the patents and could be forced to indemnify our customers. A finding of infringement could also result in an injunction that prevents us from commercializing our product candidate or forces us to cease some of our business operations.

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

Presently, we have rights to the intellectual property to develop our gene therapy product candidates. Because a key element of our business strategy is to pursue in-licensing and intellectual property acquisitions for additional

product candidates 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 terms that we find acceptable, or 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 that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

For example, we sometimes collaborate with United States and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such right of first negotiation for intellectual property, we may be unable to negotiate a license within the specified time frame or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

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.

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. We are a party to intellectual property license agreements with the University of Florida Research Foundation, an affiliate of the University of Florida, Johns Hopkins University and the UAB Research Foundation, an affiliate of The University of Alabama at Birmingham, each of which is important to our business, and we 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 payment, 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. It is possible that 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 which 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 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 that 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. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

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

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.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property or the patents or other intellectual property of our licensors, which could be expensive, time-consuming and ultimately unsuccessful.

Competitors may infringe our patents or other intellectual property or the patents or other intellectual property of our licensors. In response, we may be required to file infringement claims, which can be expensive and time-consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us, alleging that we infringe their patents. Additionally, if the party against whom we bring a claim of infringement has a relationship with one or more of our collaborators, licensors or other strategic counterparties, our relationship with that counterparty may be harmed. Similarly, because our intellectual property is potentially useful for the treatment of serious diseases, any third-party infringers may be viewed sympathetically by the public and our assertion of an infringement claim against them may hurt our reputation. In addition, in a patent infringement proceeding, a court may decide that a patent of ours or our licensors is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or 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 proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could 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. Our defense of litigation or interference 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.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

Issued patents covering our product candidates or methods of manufacturing could be found invalid or unenforceable if challenged in court.

If we or one of our licensing partners initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, or methods of manufacturing our product candidates, the defendant could counterclaim that the patent covering our product candidate or method is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the United States Patent and Trademark Office, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions. Such proceedings could result in the revocation of or amendment to our patents in such a way that they no longer cover our product candidates or manufacturing methods. 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 or unenforceability, we could lose at least part, and perhaps all, of the patent protection on one or more of our product candidates or methods of manufacturing our products. Such a loss of patent protection could have a material adverse impact on our business.

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 have enacted policies and procedures designed 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 subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patents or other intellectual property. While it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. We could be subject to ownership disputes arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on 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.

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

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and, if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by 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 United States Patent and Trademark Office and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We rely on our outside counsel to pay these fees due to non-U.S. patent agencies. The United States Patent and Trademark Office 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 reputable law firms and other 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, potential competitors might be able to enter the market, which could have a material adverse effect on our business.

Changes in United States patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity. Therefore, obtaining and enforcing biotechnology patents is costly, time-consuming and inherently uncertain.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The United States Patent and Trademark Office has developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

Moreover, recent United States Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the United States Congress, the federal courts, and the United States Patent and Trademark Office, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We have not yet sought FDA approval of names for any of our product candidates and failure to secure such approvals could adversely affect our business.

Any name we propose to use with our product candidates in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA.

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. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. 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. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property 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 prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Risks related to ownership of our common stock

The market price for our common stock has been, and is likely to continue to be, volatile, which could contribute to the loss of your investment.

Fluctuations in the price of our common stock could contribute to the loss of all or part of your investment. Our stock price has been volatile and could be subject to wide fluctuations in response to various factors, many of

which are beyond our control. Since our initial public offering in March 2014 and through August 31, 2020, the price of our common stock on the Nasdaq Global Market has ranged from $2.26 to $34.37. Any of the factors listed below could have a material adverse effect on your investment in our common stock. In such circumstances, the trading price of our common stock may not recover and may experience a further decline.

Factors affecting the trading price of our common stock may include:

- our failure to develop and commercialize our product candidates;
- actual or anticipated fluctuations in our quarterly financial results or the quarterly financial results of companies perceived to be similar to us;
- changes in the market's expectations about our operating results;
- adverse results or delays in our preclinical studies or clinical trials;
- reports of adverse events in other gene therapy products or clinical studies of such products;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- any delay in filing an IND or BLA for any of our product candidates and any adverse development or perceived adverse development with respect to the FDA's review of that IND or BLA;
- adverse regulatory decisions, including failure to receive regulatory approval for any of our product candidates;
- success of competitive products;
- adverse developments concerning our collaborations and our manufacturers;
- inability to obtain adequate product supply for any product candidate for clinical trials or commercial sale or inability to do so at acceptable prices;
- the termination of a collaboration or the inability to establish additional collaborations;
- unanticipated serious safety concerns related to the use of any of our product candidates;
- our ability to effectively manage our growth;
- the size and growth, if any, of the orphan ophthalmology and other targeted markets;
- our operating results failing to meet the expectation of securities analysts or investors in a particular period or failure of securities analysts to publish reports about us or our business;
- changes in financial estimates and recommendations by securities analysts concerning our company, the gene therapy market, or the biotechnology and pharmaceutical industries in general;
- operating and stock price performance of other companies that investors deem comparable to us;
- overall performance of the equity markets;
- announcements by us or our competitors of acquisitions, new product candidates or programs, significant contracts, commercial relationships or capital commitments;
- our ability to successfully market our product candidates;
- changes in laws and regulations affecting our business, including but not limited to clinical trial requirements for approvals;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our product candidates and gene therapy platform;
- commencement of, or involvement in, litigation involving our company, our general industry, or both;

- changes in our capital structure, such as future issuances of securities or the incurrence of additional debt;
- the volume of shares of our common stock available for public sale;
- additions or departures of key scientific or management personnel;
- any major change in our board or management;
- changes in accounting practices;
- ineffectiveness of our internal control over financial reporting;
- sales of substantial amounts of common stock by our directors, executive officers or significant stockholders or the perception that such sales could occur; and
- general economic and political conditions, such as recessions, interest rate, fuel price and international currency fluctuations, global health pandemics, such as COVID-19, and acts of war or terrorism.

Broad market and industry factors may materially harm the market price of our common stock irrespective of our operating performance. The stock market in general, and the Nasdaq Global Market and the market for biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of the particular companies affected. The trading prices and valuations of these stocks, and of ours, may not be predictable. A loss of investor confidence in the market for technology or software stocks or the stocks of other companies that investors perceive to be similar to us, the opportunities in the digital simulation market or the stock market in general could each depress our stock price regardless of our business, prospects, financial conditions or results of operations.

If securities analysts do not publish research or reports about our business or if they downgrade our stock, the price of our common stock could decline.

The trading market for our common stock relies in part on the research and reports that industry or financial analysts publish about us, our business, our markets and our competitors. We do not control these analysts. If securities analysts do not continue to cover our common stock, the lack of research coverage may adversely affect the market price of our common stock. Furthermore, if one or more of the analysts who do cover us downgrade our stock or if those analysts issue other unfavorable commentary about us or our business, our stock price would likely decline. If one or more of these analysts cease coverage of us or fails to regularly publish reports on us, we could lose visibility in the market and interest in our stock could decrease, which in turn could cause our stock price or trading volume to decline and may also impair our ability to expand our business with existing customers and attract new customers.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that significant additional capital may be needed in the future to continue our planned operations, including conducting clinical trials, commercialization efforts, expanded research and development activities, potential acquisitions, in-licenses, or collaborations and costs associated with operating a public company. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions, including sales made at-the-market under our existing sales agreement with Cantor Fitzgerald & Co., at prices and in a manner that we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, and new investors could gain rights, preferences and privileges senior to the holders of our common stock.

We do not currently intend to pay dividends on our common stock and, consequently, your ability to achieve a return on your investment will depend on the appreciation in the price of our common stock.

We have never declared or paid any cash dividends on our common stock. We currently intend to retain any future earnings to fund our future growth and do not expect to declare or pay any dividend on shares of our common stock in the foreseeable future. As a result, you may only receive a return on your investment in our common stock if the market price of our common stock appreciates and you sell your shares at a price above your cost.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biotechnology companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and divert management's attention and resources, which could harm our business.

Anti-takeover provisions in our amended and restated certificate of incorporation and amended and restated bylaws, as well as provisions in Delaware law, might discourage, delay or prevent a change of control of our company or changes in our management and, therefore, depress the trading price of our common stock.

Our certificate of incorporation, bylaws and Delaware law contain provisions that could have the effect of rendering more difficult or discouraging an acquisition deemed undesirable by our board of directors, even if doing so would benefit our stockholders or remove our current management. Our corporate governance documents include provisions:

- providing for three classes of directors with the term of office of one class expiring each year, commonly referred to as a staggered board;
- authorizing blank check preferred stock, which could be issued with voting, liquidation, dividend and other rights superior to our common stock;
- limiting the liability of, and providing indemnification to, our directors and officers;
- eliminating the ability of our stockholders to call and bring business before special meetings and to take action by written consent in lieu of a meeting;
- requiring advance notice of stockholder proposals for business to be conducted at meetings of our stockholders and for nominations of candidates for election to our board of directors;
- controlling the procedures for the conduct and scheduling of board and stockholder meetings;
- limiting the determination of the number of directors on our board and the filling of vacancies or newly created seats on the board to our board of directors then in office; and
- providing that directors may be removed by stockholders only for cause.

These provisions, alone or together, could delay hostile takeovers and changes in control or changes in our management.

As a Delaware corporation, we are also subject to provisions of Delaware law, including Section 203 of the Delaware General Corporation Law, which prohibits a publicly-held Delaware corporation from engaging in a business combination with an interested stockholder, generally a person which together with its affiliates owns, or within the last three years has owned, 15% of our voting stock, for a period of three years after the date of the transaction in which the person became an interested stockholder, unless the business combination is approved in a prescribed manner.

The existence of the foregoing provisions and anti-takeover measures could limit the price that investors might be willing to pay in the future for shares of our common stock. They could also deter potential acquirers of our company, thereby reducing the likelihood that you could receive a premium for your common stock in an acquisition.

ITEM 1B. UNRESOLVED STAFF COMMENTS

We are a smaller reporting company, as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and are not required to provide this information.

ITEM 2. PROPERTIES

Alachua, Florida

Our corporate headquarters is located in Alachua, Florida where we lease approximately 21,500 square feet of office and laboratory space under a lease arrangement that expires in December 2027. We have options to extend the term of the Alachua lease for three additional five-year periods.

Cambridge, Massachusetts

We lease approximately 8,000 square feet of office and laboratory space in Cambridge, Massachusetts under a lease arrangement that expires in February 2025. We have an option to extend the Cambridge lease for one additional three-year term. The Cambridge facility primarily focuses on business development, pharmacology and basic research and development.

ITEM 3. LEGAL PROCEEDINGS

We are not a party to any pending legal proceedings. However, due to the nature of our business, we may be subject to lawsuits or other claims arising at any particular time in the ordinary course of our business, and we expect that this situation will continue to be the case in the future.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

Market Information

Our common stock has been listed on the Nasdaq Global Market under the symbol "AGTC" since March 27, 2014. Prior to that date, there was no public market for our common stock.

As of September 2, 2020, a total of 25,857,883 shares of our common stock were outstanding and we had 30 holders of record of our common stock.

Dividend Policy

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and future earnings, if any, to finance the growth and development of our business. We do not expect to pay any cash dividends on our common stock in the foreseeable future. Moreover, as discussed in Note 8 to our financial statements in this Annual Report on Form 10-K, our ability to declare or pay any cash dividends on our common stock is restricted by the agreement governing our outstanding debt.

Securities Authorized for Issuance Under Equity Compensation Plans

For information regarding securities authorized for issuance under our equity compensation plans, see Part III, Item 12, "Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters."

ITEM 6. SELECTED FINANCIAL DATA

We are a smaller reporting company, as defined by Rule 12b-2 of the Exchange Act, and are not required to provide this information.

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with the financial statements and notes included in Part IV, Item 15 of this Annual Report on Form 10-K. In addition to historical financial information, the following discussion contains forward-looking statements that reflect our plans, estimates and beliefs. Our actual results could differ materially from those discussed in the forward-looking statements. Factors that could cause or contribute to these differences include those discussed below and elsewhere in this Annual Report, including, but not limited to, those set forth in "Cautionary Note Regarding Forward-Looking Statements" and "Risk Factors."

Overview

We are a clinical-stage biotechnology company that uses a proprietary gene therapy platform to develop transformational genetic therapies for patients suffering from rare and debilitating diseases. Our initial focus is in the field of ophthalmology, where we have active clinical programs in X-linked retinitis pigmentosa ("XLRP"), achromatopsia ("ACHM"), and optogenetics as well as preclinical programs in, Stargardt disease and age-related macular degeneration ("AMD"). In addition to ophthalmology, we have initiated one preclinical program in otology and three preclinical programs targeting central nervous system disorders ("CNS"), including adrenoleukodystrophy ("ALD"), frontotemporal dementia ("FTD") and amyotrophic lateral sclerosis ("ALS"). Our optogenetics program is being developed in collaboration with Bionic Sight, LLC ("Bionic Sight") and our otology program is being developed in collaboration with Otonomy, Inc. ("Otonomy"). With a number of

important clinical milestones on the horizon, we believe that we are well positioned to advance multiple programs toward pivotal studies. In addition to our product pipeline, we have also developed broad technological and manufacturing capabilities utilizing both our internal scientific resources and collaborations with others, such as our efforts with Synpromics Limited ("Synpromics"), which was acquired by AskBio and provides expertise in synthetic promoter development and optimization, and the University of Florida, which provides us with expertise in vector design and access to novel capsids.

Since our inception in 1999, we have devoted substantially all of our resources to development efforts relating to our proof-of-concept programs in ophthalmology, otology, CNS, and alpha-1 antitrypsin deficiency, or AAT deficiency, an inherited orphan lung disease, including activities to manufacture product in compliance with good manufacturing practices, preparing to conduct and conducting clinical trials of our product candidates, providing general and administrative support for these operations and protecting our intellectual property. We do not have any products approved for sale and have not generated any revenue from product sales. We have funded our operations to date primarily through public offering of our common stock, private placement of preferred stock, collateralized borrowing and collaborations. We have also been the recipient, either independently or with our collaborators, of grant funding administered through federal, state, and local governments and agencies, including the United States Food and Drug Administration, or FDA, and by patient advocacy groups such as The Foundation Fighting Blindness, or FFB, and the Alpha-1 Foundation.

We have incurred losses from operations in each year since inception, except for fiscal year 2017, in which we reported net income of $0.4 million due, in part, to the amortization associated with a collaboration agreement with Biogen that was terminated in March 2019. For the years ended June 30, 2020 and 2019, we reported net losses of $45.9 million and $2.0 million, respectively. Substantially all our net losses resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We expect to continue to incur significant operating expenses for at least the next several years and anticipate that such expenses will increase substantially in connection with our ongoing activities, as we:

- continue to conduct preclinical studies and clinical trials for our XLRP and ACHM product candidates and preclinical studies for our other ophthalmology, otology and CNS product candidates;
- continue our research and development efforts, including exploration through early preclinical studies of potential applications of our gene therapy platform in:
  - orphan ophthalmology indications;
  - non-orphan ophthalmology indications, including wet AMD and other retinal diseases; and
  - other inherited diseases, such as otology and CNS indications.
- manufacture clinical trial materials and develop larger-scale manufacturing capabilities;
- seek regulatory approval for our product candidates;
- further develop our gene therapy platform;
- add personnel to support our scientific, collaboration, product development and commercialization efforts; and
- continue to operate as a public company.

As of June 30, 2020, we had cash and cash equivalents and liquid investments totaling $80.5 million. We do not expect to generate revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years and which we believe is subject to significant uncertainty. We believe that our existing cash and cash equivalents and investments as of June 30, 2020 will be sufficient to allow us to generate data from our ongoing clinical programs, initiate a Phase 2/3 trial for XLRP and fund currently planned research and discovery programs into

the fourth quarter of calendar year 2021. In order to complete the XLRP Phase 2/3 trial, obtain regulatory approval for our lead product candidates and build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize our lead product candidates, if approved, we will require substantial additional funding. Also, our current operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements, acquisitions or other business development activities, or a combination of these approaches. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements as and when needed would have a negative impact on our financial condition and our ability to develop our product candidates.

Recent Developments

In September 2019, we released positive interim six-month data from our ongoing Phase 1/2 clinical program in XLRP for an initial set of patients dosed peripherally. The data showed that for nine patients dosed peripherally in dose groups 1-3 that their visual sensitivity, as measured by perimetry, was stable over the six months of follow-up, especially for the high dose group patients. We also released three-month data on eight patients dosed centrally showing improved retinal sensitivity.

In January 2020, we released a second set of positive interim six-month data from our ongoing Phase 1/2 clinical program in XLRP. The data showed that of the nine patients dosed centrally – two patients in the 2nd dose group and seven patients in the 4th dose group – improved retinal sensitivity (as measured by microperimetry) was observed in 50% (4/8) of evaluable patients through six months and, importantly, these data were consistent with the previous data reported at three months. In addition to retinal sensitivity, we have observed stable and, in some cases, improving trends in visual acuity in 77% (7/9) of patients.

In both data releases, we reported that the study drug was safe and well-tolerated. As of June 30, 2020, we have completed targeted enrollment of 28 patients and have not observed dose limiting toxicity. We will continue to monitor patients for safety and efficacy and will release additional data on two new higher dose groups – as well as 12-month data on the first four dose groups – in the second half of calendar year 2020.

On January 23, 2020, we announced encouraging interim six-month data from the dose-escalation cohorts of our ongoing Phase 1/2 clinical programs in patients with achromatopsia due to mutation in the CNGB3 or CNGA3 genes. The studies' primary endpoint is safety, with various secondary efficacy endpoints, including light discomfort. The study agent was found to have a favorable safety profile with no serious study drug-related adverse events. On the secondary efficacy endpoints, we saw preliminary signs of biologic activity based on light discomfort testing at Month 3. Light discomfort is a clinically relevant symptom for these patients. On the additional secondary efficacy endpoints, we did not see consistent improvements, but we will continue to monitor these patients over time at higher doses and in younger patient groups. As of June 30, 2020, we have dosed 22 adult patients and 4 pediatric patients in the ACHMB3 trial; adult enrollment is now complete and approximately 5 more pediatric patients will be enrolled. As of June 30, 2020, we have dosed 15 adult patients and 4 pediatric patients in the ACHMA3 trial; adult enrollment is now complete and approximately 5 more pediatric patients will be enrolled.

In February 2020, we announced completion of enrollment in the two highest dose groups of our Phase 1/2 clinical trial, evaluating the safety and efficacy of sub-retinal injection of our XLRP product candidate, AGTC-501, for the treatment of XLRP caused by mutations in the RPGR gene.

During February 2020, we closed an underwritten public offering of an aggregate of approximately 7.5 million shares of our common stock at $5.00 per share, generating total gross proceeds of $37.4 million, before deducting underwriting discounts, commissions and other offering expenses payable by us.

In June 2020, we announced the launch of a nationwide mobile vision testing program to conduct follow-up assessments during the COVID-19 pandemic for patients enrolled in our ongoing clinical trials in XLRP and achromatopsia (ACHM CNGB3 and ACHM CNGA3). Our mobile vision testing program was launched in partnership with 2020 On-Site and has been customized to include the necessary testing equipment needed, as well as appropriate safety protocols, to conduct the key follow-up assessments required to determine the potential safety and efficacy of our investigational products.

In June 2020, we provided an update on the continued productivity and quality improvements made in our proprietary manufacturing platform that is currently being used to create clinical trial material for our planned pivotal XLRP clinical trials. We are now achieving finished product specifications that demonstrate nearly 90% full capsids with extremely low residuals, many of which fall below the level of detection, resulting in purity levels exceeding 97%. These outcomes, in addition to yields that are more than 10-fold higher than what we achieved in our Phase 1/2 manufacturing campaigns, put us at commercial scale for our ophthalmology programs.

In June 2020, we received comprehensive feedback from the FDA on the end of Phase 2 package that we submitted earlier in the year and we will use such feedback to design our XLRP clinical trials going forward.

On June 30, 2020, we entered into a loan agreement for a term loan in the aggregate principal amount of up to $25.0 million. On that date, we received net loan proceeds of $9.9 million, before consideration of any related debt financing fees. The loan agreement is further discussed at Note 8 to our financial statements in this Annual Report on Form 10-K.

In July 2020, we announced our updated development plans for the XLRP clinical program, including additional patient dosing in our current Phase 1/2 trial to collect more functional data and our planned start of a Phase 2/3 trial during the first quarter of calendar year 2021.

Strategic Collaborations

Bionic Sight

During February 2017, we entered into a strategic research and development collaboration agreement with Bionic Sight to develop therapies for patients with visual deficits and blindness due to retinal disease. Through the AGTC-Bionic Sight collaboration, the companies seek to develop a new optogenetic therapy that leverages AGTC's deep experience in gene therapy and ophthalmology and Bionic Sight's innovative neuro-prosthetic device and algorithm for retinal coding. The collaboration agreement grants to us, subject to achievement by Bionic Sight of certain development milestones, an option to exclusively negotiate for a limited period of time to acquire (i) a majority equity interest in Bionic Sight, (ii) the Bionic Sight assets to which the collaboration agreement relates, or (iii) an exclusive license with respect to the product to which the collaboration agreement relates.

Otonomy

In October 2019, we entered into a strategic collaboration agreement with Otonomy to co-develop and co-commercialize an AAV-based gene therapy to restore hearing in patients with sensorineural hearing loss caused by a mutation in the gap junction protein beta 2 gene (GJB2) – the most common cause of congenital hearing loss. Mutations in GJB2 account for approximately 30% of all genetic hearing loss cases. Patients with this mutation can have severe-to-profound deafness in both ears that is identified in screening tests routinely performed in newborns. Under the collaboration agreement, the parties began equally sharing the program costs and proceeds, if any, in January 2020 and can include additional genetic hearing loss targets in the future.

Additional information regarding the Bionic Sight and Otonomy collaborative agreements can be found in Note 9 to our financial statements in this Annual Report on Form 10-K.

Financial operations overview

Revenue

We primarily generate revenue through collaboration agreements, sponsored research arrangements with nonprofit organizations for the development and commercialization of product candidates and from federal research and development grant programs. In December 2019, Bionic Sight notified us that it had achieved an IND Trigger under our collaboration agreement. As a result of such IND Trigger achievement and in connection with certain in-kind contribution payments previously made by us, we recognized $2.2 million of collaboration revenue during the year ended June 30, 2020. No additional collaboration revenue related to the Bionic Sight collaboration agreement will be recognized. Additional information regarding the Bionic Sight collaborative agreement can be found in Note 9 to our financial statements in this Annual Report on Form 10-K. In the future, we may generate revenue from a combination of: product sales (if any products are approved), license fees, milestone payments, development services, research and development grants, and from collaboration and royalty payments for the sales of products developed under licenses of our intellectual property.

We expect that any revenue we generate will fluctuate from quarter to quarter as a result of the timing and amount of license fees, research and development programs, manufacturing efforts and reimbursements, collaboration milestone payments, and the sale of our products, to the extent that any are approved and successfully commercialized. We do not expect to generate revenue from product sales for the foreseeable future, if at all. If we or our collaborators fail to complete the development of our product candidates in a timely manner or obtain regulatory approval for them, our ability to generate future revenue, our results of operations and financial position would be materially adversely affected.

Research and development expenses

Research and development expenses consist primarily of costs incurred for the development of our product candidates, which include:

- employee-related expenses, including salaries, benefits, travel and share-based compensation expense;
- expenses incurred under agreements with academic research centers, contract research organizations, or CROs, and investigative sites that conduct our clinical trials;
- license and sublicense fees and collaboration expenses;
- the cost of acquiring, developing and manufacturing clinical trial materials; and
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies.

Research and development costs are expensed as incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and our clinical sites.

We cannot determine with certainty the duration and completion costs of the current or future clinical trials of our product candidates or if, when, or to what extent we will generate revenue from the commercialization and sale of any of our product candidates that obtain regulatory approval. We may never succeed in achieving regulatory approval for any of our product candidates. The duration, costs and timing of clinical trials and development of our product candidates will depend on a variety of factors, including:

- the scope, rate of progress and expense of our ongoing clinical trials, as well as any additional clinical trials that we are required to, or decide to, initiate and other research and development activities;
- the timing and level of activity as determined by us or jointly with our partners;
- the level of funding received from our partners;

- whether or not we elect to cost share with our collaborators;
- the countries in which trials are conducted;
- future clinical trial results;
- uncertainties in clinical trial enrollment rates or drop-out or discontinuation rates of patients;
- potential additional safety monitoring or other studies requested by regulatory agencies or elected as best practice by us;
- increased cost and delay associated with manufacturing or testing issues, including ongoing quality assurance, qualifying new vendors and developing in-house capabilities;
- significant and changing government regulation; and
- the timing and receipt of any regulatory approvals.

A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the FDA, or another regulatory authority were to require us to conduct clinical trials beyond those that we currently anticipate will be required for the completion of clinical development of a product candidate or if we experience significant delays in enrollment in or execution of any of our clinical trials, which could be adversely impacted by the COVID-19 pandemic, we could be required to expend significant additional financial resources and time on the completion of clinical development.

From inception and through June 30, 2020, we have incurred approximately $238.2 million in research and development expenses. We expect our research and development expenses to increase for the foreseeable future as we continue the development of our product candidates and explore potential applications of our gene therapy platform in other indications.

General and administrative expenses

General and administrative expenses consist primarily of salaries and related costs for personnel, including share-based compensation and travel expenses for our employees in executive, operational, legal, business development, finance and human resource functions. Other general and administrative expenses include costs to support employee training and development, board of directors' costs, depreciation, insurance expenses, facility-related costs not otherwise included in research and development expense, professional fees for legal services, including patent-related expenses, and accounting, investor relations, corporate communications and information technology services. We anticipate that our general and administrative expenses will continue to increase in the future as we hire additional employees to support our continued research and development efforts, collaboration arrangements, and the potential commercialization of our product candidates. Additionally, if and when we believe that a regulatory approval of the first product candidate appears likely, we anticipate an increase in payroll and related expenses as a result of our preparation for commercial operations, especially as it relates to the sales and marketing of our product candidates.

Other income (expense), net

Other income (expense), net primarily consists of interest earned on cash and cash equivalents and our held-to-maturity investments.

Provision for income taxes

Income tax expense for the years ended June 30, 2020 and 2019 was $83,000 and $76,000, respectively. During both years, income tax expense was primarily driven by interest expense related to the Company's uncertain tax

positions. The uncertain tax position liability as of June 30, 2020 was $2.1 million. Additional information regarding our income taxes can be found in Note 11 to our financial statements in this Annual Report on Form 10-K.

Critical accounting policies and estimates

Management's Discussion and Analysis of Financial Condition and Results of Operations included in this Annual Report on Form 10-K is based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles ("U.S. GAAP"). The preparation of those financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates, judgments and methodologies, including those related to accrued expenses and share-based compensation. We base our estimates on historical experience, current conditions, known trends and events, and various other factors that are believed 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 our estimates under different assumptions or conditions. Moreover, we may need to change the assumptions underlying our estimates due to risks and uncertainties related to the COVID-19 pandemic or otherwise and those changes could have a material adverse effect on our statements of operations, liquidity and financial condition. While our significant accounting policies are described in Note 2 to our financial statements in this Annual Report on Form 10-K, we believe that the following accounting policies are most critical to the preparation of our financial statements.

Revenue recognition

We generate revenue primarily through: (i) collaboration agreements; (ii) sponsored research arrangements with nonprofit organizations for the development and commercialization of product candidates; and (iii) federal research and development grant programs. We recognize revenue when our customer obtains control of promised goods or services, in an amount that reflects the consideration that we expect to receive in exchange for those goods or services. To determine the appropriate amount of revenue to be recognized for arrangements determined to be within the scope of Accounting Standards Codification Topic 606, *Revenue from Contracts with Customers*, we perform the following five steps: (i) identification of the contract; (ii) determination of whether the promised goods or services are performance obligations; (iii) measurement of the transaction price, including any constraints on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) we satisfy each performance obligation. We only apply the five-step model to contracts if it is probable that we will collect consideration that we are entitled to in exchange for the goods or services we transfer to the customer.

Performance obligations are promises to transfer distinct goods or services to a customer. Promised goods or services are considered distinct when (i) the customer can benefit from the good or service on its own or together with other readily available resources and (ii) the promised good or service is separately identifiable from other promises in the contract. When assessing whether promised goods or services are distinct, we consider factors such as the stage of development of the underlying intellectual property, the capabilities of a customer to develop the intellectual property on its own or whether the required expertise is readily available.

We estimate the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include both fixed consideration and variable consideration. At the inception of an arrangement that includes variable consideration and at the end of each reporting period, we evaluate the amount of potential customer payments and the likelihood that such payments will be received. We utilize either the most likely amount method or the expected amount method to estimate the amount to be received based on which method better predicts the amount expected to be received. If it is probable that a significant revenue reversal would not occur, the variable consideration is included in the transaction price. We will assess our revenue generating arrangements to determine whether a significant

financing component exists and conclude that a significant financing component does not exist in an arrangement if the: (a) promised consideration approximates the cash selling price of the promised goods and services or any significant difference is due to factors other than financing; and (b) timing of payment approximates the transfer of goods and services and performance is over a relatively short period of time within the context of the entire term of the contract.

Our contracts often include development and regulatory milestone payments. At contract inception, we evaluate whether any such milestones are considered probable of being reached and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within our control or the customer's control, such as regulatory approvals, are not included in the transaction price. At the end of each subsequent reporting period, we reevaluate the probability of achievement of such development milestones and any related constraint and, if necessary, adjust our estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenue and earnings in the period of adjustment.

For arrangements that may include sales-based royalties, including milestone payments based on the level of sales, and the license is deemed to be the predominant item to which the royalties relate, we recognize revenue at the later of (i) when the related sale occurs or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any royalty revenue resulting from any of our collaboration arrangements.

We allocate the transaction price based on the estimated stand-alone selling price of the underlying performance obligation or, in the case of certain variable consideration, to one or more performance obligations. We use assumptions that require judgment to determine the stand-alone selling price for each performance obligation identified in a contract. We utilize key assumptions to determine the stand-alone selling price, which may include other comparable transactions, pricing considered in negotiating the transaction and the estimated costs to complete the related performance obligation. Certain variable consideration is allocated specifically to one or more performance obligation in a contract when the terms of the variable consideration relate to the satisfaction of a performance obligation and the resulting amounts allocated to each performance obligation are consistent with the amounts we would expect to receive for each performance obligation.

For performance obligations consisting of licenses and other promises, we utilize judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue from upfront fees. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure of performance and related revenue recognition. If the license to our intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, we will recognize revenue from upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license.

We receive payments from our customers based on billing terms established in each contract. Such billings generally have 30-day payment terms. Upfront payments and fees are recorded as deferred revenue upon receipt or when due until we perform our obligations under those arrangements. Amounts are recorded as accounts receivable when the right to consideration is unconditional.

Research and development expenses

Research and development expenses include costs incurred in identifying, developing and testing product candidates and generally comprise compensation and related benefits and non-cash share-based compensation to research-related employees; laboratory costs; animal and laboratory maintenance and supplies; rent; utilities; clinical and pre-clinical expenses; and payments for sponsored research, scientific and regulatory consulting fees and testing.

As part of the process of preparing our financial statements, we estimate our accrued expenses. This process involves reviewing quotations and contracts, identifying services that have been performed on our behalf and estimating the level of services performed and the associated cost incurred for services for which we have not yet been invoiced or otherwise notified of the actual cost. The majority of our service providers invoice monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued expenses at the end of each reporting period based on the facts and circumstances known to us at that time. The significant estimates in our accrued research and development expenses relate to expenses incurred with respect to academic research centers, contract research organizations, and other vendors in connection with research and development activities for which we have not yet been invoiced.

There are instances where our service providers require advance payments at the inception of a contract and other circumstances where our payments to a vendor will exceed the level of services provided, in both cases resulting in a prepayment of research and development expenses. Such prepayments are charged to research and development expense as and when the service is provided or when a specific milestone outlined in the contract is reached.

Share-based compensation

We account for share-based awards issued to employees in accordance with Accounting Standards Codification ("ASC") Topic 718, *Compensation—Stock Compensation,* and generally recognize share-based compensation expense on a straight-line basis over the period during which an employee is required to provide service in exchange for the award. In addition, we award stock options and restricted shares of common stock to nonemployees in exchange for consulting services. Prior to July 1, 2019, we accounted for these awards in accordance with the provisions of ASC Subtopic 505-50, *Equity-Based Payments to Non-employees* ("ASC 505-50"). Under ASC 505-50, share-based awards to nonemployees were subject to periodic fair value re-measurement over their vesting terms. As discussed in Note 2 to our financial statements in this Annual Report on Form 10-K, we adopted ASU No. 2018-07, *Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting*, on July 1, 2019. As a result, our accounting for nonemployee awards is now generally consistent with that of employee awards. Beginning on July 1, 2019, the measurement date for nonemployee awards is the date of grant without any subsequent changes in the fair value of the award. Share-based compensation costs for nonemployees are generally recognized as expense as services are provided to us over the related service period.

For purposes of calculating share-based compensation expense, we estimate the fair value of stock options using a Black-Scholes option-pricing model. The determination of the fair value of a share-based compensation award utilizing the Black-Scholes model is affected by our stock price and a number of assumptions, including the expected volatility of our stock, the expected life of the stock option, the risk-free interest rate and expected dividends. Additionally, we use a Monte Carlo simulation model to determine the fair value of restricted stock units for purposes of calculating share-based compensation expense. The Monte Carlo simulation model incorporates the probability of satisfying a market condition and uses transaction details such as our stock price, contractual terms, maturity and risk-free rates, as well as volatility.

If factors change and we employ different assumptions, share-based compensation expense may differ significantly from what we have recorded in the past. If there is a difference between the assumptions used in determining share-based compensation expense and the actual factors that become known over time, specifically with respect to anticipated forfeitures, we may change the input factors used in determining share-based compensation costs for future awards. These changes, if any, may materially impact our results of operations in the period that such changes are made.

Income taxes

We use the asset and liability method to account for income taxes. Under this method, deferred tax assets and liabilities are recognized for the estimated future tax consequences attributable to differences between the

financial statement carrying amounts of existing assets and liabilities and their respective income tax bases. Deferred tax assets and liabilities are measured using enacted rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled.

As required by U.S. GAAP, we recognize the financial statement benefit of a tax position only after determining that the relevant tax authority would more likely than not sustain the position following an audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements is the largest benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the relevant tax authority. Interest and penalties related to uncertain tax positions are reflected in the provision for income taxes. The Company is subject to examination of its income tax returns in the federal and state tax jurisdictions in which it operates for the tax years ended June 30, 2016 through June 30, 2020.

On March 27, 2020, the Coronavirus Aid, Relief, and Economic Security ("CARES") Act was signed into law. The CARES Act contains several significant provisions that affect corporations, including, among others, provisions addressing the use of net operating losses, interest deduction limitations and employer payroll tax payments. We do not believe that the CARES Act will have a material impact on the Company; however, we will continue to monitor ongoing developments, new regulations and interpretive guidance regarding such legislation and evaluate any potential impact on the Company's overall business and tax position.

Recent Accounting Pronouncements

See Note 2 to our financial statements in this Annual Report on Form 10-K for a description of recent accounting pronouncements applicable to our business, including a new lease standard that was adopted by the Company on July 1, 2019.

Results of operations

Comparison of the years ended June 30, 2020 and 2019

Revenue

The table below summarizes our revenue for the years indicated.

In thousands	Year Ended June 30, 2020	2019	Increase (Decrease)	% Increase (Decrease)
Collaboration revenue:				
Licenses and related services	$ —	$27,000	$(27,000)	nm%
Development services	—	2,736	(2,736)	nm%
Non-cash consideration	2,197	—	2,197	nm%
Milestone revenue	100	11,392	(11,292)	(99)%
Total collaboration revenue	2,297	41,128	(38,831)	(94)%
Grant revenue	156	564	(408)	(72)%
Total revenue	$2,453	$41,692	$(39,239)	(94)%

Collaboration revenue for the year ended June 30, 2019 related to a collaboration agreement with Biogen that was terminated effective March 8, 2019. As a result, we recognized collaboration revenue for the remaining deferred revenue balance as of the termination date. Thereafter, no additional collaboration revenue was recognized. Milestone revenue during the year ended June 30, 2019 was primarily attributable to (i) $8.3 million of revenue related to the receipt of a $10.0 million milestone payment from Biogen and (ii) $2.4 million of revenue resulting from the termination of the Biogen collaboration agreement.

In December 2019, Bionic Sight met a milestone related to clearance of filing of an Investigational New Drug application under its collaboration agreement with us and, as a result, we recognized $2.2 million of non-cash

collaboration revenue during the year ended June 30, 2020 in connection with in-kind contributions made since inception of the Bionic Sight collaboration agreement.

Additional information regarding the Biogen and Bionic Sight collaborative agreements can be found in Note 9 to our financial statements in this Annual Report on Form 10-K.

Research and development expenses

The table below summarizes our research and development expenses by product candidate or program for the years indicated.

In thousands	Year Ended June 30, 2020	2019	Increase (Decrease)	% Increase (Decrease)
External research and development expenses:				
ACHM	$ 5,956	$ 4,729	$ 1,227	26%
XLRS	706	1,197	(491)	(41)%
XLRP	6,492	4,539	1,953	43%
Research and discovery programs	1,781	3,363	(1,582)	(47)%
Total external research and development expenses	14,935	13,828	1,107	8%
Internal research and development expenses:				
Employee-related costs	12,466	10,908	1,558	14%
Share-based compensation	1,451	1,895	(444)	(23)%
Other	6,926	6,552	374	6%
Total internal research and development expenses	20,843	19,355	1,488	8%
Total research and development expenses	$35,778	$33,183	$ 2,595	8%

Research and development expenses for the years ended June 30, 2020 and 2019 were $35.8 million and $33.2 million, respectively, an increase of $2.6 million, or 8%. Such increase was primarily attributable to:

- $2.0 million of increased external spending related to XLRP due to our planned manufacturing, clinical site preparation and other activities related to our proposed Phase 2/3 clinical trial that will enable us to initiate our studies as quickly as possible, partially offset by decreased sublicense expenses associated with receiving a $10.0 million XLRP milestone payment from Biogen in September 2018;
- $1.6 million of increased employee-related costs, primarily associated with the hiring of new employees to support clinical trial execution and research and development activities; and
- $1.2 million of increased external spending related to ACHM, primarily due to an increase in patient enrollment, new site activations and deployment of our mobile vision center.

Such increases were partially offset by:

- $1.6 million of decreased external research and discovery spending, primarily due to decreased otology and pre-clinical ophthalmology activities;
- $0.5 million of decreased external XLRS expenses, primarily due to reaching full enrollment on Phase 1/2 clinical trials and the decision to stop further development of our XLRS product candidate; and
- $0.4 million of decreased share-based compensation expense, primarily due to lower fair value of awards granted during the year ended June 30, 2020 compared to the prior year.

General and administrative expenses

The table below summarizes our general and administrative and other expenses for the years indicated.

In thousands	Year Ended June 30, 2020	2019	Increase (Decrease)	% Increase (Decrease)
Employee-related costs	$ 5,746	$ 4,773	$ 973	20%
Share-based compensation	1,547	2,134	(587)	(28)%
Legal and professional fees	468	774	(306)	(40)%
Other	5,856	5,178	678	13%
Total general and administrative and other expenses	$13,617	$12,859	$ 758	6%

General and administrative and other expenses for the years ended June 30, 2020 and 2019 were $13.6 million and $12.9 million, respectively, an increase of $0.8 million, or 6%. Such increase was primarily driven by (i) higher employee-related costs of $1.0 million that was largely due to the hiring of new employees and (ii) increased other general and administrative expenses of $0.7 million associated with normal business operations. These items were partially offset by decreased share-based compensation expense, primarily due to lower fair value of awards granted during the year ended June 30, 2020 compared to the prior year, and decreased legal and professional fees.

Liquidity and capital resources

We have incurred cumulative losses and negative cash flows from operations since our inception in 1999 and, as of June 30, 2020, we had an accumulated deficit of $181.4 million. It will be several years, if ever, before we have a product candidate ready for commercialization. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we anticipate that we will require additional capital to fund our operations, which we may raise through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements. During the year ended June 30, 2020, the Company received (i) $34.8 million of proceeds from the issuance of its common stock, net of issuance costs, and (ii) $9.9 million of loan proceeds, net of debt discounts. Those transactions are further discussed in Note 1 to our financial statements in this Annual Report on Form 10-K.

The Company is closely monitoring ongoing developments in connection with the COVID-19 pandemic, which may negatively impact its projected cash position and access to capital. The Company will continue to assess its cash position and, if circumstances warrant, make appropriate adjustments to its operating plan.

As of June 30, 2020, we had cash and cash equivalents and liquid investments totaling $80.5 million, which we believe will be sufficient to allow us to generate data from our ongoing clinical programs, initiate the XLRP Phase 2/3 clinical trial and fund currently planned research and discovery programs into the fourth quarter of calendar year 2021.

Cash in excess of immediate requirements is invested in accordance with our investment policy, which primarily seeks to maintain adequate liquidity and preserve capital by generally limiting investments to certificates of deposit and investment-grade debt securities that mature within twelve months. As of June 30, 2020, our cash and cash equivalents were held in bank accounts and money market funds, while our investments consisted of U.S. Treasury securities, none of which mature more than twelve months after the balance sheet date, consistent with our investment policy that seeks to maintain adequate liquidity and preserve capital.

Cash flows

The table below sets forth the primary sources and uses of cash for the years indicated.

In thousands	Year Ended June 30, 2020	2019	Increase (Decrease)	% Increase (Decrease)
Cash provided by (used in):				
Operating activities	$(41,620)	$(23,457)	$(18,163)	(77)%
Investing activities	8,399	19,027	(10,628)	(56)%
Financing activities	44,981	68	44,913	>100%
Net increase (decrease) in cash and cash equivalents	$ 11,760	$ (4,362)	$ 16,122	>100%

Operating activities. For both the years ended June 30, 2020 and 2019, cash used in operating activities was primarily the result of research and development and general and administrative expenses incurred in conducting normal business operations. Specifically, the cash used in operating activities of $41.6 million during the year ended June 30, 2020 was due to a net loss of $45.9 million, partially offset by non-cash items in our statement of operations of $2.2 million and favorable changes in our operating assets and liabilities of $2.1 million. The cash used in operating activities of $23.5 million during the year ended June 30, 2019 was due to a net loss of $2.0 million and unfavorable changes in our operating assets and liabilities of $25.6 million, partially offset by non-cash items in our statement of operations of $4.2 million. Such change in operating assets and liabilities was primarily due to recognizing our entire deferred revenue balance upon the termination of the Biogen collaboration agreement.

Investing activities. Cash provided by investing activities of $8.4 million for the year ended June 30, 2020 consisted primarily of cash proceeds of $72.5 million from maturities of investments, net of investment purchases of $58.9 million, partially offset by an equity investment in Bionic Sight of $4.0 million, purchases of property and equipment of $0.9 million and intellectual property costs of $0.3 million. Cash provided by investing activities of $19.0 million for the year ended June 30, 2019 consisted primarily of cash proceeds of $94.1 million from maturities of investments, net of investment purchases of $74.8 million, partially offset by purchases of property and equipment of $0.2 million and intellectual property costs of $0.1 million.

Financing activities. Cash provided by financing activities of $45.0 million for the year ended June 30, 2020 primarily consisted of: (i) proceeds of $34.8 million from the issuance of common stock, net of issuance costs; (ii) net cash received of $9.9 million from a collateralized debt arrangement that closed on June 30, 2020; and (iii) proceeds from exercises of common stock options. These items were partially offset by principal payments on a finance lease. Cash provided by financing activities of $68,000 for the year ended June 30, 2019 consisted of proceeds from exercises of common stock options, partially offset by principal payments on a capital lease and taxes paid related to equity awards.

Operating capital requirements

To date, we have not generated any revenue from product sales. We do not know when, or if, we will generate any revenue from product sales. We do not expect to generate significant revenue from product sales unless and until we obtain regulatory approval of and commercialize one of our current or future product candidates. We anticipate that we will continue to generate losses for the foreseeable future as we continue the development of, and seek regulatory approvals for, our product candidates, and begin to commercialize any approved products. We are subject to all of the risks incident in the development of new gene therapy products, and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business.

We believe that our existing cash and cash equivalents and investments as of June 30, 2020 will be sufficient to allow us to generate data from our ongoing clinical programs, initiate the XLRP Phase 2/3 clinical trial and fund

currently planned research and discovery programs into the fourth quarter of calendar year 2021. However, we will require substantial additional funding to finish the XLRP Phase 2/3 clinical trial, complete the process necessary to seek regulatory approval for our lead product candidates and build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize our lead product candidates, if approved.

Contractual obligations and commitments

We are a smaller reporting company, as defined by Rule 12b-2 of the Exchange Act, and are not required to provide this information.

Off-balance sheet arrangements

During the periods presented in this Annual Report on Form 10-K and as of June 30, 2020, we did not have any off-balance sheet arrangements, as defined in the rules and regulations of the Securities and Exchange Commission.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

We are a smaller reporting company, as defined by Rule 12b-2 of the Exchange Act, and are not required to provide this information.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

APPLIED GENETIC TECHNOLOGIES CORPORATION
INDEX TO FINANCIAL STATEMENTS

Report of Independent Registered Public Accounting Firm

To the Shareholders and the Board of Directors of Applied Genetic Technologies Corporation

Opinion on the Financial Statements

We have audited the accompanying balance sheets of Applied Genetic Technologies Corporation (the Company) as of June 30, 2020 and 2019, the related statements of operations, stockholders' equity and cash flows for each of the two years in the period ended June 30, 2020, and the related notes and financial statement schedule listed in the Index at Item 15(a) (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company at June 30, 2020 and 2019, and the results of its operations and its cash flows for each of the two years in the period ended June 30, 2020, in conformity with U.S. generally accepted accounting principles.

Adoption of New Accounting Standard

As discussed in Note 2 to the financial statements, the Company changed its method for accounting for leases in 2020.

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 Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits 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. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the 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.

/s/ Ernst & Young LLP
We have served as the Company's auditor since 2017.

Tampa, Florida
September 18, 2020

APPLIED GENETIC TECHNOLOGIES CORPORATION
BALANCE SHEETS

In thousands, except per share data	June 30, 2020	June 30, 2019
ASSETS		
Current assets:		
Cash and cash equivalents	$ 38,463	$ 26,703
Investments	41,995	55,292
Grants receivable	—	13
Prepaid and other current assets	2,506	2,276
Total current assets	82,964	84,284
Property and equipment, net	4,311	4,430
Intangible assets, net	1,098	1,013
Investment in Bionic Sight, LLC	8,096	1,945
Right-of-use assets - operating leases	3,422	—
Right-of-use asset - financing lease	80	—
Other assets	348	544
Total assets	$ 100,319	$ 92,216
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,355	$ 1,331
Accrued and other liabilities	10,502	8,024
Lease liabilities - operating	1,058	—
Lease liability - finance	48	—
Total current liabilities	12,963	9,355
Lease liabilities - operating, net of current portion	4,070	—
Lease liability - finance, net of current portion	38	—
Long-term debt, net of debt discounts and deferred financing fees	9,677	—
Other liabilities	2,555	4,152
Total liabilities	29,303	13,507
Stockholders' equity:		
Preferred stock, par value $0.001 per share, 5,000 shares authorized; no shares issued and outstanding	—	—
Common stock, par value $0.001 per share, 150,000 shares authorized; 25,813 and 18,226 shares issued; 25,793 and 18,207 shares outstanding at June 30, 2020 and 2019, respectively	25	18
Additional paid-in capital	252,519	214,324
Shares held in treasury of 20 and 19 at June 30, 2020 and 2019, respectively	(88)	(85)
Accumulated deficit	(181,440)	(135,548)
Total stockholders' equity	71,016	78,709
Total liabilities and stockholders' equity	$ 100,319	$ 92,216

The accompanying notes are an integral part of these financial statements.

APPLIED GENETIC TECHNOLOGIES CORPORATION
STATEMENTS OF OPERATIONS

In thousands, except per share data	Year Ended June 30, 2020	2019
Revenue:		
Collaboration revenue	$ 2,297	$41,128
Grant and other revenue	156	564
Total revenue	2,453	41,692
Operating expenses:		
Research and development	35,778	33,183
General and administrative and other	13,617	12,859
Total operating expenses	49,395	46,042
Loss from operations	(46,942)	(4,350)
Other income (expense), net:		
Investment income, net	1,185	2,009
Other income (expense), net	(5)	446
Total other income (expense), net	1,180	2,455
Loss before provision for income taxes	(45,762)	(1,895)
Provision for income taxes	83	76
Loss before equity in net losses of an affiliate	(45,845)	(1,971)
Equity in net losses of an affiliate	(47)	(35)
Net loss	$(45,892)	$ (2,006)
Weighted average shares outstanding:		
Basic	21,102	18,157
Diluted	21,102	18,157
Net loss per common share:		
Basic	$ (2.17)	$ (0.11)
Diluted	$ (2.17)	$ (0.11)

The accompanying notes are an integral part of these financial statements.

APPLIED GENETIC TECHNOLOGIES CORPORATION
STATEMENTS OF STOCKHOLDERS' EQUITY
YEARS ENDED JUNE 30, 2020 and 2019

In thousands	Common Stock Outstanding Shares	Common Stock Amount	Treasury Stock Shares	Treasury Stock Amount	Additional Paid-in Capital	Accumulated Deficit	Totals
Balances at June 30, 2018	18,126	$ 18	11	$ (49)	$210,139	$ (110,926)	$ 99,182
Cumulative impact of adopting ASC Topic 606	—	—	—	—	—	(22,616)	(22,616)
Share-based compensation expense	—	—	—	—	4,029	—	4,029
Shares issued under employee plans	81	—	8	(36)	156	—	120
Net loss	—	—	—	—	—	(2,006)	(2,006)
Balances at June 30, 2019	18,207	18	19	(85)	214,324	(135,548)	78,709
Issuance of common stock, net of issuance costs	7,475	7	—	—	34,804	—	34,811
Share-based compensation expense	—	—	—	—	2,998	—	2,998
Shares issued under employee plans	111	—	1	(3)	393	—	390
Net loss	—	—	—	—	—	(45,892)	(45,892)
Balances at June 30, 2020	25,793	$ 25	$ 20	$ (88)	$252,519	$ (181,440)	$ 71,016

The accompanying notes are an integral part of these financial statements.

APPLIED GENETIC TECHNOLOGIES CORPORATION
STATEMENTS OF CASH FLOWS

In thousands	Year Ended June 30 2020	2019
Operating activities:		
Net loss	$(45,892)	$ (2,006)
Adjustments to reconcile net loss to net cash used in operating activities:		
Share-based compensation expense	2,998	4,029
Depreciation and amortization	1,335	1,273
Recovery of bad debts	—	(358)
Investment discount accretion, net	(299)	(790)
Reduction in the carrying amount of operating lease right-of-use assets	303	—
Collaboration revenue from Bionic Sight, LLC	(2,197)	—
Equity in net losses of an affiliate	47	35
Changes in operating assets and liabilities:		
Grants receivable	13	186
Prepaid and other assets	(28)	1,635
Deferred revenue	149	(28,392)
Accounts payable	83	327
Operating lease liabilities	(714)	—
Accrued and other liabilities	2,582	604
Cash used in operating activities	(41,620)	(23,457)
Investing activities:		
Purchases of property and equipment	(943)	(163)
Purchases of and capitalized costs related to intangible assets	(254)	(148)
Investment in Bionic Sight, LLC	(4,000)	—
Maturities of investments	72,500	94,119
Purchases of investments	(58,904)	(74,781)
Cash provided by investing activities	8,399	19,027
Financing activities:		
Proceeds from the issuance of common stock, net of issuance costs	34,811	—
Proceeds from exercises of common stock options	393	156
Proceeds from long-term debt borrowing, net of debt discounts	9,855	—
Payments for deferred financing fees	(30)	—
Taxes paid related to equity awards	(3)	(36)
Principal payments on finance/capital lease	(45)	(52)
Cash provided by financing activities	44,981	68
Net increase (decrease) in cash and cash equivalents	11,760	(4,362)
Cash and cash equivalents, beginning of the year	26,703	31,065
Cash and cash equivalents, end of the year	$ 38,463	$ 26,703
Supplemental information:		
Cash paid (refunds received) during the year for income taxes, net	$ (396)	$ —
Deferred financing fees included in accrued and other liabilities	149	—
Costs for purchases of property and equipment included in accrued and other liabilities	326	124
Costs for intangible assets included in accounts payable/accrued and other liabilities	60	59

The accompanying notes are an integral part of these financial statements.

APPLIED GENETIC TECHNOLOGIES CORPORATION
NOTES TO FINANCIAL STATEMENTS

1. Organization and Operations

Applied Genetic Technologies Corporation (the "Company" or "AGTC") was incorporated as a Florida corporation on January 19, 1999 and reincorporated as a Delaware corporation on October 24, 2003. The Company is a clinical-stage biotechnology company that uses a proprietary gene therapy platform to develop transformational genetic therapies for patients suffering from rare and debilitating diseases.

On February 11, 2020, the Company closed an underwritten public offering of 6.5 million shares of its common stock at $5.00 per share, generating gross proceeds of $32.5 million, before deducting underwriting discounts, commissions and other offering expenses payable by the Company. Additionally, the underwriters exercised their option to purchase an additional 975,000 shares of common stock to cover over-allotments, and such transaction closed on February 13, 2020 and generated additional gross proceeds of $4.9 million.

On June 30, 2020, the Company entered into a loan agreement for a term loan in the aggregate principal amount of up to $25.0 million. On that date, the Company received net loan proceeds of $9.9 million, before consideration of any related debt financing fees. The loan agreement is further discussed at Note 8 in these Notes to Financial Statements.

In July 2015, the Company entered into a collaboration agreement with Biogen MA, Inc., a wholly owned subsidiary of Biogen Inc. ("Biogen"), pursuant to which the Company and Biogen collaborated to develop, seek regulatory approval for and commercialize gene therapy products to treat X-linked retinoschisis ("XLRS"), X-linked retinitis pigmentosa ("XLRP") and discovery programs targeting three indications based on the Company's adeno-associated virus vector technologies. The Biogen collaboration agreement became effective in August 2015. On December 7, 2018, the Company received notice from Biogen that it had elected to terminate the collaboration agreement, which became effective on March 8, 2019. The Biogen collaboration agreement and other transactions with Biogen are further discussed at Note 9 in these Notes to Financial Statements.

The Company has devoted substantially all of its efforts to research and development, including clinical trials. The Company has not completed the development of any products. The Company has generated revenue from collaboration agreements, sponsored research payments and grants, but has not generated product revenue to date and is subject to a number of risks similar to those of other early stage companies in the biotechnology industry, including dependence on key individuals, the difficulties inherent in the development of commercially viable products, the need to obtain additional capital necessary to fund the development of its products, development by the Company or its competitors of technological innovations, risks of failure of clinical studies, protection of proprietary technology, compliance with government regulations and ability to transition to large-scale production of products. As of June 30, 2020, the Company had an accumulated deficit of $181.4 million. While the Company expects to continue to generate some revenue from partnering, the Company expects to incur losses for the foreseeable future. The Company has funded its operations to date primarily through public offerings of its common stock, private placements of its preferred stock, collateralized borrowing and collaborations. As of June 30, 2020, the Company had cash and cash equivalents and liquid investments of $80.5 million.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying financial statements have been prepared in conformity with U.S. generally accepted accounting principles ("U.S. GAAP"). The Company's fiscal year ends on June 30.

Segment Reporting

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision-maker when making decisions regarding resource allocation and assessing performance. To date, management has viewed the Company's operations and managed its business as one segment.

Use of estimates

The preparation of financial statements in conformity with U.S. GAAP and guidelines from the Securities and Exchange Commission (the "SEC"), requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenue and expenses during reporting periods. Actual results could differ from those estimates.

Cash and cash equivalents

Cash consists of funds held in bank accounts. Cash equivalents consist of short-term, highly liquid investments with original maturities of 90 days or less at the time of purchase and generally include money market accounts.

Investments

The Company's investments have historically consisted of certificates of deposit and debt securities classified as held-to-maturity. Management determines the appropriate classification of debt securities at the time of purchase and reevaluates such designation at each balance sheet date. Debt securities are classified as held-to-maturity when management has the positive intent and ability to hold the securities to maturity. Held-to-maturity securities are stated at amortized cost, adjusted for amortization of premiums and accretion of discounts to maturity. Such amortization is included in investment income, net. Interest income from debt securities classified as held-to-maturity is included in investment income, net.

The Company uses the specific identification method to determine the cost basis of securities sold.

Investments are considered to be impaired when a decline in fair value is judged to be other-than-temporary. The Company evaluates an investment for impairment by considering the length of time and extent to which market value has been less than cost or amortized cost, the financial condition and near-term prospects of the issuer, as well as specific events or circumstances that may influence the operations of the issuer, and the Company's intent to sell the security or the likelihood that it will be required to sell the security before recovery of its amortized cost. Once a decline in fair value is determined to be other-than-temporary, an impairment charge is recorded to investment income, net and a new cost basis in the investment is established.

Concentrations of Credit Risk

As of June 30, 2020, the Company's cash and cash equivalents were held on deposit with two financial institutions that are federally insured. However, a portion of those cash and cash equivalents exceed federally insured limits and, as a result, could potentially expose the Company to significant concentrations of credit risk. To date, the Company has not experienced any losses associated with this credit risk and management continues to believe that this exposure is not significant. The Company invests its excess cash primarily in money market funds, certificates of deposit, and debt instruments of corporations and U.S. government agencies. These investments generally mature within a two-year period from their purchase date, which is consistent with the Company's investment policy that seeks to maintain adequate liquidity and preserve capital.

Inventory

Purchases of clinical materials stored for master and working viral banks that remain at the sites in anticipation of their future use at that site are charged to expense when the related liability is incurred. Since the Company can

use each of the raw materials in only a single product, each raw material is deemed to have no future economic value independent of the development status of that unique drug.

Fair value of financial instruments

The Company is required to disclose information regarding all assets and liabilities reported at fair value that enables an assessment of the inputs used when determining the reported fair values. The Financial Accounting Standards Board (the "FASB") Accounting Standards Codification ("ASC") Topic 820, *Fair Value Measurements and Disclosures*, establishes a hierarchy of inputs used when available. Observable inputs are inputs that market participants would use when pricing an asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use when pricing an asset or liability and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used when determining the reported fair value of financial instruments and is not a measure of an investment's credit quality. The three levels of the fair value hierarchy are described below.

Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

Level 2—Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.

Level 3—Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that a valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company when determining fair value is greatest for financial instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

Property and equipment

Property and equipment, which consists of laboratory equipment, furniture and fixtures, computer equipment and leasehold improvements, is recorded at cost less accumulated depreciation and amortization. Depreciation is calculated using the straight-line method over the estimated useful life of the underlying asset, which is generally three to ten years (a weighted average useful life of 6.8 years as of June 30, 2020). Leasehold improvements are amortized over the shorter of the estimated useful life of the underlying asset or the related lease term, including any renewal periods that are deemed to be reasonably assured. Repair and maintenance costs that do not improve service potential or extend an asset's economic life are recorded as an expense when incurred.

Leases

On July 1, 2019, the Company adopted Accounting Standards Update ("ASU") No. 2016-02, *Leases.* Prior to July 1, 2019, the Company recognized lease expense in accordance with then-existing U.S. GAAP under FASB ASC Topic 840, *Leases.* Information regarding the Company's lease accounting, including its adoption of the new accounting standard, is provided below under the heading "New Accounting Pronouncements - Adopted during the year ended June 30, 2020" and Note 3 in these Notes to Financial Statements.

Intangible assets

Intangible assets primarily consist of licenses and patents. The Company obtains licenses from third parties and capitalizes the costs related to exclusive licenses that have alternative future use in multiple potential programs.

The Company also capitalizes costs related to filing, issuance and prosecution of patents. The Company reviews its capitalized costs periodically to determine that such costs relate to patent applications that have future value and an alternative future use. The Company writes off costs associated with patents that are no longer being actively pursued or that have no future benefit.

Amortization expense for intangible assets is computed using the straight-line method over the estimated useful life of the underlying asset, which is generally eight to twenty years (a weighted average useful life of 11.7 years as of June 30, 2020). The Company amortizes in-licensed patents and patent applications from the date of the applicable license and internally developed patents and patent applications from the date of the initial application. Licenses and patents converted to research use only are immediately expensed.

Impairment of long-lived assets

The Company reviews its long-lived asset groups for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset or an asset group may not be recoverable. Such evaluation could be triggered by a number of factors, including current and projected operating results/cash flows and changes in management's strategic direction, as well as external economic and market factors. The Company evaluates the recoverability of assets and asset groups by determining whether their carrying values can be recovered through undiscounted future cash flows. If events or circumstances indicate that the carrying values might not be recoverable based on undiscounted future cash flows, an impairment charge may be recognized. Management considers various factors when calculating an impairment charge, including trends and prospects, as well as the effects of obsolescence, demand, competition and other macroeconomic information. The Company did not record any impairment charges for long-lived assets during the years ended June 30, 2020 and 2019.

Financing fees

Financing fees consist of costs, including those for legal services, that are necessary to secure commitments under debt financing arrangements. Those costs are deferred and recognized as interest expense over the period of the related financing arrangement using the effective interest method. If a financing arrangement is terminated or otherwise satisfied, any remaining deferred financing fees are immediately recognized as interest expense. The Company's financial statements present deferred financing fees as a direct reduction of the carrying amount of the corresponding liability.

Revenue recognition

Effective July 1, 2018, the Company adopted the provisions of ASC Topic 606, *Revenue from Contracts with Customers* ("Topic 606"), using the modified retrospective transition method. Under that method, the Company recorded the cumulative effect of initially applying the new standard to all contracts in process as of the date of adoption. Topic 606 applies to all contracts with customers, except for contracts that are within the scope of other standards. The adoption of Topic 606 resulted in an increase of $22.6 million in deferred revenue and accumulated deficit as of July 1, 2018.

The Company may enter into collaboration agreements, which are within the scope of Topic 606, where it licenses rights to its technology and certain of its product candidates and performs research and development services for third parties. The terms of these arrangements may include payment of one or more of the following: upfront fees; reimbursement of research and development costs; development, regulatory and commercial milestone payments; and royalties on net sales of licensed products.

Under Topic 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration that the entity expects to receive in exchange for those goods or services. To determine the appropriate amount of revenue to be recognized for arrangements determined to be within the scope of Topic 606, the Company performs the following five steps: (i) identification of the contract;

(ii) determination of whether the promised goods or services are performance obligations; (iii) measurement of the transaction price, including any constraints on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation. The Company only applies the five-step model to contracts if it is probable that it will collect consideration that the Company is entitled to in exchange for the goods or services it transfers to the customer.

Performance obligations are promises to transfer distinct goods or services to a customer. Promised goods or services are considered distinct when (i) the customer can benefit from the good or service on its own or together with other readily available resources and (ii) the promised good or service is separately identifiable from other promises in the contract. When assessing whether promised goods or services are distinct, the Company considers factors such as the stage of development of the underlying intellectual property, the capabilities of a customer to develop the intellectual property on its own or whether the required expertise is readily available.

The Company estimates the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include both fixed consideration and variable consideration. At the inception of an arrangement that includes variable consideration and at the end of each reporting period, the Company evaluates the amount of potential customer payments and the likelihood that such payments will be received. The Company utilizes either the most likely amount method or the expected amount method to estimate the amount to be received based on which method better predicts the amount expected to be received. If it is probable that a significant revenue reversal would not occur, the variable consideration is included in the transaction price. The Company will assess its revenue generating arrangements to determine whether a significant financing component exists and conclude that a significant financing component does not exist in an arrangement if the: (a) promised consideration approximates the cash selling price of the promised goods and services or any significant difference is due to factors other than financing; and (b) timing of payment approximates the transfer of goods and services and performance is over a relatively short period of time within the context of the entire term of the contract.

The Company’s contracts often include development and regulatory milestone payments. At contract inception, the Company evaluates whether any such milestones are considered probable of being reached and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company’s control or the customer’s control, such as regulatory approvals, are not included in the transaction price. At the end of each subsequent reporting period, the Company reevaluates the probability of achievement of such development milestones and any related constraint and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenue and earnings in the period of adjustment.

For arrangements that may include sales-based royalties, including milestone payments based on the level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sale occurs or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of the Company’s collaboration arrangements.

The Company allocates the transaction price based on the estimated stand-alone selling price of the underlying performance obligation or, in the case of certain variable consideration, to one or more performance obligations. The Company uses assumptions that require judgment to determine the stand-alone selling price for each performance obligation identified in a contract. The Company utilizes key assumptions to determine the stand-alone selling price, which may include other comparable transactions, pricing considered in negotiating the transaction and the estimated costs to complete the related performance obligation. Certain variable consideration is allocated specifically to one or more performance obligation in a contract when the terms of the variable

consideration relate to the satisfaction of a performance obligation and the resulting amounts allocated to each performance obligation are consistent with the amounts the Company would expect to receive for each performance obligation.

For performance obligations consisting of licenses and other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue from upfront fees. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. If the license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company will recognize revenue from upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license.

The Company receives payments from its customers based on billing terms established in each contract. Such billings generally have 30-day payment terms. Upfront payments and fees are recorded as deferred revenue upon receipt or when due until the Company performs its obligations under those arrangements. Amounts are recorded as accounts receivable when the right to consideration is unconditional.

Collaboration arrangements

The Company analyzes its collaboration arrangements to assess whether they are within the scope of ASC Topic 808, *Collaborative Arrangements* ("Topic 808"), and to determine whether such arrangements involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities. This assessment is performed throughout the life of a collaboration arrangement with special consideration given to changes in the responsibilities of the parties in the arrangement. For collaboration arrangements within the scope of Topic 808 that contain multiple elements, the Company first determines which elements of the collaboration are deemed to be within the scope of Topic 808 and those that are more reflective of a vendor-customer relationship and, therefore, within the scope of Topic 606. For elements of collaboration arrangements that are accounted for pursuant to Topic 808, an appropriate recognition method is determined and applied consistently, generally by analogy to Topic 606.

As discussed below under the heading "New Accounting Pronouncements - Adopted effective July 1, 2020," the Company adopted ASU No. 2018-18, *Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606*, on July 1, 2020; however, the new standard did not have a significant impact on the Company's financial statements.

The Company's collaboration arrangements are further discussed at Note 9 in these Notes to Financial Statements.

Income taxes

The Company uses the asset and liability method to account for income taxes. Under this method, deferred tax assets and liabilities are recognized for the estimated future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective income tax bases. Deferred tax assets and liabilities are measured using enacted rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled.

As required by U.S. GAAP, the Company recognizes the financial statement benefit of a tax position only after determining that the relevant tax authority would more likely than not sustain the position following an audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements is the

largest benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the relevant tax authority. Interest and penalties related to uncertain tax positions are reflected in the provision for income taxes. The Company is subject to examination of its income tax returns in the federal and state tax jurisdictions in which it operates for the tax years ended June 30, 2016 through June 30, 2020.

For the years ended June 30, 2020 and 2019, the Company's provision for income taxes consisted of $83,000 and $76,000, respectively, related to estimated interest and penalties on the Company's uncertain tax positions. The uncertain tax position liability as of June 30, 2020 and 2019 was $2.1 million and $2.0 million, respectively. The Company's income taxes are further discussed at Note 11 in these Notes to Financial Statements.

Research and development expenses

Research and development expenses include costs incurred in identifying, developing and testing product candidates and generally comprise compensation and related benefits and non-cash share-based compensation to research-related employees; laboratory costs; animal and laboratory maintenance and supplies; rent; utilities; clinical and pre-clinical expenses; and payments for sponsored research, scientific and regulatory consulting fees and testing.

As part of the process of preparing its financial statements, the Company estimates its accrued expenses. This process involves reviewing quotations and contracts, identifying services that have been performed on the Company's behalf and estimating the level of services performed and the associated cost incurred for services for which the Company has not yet been invoiced or otherwise notified of the actual cost. The majority of the Company's service providers invoice monthly in arrears for services performed or when contractual milestones are met. The Company makes estimates of its accrued expenses at the end of each reporting period based on the facts and circumstances known to the Company at that time. The significant estimates in the Company's accrued research and development expenses relate to expenses incurred with respect to academic research centers, contract research organizations, and other vendors in connection with research and development activities for which the Company has not yet been invoiced.

There are instances where the Company's service providers require advance payments at the inception of a contract and other circumstances where the Company's payments to a vendor will exceed the level of services provided, in both cases resulting in a prepayment of research and development expenses. Such prepayments are charged to research and development expense as and when the service is provided or when a specific milestone outlined in the contract is reached.

Prepayments related to research and development activities were $1.0 million and $0.7 million at June 30, 2020 and 2019, respectively, and are included in prepaid and other current assets on the Company's balance sheets.

Share-based compensation

The Company accounts for share-based awards issued to employees in accordance with ASC Topic 718, *Compensation—Stock Compensation,* and generally recognizes share-based compensation expense on a straight-line basis over the period during which an employee is required to provide service in exchange for the award. In addition, the Company awards stock options and restricted shares of common stock to nonemployees in exchange for consulting services. Prior to July 1, 2019, the Company accounted for these awards in accordance with the provisions of ASC Subtopic 505-50, *Equity-Based Payments to Non-employees* ("ASC 505-50"). Under ASC 505-50, share-based awards to nonemployees were subject to periodic fair value re-measurement over their vesting terms. As discussed below under the heading "New Accounting Pronouncements - Adopted during the year ended June 30, 2020," the Company adopted ASU No. 2018-07, *Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting*, on July 1, 2019. As a result, the Company's accounting for nonemployee awards is now generally consistent with that of employee awards. Beginning on July 1, 2019, the measurement date for nonemployee awards is the date of grant without any

subsequent changes in the fair value of the award. Share-based compensation costs for nonemployees are generally recognized as expense as services are provided to the Company over the related service period.

For purposes of calculating share-based compensation expense, the Company estimates the fair value of stock options using a Black-Scholes option-pricing model. The determination of the fair value of a share-based compensation award utilizing the Black-Scholes model is affected by the Company's stock price and a number of assumptions, including the expected volatility of the Company's stock, the expected life of the stock option, the risk-free interest rate and expected dividends. Additionally, the Company uses a Monte Carlo simulation model to determine the fair value of restricted stock units for purposes of calculating share-based compensation expense. The Monte Carlo simulation model incorporates the probability of satisfying a market condition and uses transaction details such as the Company's stock price, contractual terms, maturity and risk-free rates, as well as volatility.

If factors change and the Company employs different assumptions, share-based compensation expense may differ significantly from what has been recorded in the past. If there is a difference between the assumptions used in determining share-based compensation expense and the actual factors that become known over time, specifically with respect to anticipated forfeitures, the Company may change the input factors used in determining share-based compensation costs for future awards. These changes, if any, may materially impact the Company's results of operations in the period that such changes are made.

Net income or loss per share

Basic net income or loss per share is calculated by dividing net income or loss by the weighted average shares outstanding during the period, without consideration of common stock equivalents. Diluted net income or loss per share is calculated by adjusting weighted average shares outstanding for the dilutive effects of common stock equivalents outstanding during the period, determined using the treasury stock method. For purposes of diluted net income or loss per share calculations, stock options, restricted stock awards and performance service awards are considered to be common stock equivalents if they are dilutive. The dilutive impact of common stock equivalents for each of the years ended June 30, 2020 and 2019 was approximately 0.2 million shares. However, the dilutive impact of common stock equivalents was excluded from the calculations of diluted net loss per share for each of the years ended June 30, 2020 and 2019 because their effects were anti-dilutive. Therefore, for each of the years ended June 30, 2020 and 2019, basic and diluted net loss per share were the same.

Comprehensive loss

Comprehensive income or loss consists of net income or loss and changes in equity during a period from transactions and other equity and circumstances generated from non-owner sources. For the years ended June 30, 2020 and 2019, the Company's net loss is the same as its comprehensive loss.

New Accounting Pronouncements

Adopted during the year ended June 30, 2020

Leases

In February 2016, the FASB issued ASU No. 2016-02, *Leases* ("ASU 2016-02"), which superseded FASB ASC Topic 840, *Leases* ("Topic 840") and provides principles for the recognition, measurement, presentation and disclosure of leases for both lessees and lessors. The new accounting guidance requires the recognition of long-term lease assets and lease liabilities by lessees and sets forth new disclosure requirements for leases. The standard requires lessees to recognize right-of-use assets and lease liabilities on the balance sheet. An entity can transition to ASU 2016-02 using a modified retrospective approach at either the beginning of the earliest comparative period in the financial statements or at the beginning of the period of adoption. A lessee is also required to record a right-of-use asset and a lease liability regardless of lease classification. The FASB

subsequently issued several ASUs amending the new standard. The Company adopted the new standard effective July 1, 2019 using the modified retrospective approach at the beginning of the period of adoption. As a result, prior periods are presented in accordance with the previous guidance in Topic 840.

ASU 2016-02 provides a number of optional practical expedients during transition. The Company elected a package of practical expedients that permits it to not reassess: (i) whether any expired or existing contracts are or contain leases; (ii) the lease classification for any expired or existing leases; and (iii) initial direct costs for any existing leases. Additionally, upon adopting ASU 2016-02, the Company did not elect the hindsight practical expedient, which allows companies to use current knowledge and expectations when determining the likelihood of exercising lease options. By not electing this practical expedient, the Company did not reassess the lease term for any leases identified under Topic 840.

The Company will continue to differentiate between finance leases (previously referred to as capital leases) and operating leases using classification criteria in ASU 2016-02 that are substantially similar to the previous guidance in Topic 840. The adoption of ASU 2016-02 resulted in the recognition of right-of-use assets – operating leases and related lease liabilities of $3.7 million and $5.8 million, respectively, on the Company's balance sheet as of July 1, 2019. There was no material impact from ASU 2016-02 on the Company's Statement of Operations for the year ended June 30, 2020. The Company made a policy election not to record a right-of-use asset or lease liability for leases with an expected duration of 12 months or less at inception; however, such leases are not material to the Company. For leases with terms greater than 12 months, the Company records a right-of-use asset and lease liability at the present value of the future lease payments. When it is reasonably certain that the Company will exercise a renewal option for one of its leases, the present value of the lease payments for the affected lease is adjusted accordingly. Variable lease payments that are not dependent on an index or a rate are excluded from the determination of the Company's right-of-use assets and lease liabilities and such payments are recognized as expense in the period when the related obligation is incurred. As the Company's leases do not provide readily determinable implicit interest rates, an incremental borrowing rate commensurate with a lease's term is used to discount future lease payments. The application of ASU 2016-02 required netting the unamortized balance of lease incentives and deferred lease obligations against the right-of-use assets on the date of adoption. The Company's operating leases include rent escalation clauses that are factored into the determination of future lease payments when appropriate. The Company does not separate lease and nonlease components of its contracts when applying the provisions of ASU 2016-02.

Refer to Note 3, Leases, in these Notes to Financial Statements for additional information about the Company's leasing activities.

Share-Based Compensation

In June 2018, the FASB issued ASU No. 2018-07, *Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting*. The new standard aligns the measurement and classification guidance for share-based payments to nonemployees with the guidance for share-based payments to employees, with certain exceptions. Under the guidance, the measurement of equity-classified nonemployee awards is fixed on the date of grant, which may lower the total recognized cost and reduce income statement volatility. The Company adopted the new standard on July 1, 2019; however, there was only an immaterial impact on the Company's financial position and results of operations as of and for the year ended June 30, 2020.

Adopted effective July 1, 2020

Fair Value Measurement

In August 2018, the FASB issued ASU No. 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement*. The new standard eliminates, adds and modifies certain disclosure requirements for fair value measurement as part of the FASB's

disclosure framework project. Under the new standard, the amount and reason for a transfer between Level 1 and Level 2 of the fair value hierarchy is no longer required to be disclosed but public companies are required to disclose a range and weighted average of significant unobservable inputs for Level 3 fair value measurements. The Company adopted the new standard on July 1, 2020; however, it did not have a significant impact on the Company's financial statements.

Collaborative Arrangements

In November 2018, the FASB issued ASU No. 2018-18, *Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606*. The new standard clarifies that certain transactions between participants in a collaborative arrangement should be accounted for under Topic 606 when the counterparty is a customer. The new standard also 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. The guidance amends Topic 808 to refer to the unit-of-account guidance in Topic 606 and requires it to be used only when assessing whether a transaction is in the scope of Topic 606. The Company adopted the new standard on July 1, 2020; however, it did not have a significant impact on the Company's financial statements.

To be adopted in future periods

Financial Instruments—Credit Losses

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments – Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments*. The new standard requires that financial assets measured at amortized cost be presented at the net amount expected to be collected and separately measure an allowance for credit losses that is deducted from the amortized cost basis of those financial assets. This standard will be effective for the Company on July 1, 2023. Early adoption is permitted. Management continues to evaluate the provisions of this new standard and its potential impact; however, the adoption thereof is not expected to have a significant impact on the Company's financial statements.

Income Taxes

In December 2019, the FASB issued ASU No. 2019-12, *Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes*. The new standard includes several provisions that simplify accounting for income taxes by removing certain exceptions to the general principles in Topic 740 and increasing consistency and clarity for the users of financial statements. This standard will be effective for the Company on July 1, 2021. Early adoption is permitted. The adoption of this guidance is not expected to have a significant impact on the Company's financial statements.

Investments – Equity Securities, Investments – Equity Method and Joint Ventures, and Derivatives and Hedging

In January 2020, the FASB issued ASU No. 2020-01, *Investments – Equity Securities (Topic 321), Investments – Equity Method and Joint Ventures (Topic 323), and Derivatives and Hedging (Topic 815)—Clarifying the Interactions between Topic 321, Topic 323, and Topic 815*. The new standard addresses interactions between the guidance to account for certain equity securities under ASC Topic 321, the guidance to account for investments under the equity method of accounting in ASC Topic 323, and the guidance in ASC Topic 815, which could change how an entity accounts for an equity security under the measurement alternative or a forward contract or purchased option to purchase securities that, upon settlement of the forward contract or exercise of the purchased option, would be accounted for under the equity method of accounting or the fair value option in accordance with ASC Topic 825, *Financial Instruments*. These amendments improve current U.S. GAAP by reducing diversity in practice and increasing comparability of the accounting for any such interactions. This standard will be effective for the Company on July 1, 2021. Early adoption is permitted. The adoption of this guidance is not expected to have a significant impact on the Company's financial statements.

3. Leases

The Company leases certain laboratory and office space under operating leases, which are described below.

Alachua, Florida

The Company's headquarters is located in Alachua, Florida where it leases approximately 21,500 square feet of office and laboratory space under a lease arrangement that expires in December 2027. The Company has options to extend the term of the Alachua lease for three additional five-year periods.

Cambridge, Massachusetts

The Company leases approximately 8,000 square feet of office and laboratory space in Cambridge, Massachusetts under a lease arrangement that expires in February 2025. The Company has an option to extend the Cambridge lease for one additional three-year term. The Cambridge facility primarily focuses on business development, pharmacology and basic research and development.

Additionally, the Company leases certain office equipment under a finance lease.

Lease costs recognized under ASU 2016-02

The table below summarizes the lease costs recognized under ASU 2016-02 and other information pertaining to the Company's operating and finance leases for the year ended June 30, 2020.

In thousands	
Lease cost:	
Finance lease cost	
Amortization of right-of-use asset	$ 46
Interest on lease liability	8
Operating lease cost	770
Variable lease cost	379
Total lease cost	$1,203
Cash paid for amounts included in the measurement of lease liabilities:	
Operating cash flows used for finance lease	$ 8
Operating cash flows used for operating leases	$1,091
Financing cash flows used for finance lease	$ 45
Other information:	
Weighted average remaining lease term - operating leases (in years)	6.1
Weighted average remaining lease term - finance lease (in years)	1.8
Weighted average discount rate - operating leases	8.5%
Weighted average discount rate - finance lease	6.9%

Amortization of the right-of-use asset - finance lease is included in general and administrative and other in the Company's Statements of Operations. Operating lease cost and variable lease cost are included as rent expense in general and administrative and other, and research and development in the Company's Statements of Operations. Variable lease cost primarily includes the Company's allocated share of the expenses incurred by its landlords to operate and manage the office and laboratory space that the Company leases.

As of June 30, 2020, future minimum commitments for the Company's operating and finance leases for the years ending June 30 are summarized below.

In thousands	
Operating lease liabilities:	
2021	$ 1,109
2022	1,128
2023	1,148
2024	1,168
2025	888
Thereafter	1,159
Total future minimum payments for operating leases	6,600
Imputed interest	(1,472)
Operating lease liabilities per the balance sheet	$ 5,128
Finance lease liability:	
2021	$ 53
2022	39
Total future minimum payments for finance lease	92
Imputed interest	(6)
Total finance lease liability per the balance sheet	$ 86

Based on the Company's selected method of adoption for ASU 2016-02, the prior U.S. GAAP disclosures under Topic 840 are required to be presented in the Company's financial statements during the year ended June 30, 2020. As such, below are the Company's annual minimum lease payments, including estimated common area maintenance charges, for the years ended or ending June 30 under non-cancelable operating leases as of June 30, 2019.

In thousands	
2020	$1,353
2021	1,376
2022	1,400
2023	1,425
2024	1,450
Thereafter	2,638
Total	$9,642

Under Topic 840, the Company's rent expense for operating leases was $1.1 million during the year ended June 30, 2019. Contractual rent increases, rent concessions and landlord incentives were recognized ratably over the life of the related lease agreement under Topic 840.

4. Investments

Cash in excess of immediate requirements is invested in accordance with the Company's investment policy, which primarily seeks to maintain adequate liquidity and preserve capital. At both June 30, 2020 and 2019, the Company's investments consisted entirely of held-to-maturity debt securities that were due in one year or less from the respective balance sheet dates.

The Company's debt securities that are classified as held-to-maturity are summarized below.

In thousands	June 30, 2020	2019
U.S. Treasury Securities:		
Amortized cost	$41,995	$55,292
Gross unrealized gains	54	78
Gross unrealized losses	(3)	—
Fair value of investments	$42,046	$55,370

The Company expects to collect the principal and interest due on its debt securities that have an amortized cost in excess of fair value. At the end of each reporting period, the Company evaluates its securities for impairment, if and when, the fair value of an investment is less than its amortized cost. In the event that the fair value of an investment is less than its amortized cost, the Company will evaluate the underlying credit quality and credit ratings of the issuer. Specifically, the Company believes that the unrealized losses disclosed in the above table were primarily driven by interest rate changes rather than by unfavorable changes in the credit ratings associated with those securities. The Company does not intend to sell any of its investments before recovering its amortized cost base, which may be at maturity.

5. Fair Value of Financial Instruments and Investments

Certain assets and liabilities are measured at fair value in the Company's financial statements or have fair values disclosed in these Notes to Financial Statements. Such assets and liabilities are classified into one of three levels of a hierarchy defined by U.S. GAAP. The Company's assessment of the significance of a particular item to the fair value measurement in its entirety requires judgment, including the consideration of inputs specific to the asset or liability. The methods and assumptions described below were used to estimate fair values and determine the fair value hierarchy classification of each class of financial instrument held by the Company.

Cash and Cash Equivalents. The carrying value of cash and cash equivalents approximates fair value as maturities are less than three months.

Debt securities—held-to-maturity. The Company's investments in debt securities classified as held-to-maturity consist of U.S. Treasury Securities, which are valued using quoted market prices. Valuation adjustments are not applied. Accordingly, U.S. Treasury Securities are valued using Level 1 inputs under the fair value hierarchy.

The fair value hierarchy table below provides information about each major category of the Company's financial assets and liabilities measured at fair value on a recurring basis or disclosed at fair value in these Notes to Financial Statements.

In thousands	Level 1	Level 2	Level 3	Total Fair Value
June 30, 2020				
Cash and cash equivalents	$38,463	$ —	$ —	$ 38,463
Held-to-maturity investments:				
U.S. Treasury Securities	42,046	—	—	42,046
Total assets	$80,509	$ —	$ —	$ 80,509
June 30, 2019				
Cash and cash equivalents	$26,703	$ —	$ —	$ 26,703
Held-to-maturity investments:				
U.S. Treasury Securities	55,370	—	—	55,370
Total assets	$82,073	$ —	$ —	$ 82,073

The Company's financial instruments also include its variable-rate borrowing under a debt agreement that is described at Note 8 in these Notes to Financial Statements. The Company believes that such debt's carrying amount of $9.7 million reasonably approximates its fair value because the rate of interest on such borrowing reflects current market rates of interest for similar instruments with comparable maturities and risk profiles. This assessment primarily uses Level 2 inputs under the fair value hierarchy.

6. Property and Equipment, Net

The table below summarizes the Company's property and equipment, net as of the dates indicated.

	June 30,	
In thousands	2020	2019
Laboratory equipment	$ 4,249	$ 2,938
Equipment (construction in progress)	2	206
Leasehold improvements	3,881	3,851
Office equipment, including a $249 capital lease asset in 2019	837	1,074
Property and equipment, gross	8,969	8,069
Accumulated depreciation and amortization	(4,658)	(3,639)
Property and equipment, net	$ 4,311	$ 4,430

The Company recognized depreciation and amortization expense of $1.2 million and $1.1 million during the years ended June 30, 2020 and 2019, respectively, including $0.5 million of amortization expense for leasehold improvements during each year.

7. Intangible Assets, Net

The tables below summarize the Company's intangible assets, net as of the dates indicated.

	June 30, 2020		
In thousands	Cost	Accumulated Amortization	Intangible Assets, net
Patents	$2,637	$ (1,649)	$ 988
Licenses	289	(214)	75
Other	59	(24)	35
Totals	$2,985	$ (1,887)	$ 1,098

	June 30, 2019		
In thousands	Cost	Accumulated Amortization	Intangible Assets, net
Patents	$2,392	$ (1,499)	$ 893
Licenses	289	(199)	90
Other	49	(19)	30
Totals	$2,730	$ (1,717)	$ 1,013

The Company recognized amortization expense related to intangible assets of $170,000 and $163,000 during the years ended June 30, 2020 and 2019, respectively.

Estimated amortization expense for the years ending June 30 for the next five years and thereafter is summarized in the table below.

In thousands	
2021	$ 187
2022	170
2023	83
2024	50
2025	42
Thereafter	543
	$1,075

8. Debt

On June 30, 2020, the Company entered into a Loan and Security Agreement (the "Loan Agreement") with several banks and other financial institutions or entities from time to time parties to the Loan Agreement (collectively, referred to as the "Lenders") and Hercules Capital, Inc., in its capacity as administrative agent and collateral agent for itself and the Lenders (in such capacity, the "Agent").

The Loan Agreement provides for a term loan in an aggregate principal amount of up to $25.0 million to be delivered in multiple tranches (the "Term Loan"). The tranches consist of (i) a term loan advance of $10.0 million on June 30, 2020 (the "Closing Date") and (ii) subject to the Lenders' investment committee's sole discretion, the Company has the right to request that the Lenders make additional term loan advances in an aggregate principal amount of up to $15.0 million prior to January 1, 2022 or, if certain conditions are satisfied, then July 1, 2022. There can be no assurances that any Term Loan advances will be funded by the Lenders in the future.

In connection with entering into the Loan Agreement, the Company paid an aggregate of $165,000 to the Lenders for an initial facility charge, due diligence fees and reimbursement of legal expenses. Such amount was recorded as either a debt discount or a deferred financing fee, both of which reduce the carrying value of the outstanding Term Loan.

The Term Loan matures on December 1, 2023; provided that, in the event that the Company meets certain conditions, including achievement of performance milestones, then the Term Loan matures on June 1, 2024. The date on which the Term Loan matures (i.e., either December 1, 2023 or June 1, 2024) is referred to as the "Term Loan Maturity Date."

The Term Loan bears interest at a rate equal to the greater of either: (i) the sum of (x) the prime rate as reported in *The Wall Street Journal* minus 3.25% and (y) 9.75%; or (ii) 9.75%. Borrowings under the Loan Agreement are being repaid in monthly interest-only payments from the Closing Date through December 31, 2021, with the possibility to extend that date until June 30, 2022 upon the Company's achievement of certain performance milestones. After the interest-only period ends, borrowings under the Loan Agreement will be repaid in equal monthly installments of principal and interest until the Term Loan Maturity Date.

The Company may, at its option, prepay all, but not less than all, of the outstanding Term Loan balance plus all accrued and unpaid interest thereon, together with a prepayment charge equal to: (i) 3.0% of the amount so prepaid if such prepayment occurs during the first 12 months following the Closing Date; (ii) 2.0% of the amount so prepaid if such prepayment occurs after 12 months but prior to 24 months from the Closing Date; (iii) 1.0% of the amount so prepaid if such prepayment occurs after 24 months but prior to 36 months from the Closing Date; and (iv) zero percent of the amount so prepaid if such prepayment occurs three years or more after the Closing Date.

On the earliest to occur of the (i) Term Loan Maturity Date, (ii) date that the Company prepays the outstanding secured payment obligations in full or (iii) date that the secured payment obligations become due and payable, the Company will pay (in addition to any prepayment charge) an end of term charge of 6.95% of the aggregate term loan advances. Such charge was recorded as a discount to the carrying value of the outstanding Term Loan.

The Term Loan is secured by substantially all of the Company's assets, other than its intellectual property. However, the Company has agreed to not pledge or secure its intellectual property to others. In connection with granting security interests in its cash and cash equivalents and investments, the Company was required to enter into certain account control agreements with the Agent regarding future control of the underlying bank and securities accounts. Pursuant to the terms of the account control agreements, the Company's control of those accounts will not be affected unless the Agent elects to obtain unilateral control by declaring that an event of default under the Loan Agreement has occurred and is continuing.

During the term of the Loan Agreement, the Lenders or their assignee or nominee have the right to participate in any equity offerings by the Company that are broadly marketed to multiple investors, in an amount up to $2.0 million, on the same terms, conditions and pricing afforded to other investors participating in any such subsequent offering.

The Loan Agreement contains customary representations, warranties and both affirmative and negative covenants. The Loan Agreement requires that after July 1, 2021 and upon term loan advances exceeding $10.0 million, the Company will maintain minimum unrestricted cash of at least $5.0 million, plus the amount of the Company's accounts payable not paid after the 120th day following a vendor's invoice date, until the Company has a market capitalization equal to or greater than $300.0 million. The negative covenants in the Loan Agreement include, among other things, agreements by the Company limiting additional indebtedness, liens (including a negative pledge on intellectual property and other assets), guaranties, mergers and consolidations, substantial asset sales, investments and loans, certain corporate changes, transactions with affiliates, and fundamental changes. Additionally, the Company may not declare or pay any cash dividends during the term of the Loan Agreement. The Company was in full compliance with all covenants of the Loan Agreement as of June 30, 2020.

The Loan Agreement provides for events of default customary for term loans of this type, including, but not limited to, non-payment of interest or principal when due, breaches or defaults in the performance of covenants, insolvency, bankruptcy and the occurrence of an event that could have a material adverse effect on the Company. Upon the occurrence of an event of default that has not otherwise been remedied by the Company, the Agent may: (i) accelerate payment of all or any part of the secured obligations, impose a prepayment charge and terminate the Lenders' commitments under the Loan Agreement; (ii) sign and file in the Company's name any notices, assignment or agreements necessary to perfect payment; or (iii) notify any of the Company's debtors to make payment directly to such agent.

As of June 30, 2020, the carrying value of the Term Loan on the Company's balance sheet was $9.7 million, which consisted of the outstanding principal of such loan and the end of term charge accrual, less unamortized debt discounts and deferred financing fees of $1.0 million that are being amortized to interest expense over the duration of the Loan Agreement using an effective interest method. On such date, financing fees totaling $149,000 were unpaid and have been included in accrued and other liabilities on the Company's balance sheets. As of June 30, 2020, the variable contractual interest rate on the Term Loan was 9.75% per annum and the effective rate on the Term Loan is projected to be 13.53% through the Term Loan Maturity Date. Interest expense on the Term Loan during the year ended June 30, 2020 was nominal.

Future minimum principal payments of the outstanding Term Loan balance, excluding the end of term charge, are as follows: $2.3 million in the year ending June 30, 2022; $5.0 million in the year ending June 30, 2023; and $2.7 million in the year ending June 30, 2024.

9. Collaboration Agreements and Related Revenue Matters

Bionic Sight

On February 2, 2017, the Company entered into a strategic research and development collaboration agreement with Bionic Sight, LLC ("Bionic Sight") to develop therapies for patients with visual deficits and blindness due to retinal disease. Through the AGTC-Bionic Sight collaboration, the companies seek to develop a new optogenetic therapy that leverages AGTC's deep experience in gene therapy and ophthalmology and Bionic Sight's innovative neuro-prosthetic device and algorithm for retinal coding. The collaboration agreement grants to AGTC, subject to achievement by Bionic Sight of certain development milestones, an option to exclusively negotiate for a limited period of time to acquire: (i) a majority equity interest in Bionic Sight; (ii) the Bionic Sight assets to which the collaboration agreement relates; or (iii) an exclusive license with respect to the product to which the collaboration agreement relates.

Under the agreement, AGTC made an initial $2.0 million payment to Bionic Sight for an equity interest in that company. This initial investment represented an equity interest of approximately 5% in Bionic Sight. In addition to the initial investment, AGTC contributed ongoing research and development support costs through additional payments and other in-kind contributions (the "AGTC Ongoing R&D Support"). The AGTC Ongoing R&D Support payments and in-kind contributions were made over time and continued until December 2019, the month that Bionic Sight received both Investigational New Drug ("IND") clearance from the United States Food and Drug Administration ("FDA") and receipt of written approval from an internal review board to conduct clinical trials from at least one clinical site for that product candidate (the "IND Trigger"). Prior to the achievement of the IND Trigger, the Company had incurred approximately $2.2 million of research and development support costs and in-kind contributions, which were reported as research and development expense in the Company's financial statements.

Upon achievement of the IND Trigger, AGTC was (i) entitled to receive additional equity in Bionic Sight, based on the valuation in place at the beginning of the agreement, for the AGTC Ongoing R&D Support payments and in-kind contributions, and (ii) obligated to purchase additional equity in Bionic Sight for $4.0 million and receive such equity interest based on certain pre-determined valuation criteria. The Company made the $4.0 million payment to Bionic Sight in January 2020. Thereafter, AGTC is not obligated to purchase additional equity in Bionic Sight or make any additional in-kind contributions under the agreement.

The Company received the additional shares in Bionic Sight related to its $4.0 million investment and the conversion of the $2.2 million of in-kind contributions into equity in March 2020 upon the execution of a subscription agreement between the parties. The Company's equity interest in Bionic Sight increased to approximately 15.5% upon the issuance of the additional shares.

The Company concluded that the AGTC Ongoing R&D Support was within the scope of Topic 606 because the services rendered represented a distinct service delivered to a counterparty that meets the definition of a customer. The Company further concluded that those services represented one combined performance obligation. Because the consideration that the Company was entitled to was contingent upon achievement of the IND Trigger, that consideration was determined to be variable and the amount was fully constrained until achievement of the IND Trigger. As a result of achieving the IND Trigger in December 2019, the Company recognized $2.2 million of collaboration revenue during the year ended June 30, 2020. With regard to the obligation to purchase additional equity in Bionic Sight for $4.0 million, the Company concluded at contract inception that such option represented a forward contract to be accounted for within the scope of ASC 321, *Investments—Equity Securities*. The Company assessed the fair value of this forward contract at the inception of the Bionic Sight agreement and determined the value to be de minimis. As the forward contract did not have a readily determinable fair value, the Company elected to use a measurement alternative for all subsequent measurements of the financial instrument. Under such measurement alternative, the forward contract was remeasured at fair value when observable transactions involving the underlying equity securities or impairment of those securities occurred. As noted above, the Company made a supplemental investment of $4.0 million in Bionic Sight and the underlying equity interests were delivered in March 2020, resulting in the settlement of the forward contract at

that time. From the inception of the Bionic Sight arrangement and through the settlement date in March 2020, no observable transactions or impairment involving the underlying equity securities had occurred.

The Company recorded its initial $2.0 million investment in Bionic Sight using the equity method of accounting for investments. Upon receipt of the additional shares in March 2020, the Company concluded that equity method accounting was still appropriate for the Company's investment in Bionic Sight. Given that the conversion price used to calculate the number of additional shares that the Company was to receive was based on contractually fixed valuation amounts, the Company assessed whether there was a difference between the cost of the investment and the underlying equity in the net assets of Bionic Sight. The Company concluded that any such difference was not material to the Company's financial statements and, therefore, recorded its additional investment in Bionic Sight at $6.2 million. For the years ended June 30, 2020 and 2019, the Company recorded equity in net losses of an affiliate of $47,000 and $35,000, respectively, in its Statements of Operations to reflect its equity interest in the net losses of Bionic Sight.

Otonomy, Inc.

During October 2019, the Company entered into a strategic collaboration agreement with Otonomy, Inc. ("Otonomy") to co-develop and co-commercialize an AAV-based gene therapy to restore hearing in patients with sensorineural hearing loss caused by a mutation in the gap junction protein beta 2 gene (GJB2) – the most common cause of congenital hearing loss. Mutations in GJB2 account for approximately 30% of all genetic hearing loss cases. Patients with this mutation can have severe-to-profound deafness in both ears that is identified in screening tests routinely performed on newborns. Under the collaboration agreement, the parties began equally sharing the program costs and proceeds, if any, in January 2020 and can include additional genetic hearing loss targets in the future.

The Company has concluded that the Otonomy collaboration agreement is within the scope of Topic 808, which defines collaborative arrangements and addresses the presentation of transactions between the two parties in the income statement and related disclosures. However, Topic 808 does not provide guidance on the recognition of consideration exchanged or accounting for the obligations that may arise between the parties. The Company concluded that ASC Topic 730, *Research and Development,* should be applied by analogy to payments between the parties during the development activities. As such, payments made to or received from Otonomy for development activities are recorded as research and development expenses. For the year ended June 30, 2020, settlement activity between the parties under the Otonomy agreement had an immaterial effect on the Company's research and development expenses.

Biogen

Background

On July 1, 2015, the Company entered into a collaboration agreement with Biogen, pursuant to which the Company and Biogen collaborated to develop, seek regulatory approval for and commercialize gene therapy products to treat XLRS, XLRP and discovery programs targeting three indications based on the Company's adeno-associated virus vector technologies. Effective March 8, 2019, Biogen terminated the collaboration agreement. Upon termination, the Company received back the exclusive license rights to develop, manufacture and commercialize the product candidates for all of its partnered programs, including the XLRP program, XLRS program and the three discovery programs. The Company recognized revenue as it performed under the Biogen collaboration agreement; however, subsequent to the date of termination, no additional revenue has been, or will be, recognized.

Under the Biogen collaboration agreement, the Company granted to Biogen with respect to the XLRS and XLRP programs, and upon exercise of an option for the applicable discovery program, an exclusive, royalty-bearing license, with the right to grant sublicenses, to use adeno-associated virus vector technology and other technology controlled by the Company for the licensed products or discovery programs developed under the collaboration

agreement. Biogen and the Company also granted each other worldwide licenses, with the right to grant sublicenses, of their respective interests in other intellectual property developed under the collaboration outside the licensed products or discovery programs. Biogen pre-funded the Company to conduct all development activities through the completion of a first in human trial for the XLRS program and all development activities through the date of Investigational New Drug Application and the completion of a natural history study for the XLRP program. In addition, Biogen pre-funded the Company to conduct discovery, research and development activities for additional drug candidates through the stage of clinical candidate designation for discovery programs targeting three indications (of which one indication had two development plans at contract inception), after which, Biogen had an option to continue to develop, seek regulatory approval for and commercialize the designated clinical candidate. The pre-funded research and development activities for each program are referred to as "Pre-Funded Activities."

Pursuant to a related manufacturing agreement, Biogen had an option to receive a manufacturing license for up to six genes for a fixed fee per gene elected. If exercised, the Company would have been eligible to receive certain event milestones and royalties.

Under the Biogen collaboration agreement, the Company was paid an upfront nonrefundable fee of $94.0 million, including $58.4 million that was contractually described as relating to the Pre-Funded Activities, and $35.6 million that was contractually described as relating to the access of licenses. In addition, under the terms of a related equity agreement, Biogen purchased 1,453,957 shares of the Company's common stock for an aggregate cash purchase price of $30.0 million, of which $10.8 million was considered to be allocated consideration as part of the Biogen collaboration agreement. Additionally, under the Biogen collaboration agreement, the Company was also eligible to receive payments based on the successful achievement of certain milestones. Prior to the termination of the collaboration agreement, the Company triggered total milestone payments of $17.5 million, including a $10.0 million payment in July 2018 related to the treatment of a first patient of second cohort in a Phase 1/2 Clinical XLRP study.

Accounting Analysis

The Company concluded that the Biogen collaboration agreement, manufacturing agreement and equity agreement should be accounted for as one arrangement because those agreements were with the same party and were negotiated and executed contemporaneously. The Company further concluded that certain goods and services promised under such collaboration agreement for consideration were consistent with a vendor/customer relationship and should be accounted for under Topic 606. The associated performance obligations and allocated transaction prices upon the initial application of Topic 606 on July 1, 2018 are summarized in the table below.

In thousands	
XLRS License and Pre-Funded Activities	$ 52,060
XLRP License and Pre-Funded Activities	43,570
Pre-Funded Activities associated with the discovery programs	16,700
Total Biogen transaction price	$ 112,330

The amount allocated to the Pre-Funded Activities associated with the discovery programs was comprised of four distinct performance obligations based on the separate development plans for discovery candidates at contract inception. The Company concluded that the delivered license was not distinct from the Pre-Funded Activities as Biogen could not obtain the benefit of the license without the related services. Further, each of the license and related Pre-Funded Activities performance obligation was considered a distinct performance obligation as each development plan was pursued independent of every other development plan.

Subsequent to July 1, 2018, the abovementioned milestone payment related to XLRP under the Biogen collaboration agreement increased the total Biogen transaction price by $10.0 million in July 2018.

Notwithstanding the timing difference between the Company's upfront receipt of consideration and the transfer of goods and services to Biogen, the Company concluded that there was not a significant financing component to the Biogen agreements.

The Company concluded that "Post-Funded Activities" represented customer options that were not material rights because any services requested by Biogen and provided by the Company were reimbursed at a rate that reflected the estimated stand-alone selling price for the services. As such, the Company recognized revenue related to Post-Funded Activities as the services were provided.

The Company concluded that the option to receive (i) commercial licenses for any discovery programs that achieved clinical candidate designation, as defined in the Biogen collaboration agreement, and (ii) manufacturing licenses for up to six genes pursuant to the manufacturing agreement represented customer options that were not material rights because the exercise prices for such options reflected the estimated stand-alone selling prices for the underlying performance obligation. As such, the Company only accounted for such options when they were exercised.

The Company used the most-likely method to determine the amount of variable consideration in the Biogen collaboration agreement. The Company concluded that any estimated amount of variable consideration related to clinical and regulatory milestone payments should be fully constrained as the achievement of such milestones was highly susceptible to factors outside of the Company's control. The Company further concluded that any commercial milestones and sales-based royalties would be recognized when the related sales occurred as they were deemed to relate predominately to the license granted and, therefore, were also excluded from the transaction price.

The total Biogen transaction price was allocated to the performance obligations based on the relative estimated stand-alone selling price of each performance obligation or, in the case of certain variable consideration, to one or more performance obligations. The estimated stand-alone selling prices for performance obligations, that included a license and Pre-Funded Activities, were developed using the estimated selling price of the license and an estimate of the overall effort to perform the Pre-Funded Activities. The estimated selling price of the licenses were determined using a discounted cash flow valuation utilizing forecasted revenue and costs for the Company's product candidate licenses.

The Company recognized revenue related to the performance obligations that included a license and Pre-Funded Activities over the estimated period of the research and development services using a proportional performance model. The Company measured proportional performance based on the costs incurred relative to the total costs expected to be incurred to satisfy the performance obligation. Management believes that recognizing revenue on a proportional performance basis based on costs incurred faithfully depicts the transfer of goods and services to the customer because the customer consumed the Company's services as such services were performed. The Company accounted for the termination of the Biogen collaboration agreement upon the effective date of the termination and updated its total costs incurred to satisfy the performance obligations.

The table below summarizes the Company's revenue related to the Biogen collaborative agreement for the year ended June 30, 2019. No Biogen collaboration revenue was recognized during the year ended June 30, 2020.

In thousands	
Collaboration revenue	
Licenses and related services	$ 27,000
Development services	2,736
Milestone revenue	11,392
Total collaboration revenue	$ 41,128

Licenses and related services revenue is comprised of revenue related to the Company's completion of performance obligations that include the delivery of licenses and Pre-Funded Activities. Development services

revenue relates to the delivery of Post-Funded Activities. Milestone revenue relates to the portion of milestone payments received that are recognized as revenue based on the proportional performance of the underlying performance obligation and revenue recognized due to the termination of the Biogen collaboration agreement. Among other things, the Company recognized the then outstanding deferred revenue balance upon the effective termination date of the Biogen collaboration agreement.

Summary of Contract Assets and Liabilities

The table below summarizes changes in the balances of our contract assets and liabilities during the year ended June 30, 2019.

In thousands	June 30, 2018	Additions	Deletions	June 30, 2019
Contract assets	$ —	$ —	$ —	$ —
Contract liabilities:				
Deferred revenue	$ 29,521	$ 10,000	$ (39,521)	$ —

The Company increased deferred revenue and accumulated deficit by $22.6 million as of July 1, 2018 in connection with the adoption of Topic 606. The impact of adopting Topic 606 is reflected in the balance as of June 30, 2018 in the above table. Contract liability additions during the year ended June 30, 2019 consisted of a $10.0 million milestone payment received under the Biogen collaboration agreement related to the Company's XLRP program. For the year ended June 30, 2019, the Company recognized revenue of $29.5 million related to deferred revenue that existed as of June 30, 2018.

As of June 30, 2020, accrued and other liabilities on the Company's balance sheets included $149,000 of deferred revenue. Management is unable to estimate when the Company will satisfy the performance obligations pertaining to such deferred revenue.

10. Share-Based Compensation Plans

The Company uses stock options, performance service awards, restricted stock awards and restricted stock units to provide long-term incentives to its employees, non-employee directors and certain consultants. The Company has two equity compensation plans under which awards are currently authorized for issuance: the 2013 Employee Stock Purchase Plan and the 2013 Equity and Incentive Plan. No awards have been issued to date under the 2013 Employee Stock Purchase Plan and, as such, all of the 128,571 shares previously authorized under that plan remain available for issuance. As of June 30, 2020, the total number of shares available for issuance under the 2013 Equity and Incentive Plan was 1,297,930. See Note 14 in these Notes to Financial Statements for an increase in the shares available for issuance under such plan subsequent to June 30, 2020. Currently, the Company issues new shares upon the exercise, release or settlement of share-based compensation awards.

The Compensation Committee of the Board of Directors, as the plan administrator, has the authority to select the individuals to whom share-based awards are granted and to determine the terms of each award, including: (i) the number of shares of common stock subject to a stock option or restricted stock award; (ii) the date on which a stock option becomes exercisable; (iii) the option exercise price, which, in the case of incentive stock options, must be at least 100% (110% in the case of incentive stock options granted to a stockholder owning in excess of 10% of the Company's stock) of the fair market value of the common stock on the date of grant; (iv) the vesting term; and (v) the duration of an option (which, in the case of incentive stock options, may not exceed ten years). Employee options typically vest over a three- or four-year period.

Information about the Company's stock options that do not have performance conditions is provided below.

	Year Ended June 30,			
	2020		2019	
(In thousands, except per share amounts)	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Outstanding, beginning of the year	3,585	$ 9.19	3,107	$ 10.93
Granted	1,219	3.28	1,172	4.49
Exercised	(100)	3.94	(65)	2.40
Forfeited	(474)	3.82	(317)	7.17
Expired	(384)	12.06	(312)	12.44
Outstanding, end of the year	3,846	$ 7.82	3,585	$ 9.19
Exercisable, end of the year	2,418		2,167	
Weighted average fair value of options granted during the year	$ 2.10		$ 2.89	

The intrinsic value of stock options exercised during the years ended June 30, 2020 and 2019 was $0.3 million and $0.2 million, respectively. The total fair value of stock options that vested during the years ended June 30, 2020 and 2019 was $2.8 million and $3.9 million, respectively.

The table below summarizes information about stock options (i) exercisable and (ii) vested and expected to vest as of June 30, 2020.

(In thousands, except per share amounts)	Shares	Weighted Average Exercise Price	Aggregate Intrinsic Value	Weighted Average Contractual Life (in years)
Vested and expected to vest	3,720	$ 7.96	$ 4,156	6.80
Exercisable	2,418	10.15	1,912	5.76

The aggregate intrinsic value presented in the above table was calculated as the difference between the exercise price of the underlying options and the fair value of the Company's common stock on June 30, 2020 for the options that were in the money.

In addition to the stock option activity described above, the Company also granted 100,000 performance-based stock options to a senior officer during the year ended June 30, 2020 with an exercise price of $3.91. That award: (i) was issued under the 2013 Equity and Incentive Plan; (ii) has a term of ten years; and (iii) includes six separate tranches with performance criteria that will each vest 25% upon their achievement, with the remaining 75% of the tranche vesting on a monthly basis over a period of three years subsequent to achieving the underlying performance objective (assuming continued service by the awardee). Each tranche represents one-sixth of the total award. If any of the performance criteria are not satisfied, that tranche will be forfeited. As of June 30, 2020, one of the six performance criteria has been met. The Company used the Black-Scholes stock option pricing model to estimate the grant date fair value of each option to be $2.58; however, determining the appropriate periodic share-based compensation expense for this award requires management to estimate the likelihood of the achievement of the performance targets.

The Company granted 2,000 and 24,000 restricted stock awards to a senior officer during the years ended June 30, 2020 and 2019, respectively. Those awards were fully vested on the date of grant. The weighted average market prices of such restricted stock awards on the date of grant were $3.75 and $4.54 for the years ended June 30, 2020 and 2019, respectively.

During the year ended June 30, 2020, 175,500 restricted stock units, which included a market-based vesting condition related to the trading price of our common stock, were granted to certain of the Company's employees under the 2013 Equity and Incentive Plan with a weighted average grant date fair value of $2.56. As of June 30, 2020, none of the restricted stock units had vested; however, the market condition embedded in the award had been met and 22,500 awards have been forfeited. On August 15, 2020, 76,500 restricted stock units vested and, assuming continuing service by the grantees, the remaining outstanding restricted stock units will vest on August 15, 2021. The fair value of each restricted stock unit awarded was estimated on the grant date using a Monte Carlo simulation pricing model, which incorporated the probability of satisfying the related market-based vesting condition.

Share-based compensation expense pertaining to stock options awarded to employees, nonemployee directors and consultants totaled $2.5 million and $3.9 million for the years ended June 30, 2020 and 2019, respectively. Share-based compensation expense pertaining to restricted stock awards and restricted stock units awarded to employees and consultants totaled $0.5 million and $0.1 million for the years ended June 30, 2020 and 2019, respectively.

The table below presents the allocation of total share-based expense for the years indicated.

	Year Ended June 30,	
In thousands	2020	2019
Research and development	$ 1,451	$ 1,895
General and administrative	1,547	2,134
Totals	$ 2,998	$ 4,029

The fair value of each stock option granted is estimated on the date of grant using the Black-Scholes stock option pricing model. Below are the assumptions that were used when estimating fair value for the years indicated.

	Year Ended June 30,	
Assumption	2020	2019
Dividend yield	0.00%	0.00%
Expected term	6.00 to 6.25 years	6.00 to 6.25 years
Risk-free interest rate	0.42% to 1.90%	1.82% to 3.11%
Expected volatility	71.20%	69.22%

The dividend yield assumes that the Company will not declare dividends over the lives of the options. Since adopting ASC Topic 718, *Compensation—Stock Compensation,* the Company has been unable to use historical employee exercise and option expiration data to estimate the expected term for the Black-Scholes grant-date valuation. Therefore, the Company has utilized the "simplified" method, as prescribed by SEC Staff Accounting Bulletin No. 107, *Share-Based Payment*, to estimate, on a formula basis, the expected term of the Company's stock options that are considered to have "plain vanilla" characteristics. The risk-free interest rate is based on the U.S. Treasury yield curve on the date of valuation with a maturity similar to the expected life of the award. Expected volatility is based on the historical volatility of the Company's stock price. Forfeitures are estimated at the time of the grant and revised, if necessary, in subsequent periods if actual forfeitures differ from those estimates. Share-based compensation expense recognized in the Company's Statements of Operations does not reflect tax-related effects due to the Company's historical and anticipated operating losses and offsetting changes in its valuation allowance that fully reserve against any deferred tax assets.

Unrecognized share-based compensation cost related to non-vested employee stock options that do not have performance conditions totaled $3.4 million as of June 30, 2020. Such compensation cost is expected to be expensed over a weighted average period of approximately 2.5 years. As of June 30, 2020, unrecognized share-based compensation cost for the Company's performance-based stock options and restricted stock units were $22,000 and $135,000, respectively. Such costs will be expensed over 2.1 years and 1.1 years, respectively.

11. Income Taxes

The table below summarizes the Company's provision for income taxes for the years indicated.

In thousands	Year Ended June 30, 2020	2019
Current tax expense:		
Federal	$—	$—
State	83	76
Total current tax expense	83	76
Deferred tax expense:		
Federal	—	—
State	—	—
Total deferred tax expense	—	—
Provision for income taxes	$ 83	$ 76

Deferred taxes are recognized for temporary differences between the basis of assets and liabilities for financial statement and income tax purposes. The table below summarizes the significant components of the Company's deferred tax assets (liabilities).

In thousands	June 30, 2020	2019
Deferred tax assets:		
Net operating loss carryforwards	$ 30,077	$ 20,526
Tax credit carryforwards	29,557	25,501
Lease liabilities - operating	1,314	—
Accruals and other	3,644	3,513
Depreciation and amortization	317	238
Gross deferred tax assets	64,909	49,778
Deferred tax asset valuation allowance	(64,032)	(49,778)
Total deferred tax assets, net of valuation allowance	877	—
Deferred tax liabilities:		
Right-of-use assets - operating leases	(877)	—
Total deferred tax liabilities	(877)	—
Net deferred tax asset (liability)	$ —	$ —

As of June 30, 2020, the Company had federal and state net operating losses of approximately $24.8 million and $5.8 million (tax effected), respectively, that may be applied against future taxable income and expire in various years ranging from 2022 to 2039 and federal net operating losses of $96.5 million that do not expire. As of June 30, 2020, the Company also had federal and state research and development tax credits of approximately $28.9 million and $0.9 million, respectively, which may provide future tax benefits and expire in various years ranging from 2027 to 2049.

The Company evaluated the positive and negative evidence bearing on the realizability of its deferred tax assets. Based on its history of operating losses, the Company concluded that, as of both June 30, 2020 and 2019, it was more likely than not that the benefits of its deferred tax assets would not be realized. Therefore, any tax benefits to be realized in future years as a result of the utilization of the Company's net operating loss and tax credit carryforwards, computed based on statutory federal and state rates, were completely offset by valuation

allowances on those dates. The Company's valuation allowances increased by $14.3 million and $7.6 million during the years ended June 30, 2020 and 2019, respectively, due primarily to net increases in federal net operating losses and, in 2019, equity adjustments as a result of adopting Topic 606 and its impact on deferred revenue.

On March 27, 2020, the Coronavirus Aid, Relief, and Economic Security ("CARES") Act was signed into law. The CARES Act contains several significant provisions that affect corporations, including, among others, provisions addressing the use of net operating losses, interest deduction limitations and employer payroll tax payments (see Note 12 in these Notes to Financial Statements for further discussion of employer payroll taxes). Management does not believe that the CARES Act will have a material impact on the Company; however, management will continue to monitor ongoing developments, new regulations and interpretive guidance regarding such legislation and evaluate any potential impact on the Company's overall business and tax position.

The items comprising the differences between the U.S. federal statutory income tax rate and the Company's effective tax rate on the loss before provision for income taxes for the years indicated are summarized in the table below.

	Year Ended June 30,	
	2020	2019
Federal income tax benefit at statutory rate	21%	21%
State income taxes, net of federal benefit	4	(3)
Permanent differences-incentive share-based compensation	(1)	(27)
Permanent differences-transportation and travel	—	(9)
Research and development tax credits	8	100
Rate change	—	14
Other	—	(9)
Change in valuation allowance	(32)	(91)
Effective income tax rate	0%	(4)%

Under the provisions of the Internal Revenue Code of 1986, as amended (the "Code"), the Company's net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. Net operating loss and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interests of significant stockholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Code, respectively, as well as similar state provisions. This circumstance could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of any annual limitation would be determined based on the value of the Company immediately prior to the ownership change. Since its inception, the Company has completed several financings and sales of its common stock, which have resulted in a change in control as defined by Sections 382 and 383 of the Code. Subsequent ownership changes may further affect such limitation in future years. A full valuation allowance has been provided against the Company's net operating loss and tax credit carryforwards and, if an adjustment were to be required, such an adjustment would reduce both the gross deferred tax asset established for the net operating loss and tax credit carryforwards and the valuation allowance.

Through the year ended June 30, 2020, the Company generated research and development credits but has not conducted a study to document the qualified activities. Such a study may result in an adjustment to the Company's research and development tax credit carryforwards; however, until a study is completed and an adjustment, if any, is known, no amounts are being presented as an uncertain tax position as of June 30, 2020 or 2019. A full valuation allowance has been provided against the Company's research and development tax credit carryforwards and, if an adjustment were to be required, such an adjustment would reduce both the gross deferred tax asset established for the research and development tax credit carryforwards and the valuation allowance.

The Company files income tax returns in the United States and in multiple states. The Company's federal and state returns are generally subject to tax examination for the tax years ended June 30, 2016 through June 30, 2020. To the extent that the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service or state authorities if such attributes are utilized by the Company in a future period.

The Company recognizes a tax benefit from an uncertain tax position when it is more likely than not that the position will be sustained upon examination. For the years ended June 30, 2020 and 2019, the Company increased its uncertain tax position reserve by $83,000 and $76,000, respectively, which amounts were entirely attributable to estimated interest and penalties in both years. The Company's practice is to recognize interest and penalties related to uncertain tax positions in its provision for income taxes. As of June 30, 2020 and 2019, the Company's aggregate reserve for uncertain tax positions was $2.1 million and $2.0 million, respectively. Included in those amounts were aggregate interest and penalties of $507,000 and $424,000 at June 30, 2020 and 2019, respectively. For each of the years ended June 30, 2020 and 2019, the Company's gross unrecognized tax benefits, excluding interest and penalties, was unchanged at $1.6 million. If recognized, the entire amount of the uncertain tax position liability at June 30, 2020 would reduce the Company's annual effective tax rate. It is reasonably possible that the Company's gross unrecognized tax benefits as of June 30, 2020, which relate to certain state tax matters, will decline by approximately $1.6 million within the next twelve months due to the expiration of certain statutes of limitations. Any such decline would also affect the then-outstanding balance of accrued interest and penalties. The Company's liability for uncertain tax positions is included in other long-term liabilities on its balance sheets.

12. Accrued and Other Liabilities

Accrued expenses and other liabilities by functional category are summarized in the table below as of the dates indicated.

	June 30,	
In thousands	2020	2019
Research and development-related	$ 6,715	$4,909
Compensation-related	3,298	2,406
General and administrative-related	489	709
Total accrued and other liabilities	$10,502	$8,024

As of June 30, 2020, federal payroll taxes totaling $177,000 have been deferred by the Company pursuant to the CARES Act and such amount is projected to be paid in equal installments on each of December 31, 2021 and 2022. This liability is included in other long-term liabilities on the Company's balance sheets.

13. Defined Contribution Plan

The Company sponsors an employee 401(k) salary deferral plan (the "401(k) Plan") that covers substantially all of its employees and is administered through a staff leasing company. Under the 401(k) Plan, employees may elect to defer up to 25% of their compensation per year (subject to a maximum limit prescribed by federal tax law) and the Company matches a portion of such employee contributions up to a maximum of 4% of eligible salary. The Company's matching contribution expense totaled $324,000 and $327,000 during the years ended June 30, 2020 and 2019, respectively.

14. Common Stock, Preferred Stock and Stockholders' Equity

As of June 30, 2020, there were 150 million shares of $0.001 par value common stock and five million shares of $0.001 par value preferred stock that were authorized to be issued. As of that date, a total of 25,813,185 and 25,793,589 shares of common stock were issued and outstanding, respectively, while none of the preferred shares were issued and outstanding.

The table below summarizes the shares of common stock that were reserved for future issuance as of June 30, 2020.

Stock options issued and outstanding, including those with performance milestones	3,945,870
Restricted stock units	153,000
Authorized for future grant under the 2013 Employee Stock Purchase Plan	128,571
Authorized for future grant under the 2013 Equity and Incentive Plan	1,297,930
Total shares of common stock reserved for future issuance	5,525,371

The number of shares of common stock available for issuance under the 2013 Equity and Incentive Plan is subject to an automatic annual increase on each July 1 equal to the lesser of (i) 4% of the number of shares of common stock issued and outstanding on the immediately preceding June 30 or (ii) such lesser number of shares of common stock as determined by the Company's Compensation Committee. Based on the Company's issued and outstanding common shares on June 30, 2020, the number of shares of common stock reserved and available for issuance under the 2013 Equity and Incentive Plan increased by 1,031,743 shares on July 1, 2020.

15. Commitments and Contingencies

License and Other Agreements

Under various agreements, the Company will be required to pay royalties and milestone payments upon the successful development and commercialization of products. The Company has entered into funding agreements with various not-for-profit organizations. The Company may become obligated to pay royalties on net product sales of any collaboration product that it successfully develops and subsequently commercializes or, if it out-licenses rights to a collaboration product, a specified percentage of certain payments it receives from its licensee. The Company is not obligated to make such payments unless and until annual sales of a collaboration product exceed a designated threshold. The Company's obligation to make such payments would end upon its payment of a specified amount.

The Company is also party to various agreements entered into in the ordinary course of business, principally relating to licensed technology. At June 30, 2020, the Company had four license agreements with three different entities, including three with the University of Florida Research Foundation, wherein the Company is responsible for all costs related to the preparation, filing, issuance, prosecution and maintenance of the underlying patents covered in the license agreements. Additionally, the Company is required to pay minimum annual royalty and license maintenance for those licenses until such time when the license is terminated by either expiration of the underlying patents or voluntary termination by either party per the agreement.

The license agreements also require future payments related to milestones or royalties on future sales of specified products. Payments under these agreements generally become due and payable only upon achievement of certain developmental, regulatory or commercial milestones. Amounts related to contingent milestone payments are not considered contractual obligations because they are contingent on the successful achievement of certain development, regulatory and commercial milestones. There is uncertainty regarding the various activities and outcomes needed to reach these milestones and, as such, they may not be achieved. The Company may terminate its license agreements with zero to ninety days written notice depending on the terms of each specific agreement.

The Company enters into standard indemnification agreements in the ordinary course of business. Pursuant to these agreements, the Company indemnifies, holds harmless and agrees to reimburse the indemnified party for losses suffered or incurred by the indemnified party, generally the Company's business partners or customers, in

connection with any U.S. patent or copyright or other intellectual property infringement claim by any third party with respect to the Company's products. The terms of these indemnification agreements are generally perpetual. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is unlimited. The Company has never incurred costs to defend lawsuits or settle claims related to these indemnification agreements.

COVID-19 Pandemic

On January 30, 2020, the World Health Organization ("WHO") announced a global health emergency because of a new strain of coronavirus originating in Wuhan, China ("COVID-19") and the risks to the international community as the virus spreads globally beyond its point of origin. In March 2020, the WHO classified the COVID-19 outbreak as a pandemic based on the rapid increase in exposure globally. National, state and local governments in affected regions have implemented, and may continue to implement, safety precautions, including quarantines, border closures, increased border controls, travel restrictions, shelter in place orders and shutdowns, business closures, cancellations of public gatherings and other measures. Organizations and individuals are taking additional steps to avoid or reduce infection, including limiting travel and staying home from work.

The worldwide spread of COVID-19 led to a global slowdown of economic activity and decreased demand for a broad variety of goods and services, while also disrupting sales channels and marketing activities and precipitating many corporate bankruptcy filings. As a result of the COVID-19 outbreak, the Company could experience delays in critical follow-up visits required under clinical trial protocols, which could increase the cost of those trials and also impact their expected timelines. Management's ability to fully interpret the trial outcomes and the ability of certain lab-based employees to perform their jobs due to stay-at-home orders or other restrictions related to COVID-19 could also result in delays and increase the Company's operating expenses. Furthermore, third-party vendors, such as raw material suppliers and contracted manufacturing, testing or research organizations, could also be impacted by COVID-19, which could result in unavoidable delays and/or increases in the Company's operating costs.

It is unknown how long the COVID-19 outbreak will continue before the virus is contained, the severity of the virus and the effectiveness of actions to contain and treat those who have contracted the virus. The extent to which the COVID-19 outbreak may impact the Company's financial condition, results of operations or cash flows is uncertain; however, as of the date of these financial statements, management is not aware of any specific event or circumstance that would require the Company to update its estimates, judgments or revise the carrying values of its assets or liabilities. Because future events are subject to change, management's best estimates and judgments may require future modification. Therefore, actual results could differ materially from current estimates. Management is closely monitoring the evolving impact of the pandemic on all aspects of the Company's business and periodically evaluates its estimates, which are adjusted prospectively based on such evaluations.

General

From time to time, the Company may be involved in other claims and legal actions that arise in the normal course of business. Management has no reason to believe that the outcome of any such legal actions would have a significant adverse effect on the Company's financial position, results of operations or cash flows.

16. Quarterly Financial Information (Unaudited)

The tables below summarize certain quarterly information of the Company for the years ended June 30, 2020 and 2019.

	Fiscal Year 2020 by Quarter			
In thousands, except per share data	First	Second	Third	Fourth
Revenue	$ —	$ 2,453	$ —	$ —
Loss from operations	(11,990)	(8,930)	(11,442)	(14,580)
Net loss	(11,577)	(8,623)	(11,189)	(14,503)
Net loss per common share, basic	$ (0.64)	$ (0.47)	$ (0.50)	$ (0.56)
Net loss per common share, diluted	(0.64)	(0.47)	(0.50)	(0.56)

	Fiscal Year 2019 by Quarter			
In thousands, except per share data	First	Second	Third	Fourth
Revenue	$14,034	$ 5,934	$21,318	$ 406
Income (loss) from operations	756	(4,671)	11,005	(11,440)
Net income (loss)	1,200	(4,181)	11,489	(10,514)
Net income (loss) per common share, basic	$ 0.07	$ (0.23)	$ 0.63	$ (0.58)
Net income (loss) per common share, diluted	0.07	(0.23)	0.63	(0.58)

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

None.

ITEM 9A. CONTROLS AND PROCEDURES

Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our reports under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), is recorded, processed, summarized and reported within the time periods specified in the rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure.

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15e and 15d-15e under the Exchange Act) as of the end of the period covered by this annual report. Based on this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that these disclosure controls and procedures were effective as of June 30, 2020.

Management's Report on Internal Control Over Financial Reporting

Management is responsible for establishing and maintaining adequate "internal control over financial reporting," as such term is defined under Rule 13a-15(f) of the Exchange Act. We maintain internal control over financial reporting designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with U.S. generally accepted accounting principles ("U.S. GAAP").

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Management assessed the effectiveness of the Company's internal control over financial reporting as of June 30, 2020. In making this assessment, management used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in *Internal Control—Integrated Framework (2013)*. Based on this assessment, management has concluded that the Company's internal control over financial reporting was effective as of June 30, 2020. As a non-accelerated filer, we are not required to comply with the auditor attestation requirement of Section 404 of the Sarbanes-Oxley Act of 2002.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15f and 15d-15f under the Exchange Act) identified in connection with the evaluation of our internal control performed during the quarter ended June 30, 2020 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

None.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The following biographical descriptions set forth certain information, as of September 18, 2020, with respect to our directors and executive officers who are not directors.

Name	Age	Position
Susan B. Washer	59	President, Chief Executive Officer and Director
William A. Sullivan	49	Chief Financial Officer
Stephen W. Potter	64	Vice President and Chief Business Officer
Mark S. Shearman, Ph.D.	58	Chief Scientific Officer
William Aliski, MPA (1)	73	Director
Ed Hurwitz (1)(2)	56	Director
Scott Koenig, M.D., Ph.D. (1)(3)	67	Chairman of the Board of Directors
Ivana Magovcevic-Liebisch, Ph.D. (2)(3)	53	Director
James Rosen (3)	51	Director
Anne VanLent (2)	72	Director

(1) Member of the Nominating and Corporate Governance Committee.
(2) Member of the Audit Committee.
(3) Member of the Compensation Committee.

Executive Officers

Susan B. Washer has served as our President and Chief Executive Officer since March 2002 and as a member of our board of directors since November 2003. Prior to becoming our President and Chief Executive Officer, Ms. Washer served as our Chief Operating Officer from October 2001 to March 2002. From August 1996 to October 2001, Ms. Washer was President and Chief Executive Officer of Scenic Productions Inc., a specialty construction firm providing sculpting, painting and construction services to the entertainment industry. From June 1994 to August 1996, Ms. Washer served as the Founding Executive Director and then Business Advisor for the North Florida Technology Innovation Center, a public-private organization financing and providing services to entrepreneurial companies licensing technology from Florida universities. From October

1983 to June 1994, Ms. Washer served in various research and pharmaceutical management positions with Abbott Laboratories and Eli Lilly and Company. Ms. Washer also served on the board of Histogenics Corporation from April 2018 to September 2019. Ms. Washer received a B.S. in biochemistry from Michigan State University and an M.B.A. from the University of Florida. We believe that Ms. Washer's education and professional background in science and business management, her years of experience in the pharmaceutical and biotechnology industries, her service as a senior executive of entrepreneurial companies and her extensive knowledge of our company and its business qualify her to serve as a member of our board of directors.

William A. Sullivan has served as our Chief Financial Officer since August 2017. Prior to joining AGTC, Mr. Sullivan worked at Merrimack Pharmaceuticals Inc. from November 2007 to April of 2017 where he held a number of positions of increasing responsibility including Controller, Vice President of Finance, Treasurer, Chief Financial Officer and Head of Finance. Mr. Sullivan began his career at Arthur Andersen LLP, where he obtained his certified public accountant license. Mr. Sullivan holds an M.B.A. and an M.S. in accounting from Northeastern University's Graduate School of Professional Accounting and a B.A. from Williams College.

Stephen W. Potter has served as our Vice President and Chief Business Officer since January 2015. Prior to joining us, Mr. Potter was employed most recently by NeoStem, Inc., a developer of cell-based therapeutics, where he served as Executive Vice President from July 2013 to February 2015, and was a member of the Board of Directors from January 2013 to July 2013. Previously, Mr. Potter was Senior Vice President of Operations and Corporate Development for Osiris Therapeutics, Inc., from February 2011 to November 2012, where he was part of the senior leadership team that achieved approval of the first-ever stem cell drug therapy, Prochymal. He was also responsible for the launch and overall management of the Bio-Surgery business unit as well as operational oversight for multiple functional areas including manufacturing, human resources, IT, legal, and business development. From 2006 through 2010, Mr. Potter served as Senior Vice President of Corporate and Business Development at Genzyme Corporation and as Vice President of Corporate and Business Development. While at Genzyme, he was the senior leader for its global corporate and business development team that provided strategic and transaction support, including support for many of Genzyme's gene and cell therapy opportunities. Mr. Potter has also held positions at DuPont Pharmaceuticals, E.I. Dupont de Nemours and Company, Inc., and Booz Allen & Hamilton. Mr. Potter earned a B.S. from University of Massachusetts and an MBA from Harvard Business School.

Mark S. Shearman has served as our Chief Scientific Officer since June 1, 2015. From August 2009 until June 2015, Dr. Shearman served as Senior Vice President of Research & Early Development of EMD Serono, Inc., the U.S. and Canadian subsidiary of Merck KGaA. Prior his time at EMD Serono, Dr. Shearman was Executive Director of Merck & Co. Research Laboratories, Boston, from January 2006 to July 2009 and Senior Director at the Merck Sharp & Dohme Research Laboratories Neuroscience Research Centre, U.K. from January 2004 to December 2005. Dr. Shearman earned a B.Sc. from the University of Bristol, a Ph.D. from the University of Nottingham and conducted academic research at institutes in Japan and Germany.

Directors

William Aliski has served as a member of our board of directors since September 2018. Mr. Aliski has served as a commercial consultant for early-stage orphan disease companies, including Ra Pharmaceuticals, Inc., from October 2016 to March 2017, and from March 2018 to present time, Clementia Pharmaceuticals, Inc., from December 2015 to January 2017, OxThera, from January 2015 through April 2015, Prosensa during 2014, Adimab LLC from November 2013 until December 2013, NPS Pharmaceuticals from April 2013 through December 2014, Fidelity Biosciences from August 2012 until December 2012 and Enobia Pharma from September 2011 until March 2012. Before that, Mr. Aliski served as Senior Vice President and Chief Commercial Officer of FoldRx Pharmaceuticals, a rare disease company, from June 2009 until March 2011, as Director of Simon Kucher Partners, a global consulting firm, from January 2008 until June 2009, and as General Manager of BioMarin Europe at BioMarin Pharmaceuticals Inc. from December 2005 until January 2008. Mr. Aliski also has served on the board of directors of Ultragenyx Pharmaceutical Inc. since January 2011.

Mr. Aliski received a B.S. in Economics and a Master of Social Planning from Boston College and an M.P.A. from the Kennedy School of Government at Harvard University. We believe that Mr. Aliski's extensive experience in the life sciences industry, membership on various boards of directors, and his leadership and management experience qualify him to serve as a member of our board of directors.

Ed Hurwitz has served as a member of our board of directors since November 2012. Mr. Hurwitz is a Managing Director of MPM Capital, a healthcare venture capital firm, and a Managing Director of Precision Bioventures, LLC, a consulting and investment advisory firm founded by Mr. Hurwitz. He was a director at Alta Partners from 2002 through December 2014. Mr. Hurwitz currently serves as Chairman of the board of directors of ViewPoint Therapeutics, Rekindle Therapeutics, and BioIntervene, Inc., as well as a board member of Dyne Therapeutics and Recode Therapeutics, all privately held, biotechnology companies. Mr. Hurwitz also serves as a member of the board of directors of MacroGenics, Inc., a publicly traded biotechnology company. Prior to joining Alta, Mr. Hurwitz served as Senior Vice President and CFO of Affymetrix from 1997 to 2002. From 1994 to 1997, Mr. Hurwitz was a biotechnology research analyst for Robertson Stephens & Company, and from 1992 to 1994, was a biotechnology research analyst for Smith Barney Shearson. From 1990 to 1992, he practiced commercial law at Cooley Godward LLP. Mr. Hurwitz earned a J.D. and M.B.A. from the University of California, Berkeley's Boalt School of Law and Haas School of Business, respectively. He also holds a B.A. in Molecular Biology from Cornell University. We believe that Mr. Hurwitz's education and professional background in science, business management and law, his work as a lawyer, research analyst and senior executive in the biotechnology industry and his experience as a director of other public and private biotechnology companies qualify him to serve as a member of our board of directors.

Scott Koenig, M.D., Ph.D. has served as a member of our board of directors since April 2002 and as chairman of our board of directors since April 2004. Dr. Koenig has served as the President and Chief Executive Officer and a director of MacroGenics, Inc., a publicly traded biopharmaceutical company, since September 2001 and was one of its co-founders. Prior to joining MacroGenics, Dr. Koenig served as Senior Vice President of Research at MedImmune Inc., a biopharmaceutical company, where he participated in the selection and maturation of its product pipeline. From 1984 to 1990, he worked in the Laboratory of Immunoregulation at the National Institute of Allergy and Infectious Diseases at the National Institutes of Health, or NIH, where he investigated the immune response to retroviruses and studied the pathogenesis of AIDS. Dr. Koenig currently serves on the board of directors of each of GlycoMimetics, Inc. (GLYC), The International Biomedical Research Alliance, and the Biotechnology Innovation Organization (BIO). Dr. Koenig received his A.B. and Ph.D. from Cornell University and his M.D. from the University of Texas Health Science Center in Houston. He completed his residency in Internal Medicine at the Hospital of the University of Pennsylvania, and is board certified in Internal Medicine and Allergy and Immunology. We believe that Dr. Koenig's education and professional background in science and medicine, his experience as Chief Executive Officer of MacroGenics and as a scientist and senior executive at other life science companies and research organizations and his service as a director of other biopharmaceutical companies, medical institutions and industry groups qualify him to serve as a member of our board of directors.

Ivana Magovcevic-Liebisch has served as a member of our board of directors since June 2014. Dr. Magovcevic-Liebisch has served as Chief Executive Officer and President of Vigil Neuroscience, a biotechnology company, since July 2020. Previously, Dr. Magovcevic-Liebisch served as Executive Vice President, Chief Business Officer for Ipsen, a biopharmaceutical company, from March 2018 to April 2020. Prior to that, Dr. Magovcevic-Liebisch served as Senior Vice President, Head of Global Business Development for Teva Pharmaceutical Industries Ltd., or Teva, a pharmaceutical company. Prior to joining Teva, Dr. Magovcevic-Liebisch held several senior positions within Dyax Corp., or Dyax, from April 2001 through March 2013, most recently serving as Executive Vice President and Chief Operating Officer. Prior to joining Dyax, Dr. Magovcevic-Liebisch was Director of Intellectual Property and Patent Counsel for Transkaryotic Therapies, Inc. from November 1999 until March 2001. Dr. Magovcevic-Liebisch received her J.D. from Suffolk University Law School and her Ph.D. in genetics from Harvard University. We believe that Dr. Magovcevic-Liebisch's

extensive experience in biopharmaceutical business development and operations qualify her to serve as a member of our board of directors.

James Rosen has served as a member of our board of directors since March 2010; he is currently President and CEO of Artizan Biosciences. Artizan Biosciences is engaging in early-stage immunobiology research and development for the treatment of unmet medical needs. From February 2015 through August 2016, Mr. Rosen served as Deputy Director, Venture Investing at the Bill & Melinda Gates Foundation. Prior to that, Mr. Rosen was a partner at Intersouth Partners, a venture capital firm, from January 2007 to December 2014. Prior to joining Intersouth, he spent 15 years in clinical, research and financial positions in the healthcare and biotechnology sectors, including serving as an equity research analyst at Brean Murray & Co., from 2000 to 2003, covering biopharmaceuticals, genomics, generics, drug delivery and medical device companies. Mr. Rosen holds a B.A. from Duke University, an M.B.A. from the University of North Carolina-Chapel Hill's Kenan-Flagler School of Business and an M.S.P.H. from the University of North Carolina School of Public Health. We believe that Mr. Rosen's education and professional background in science, business management and finance and his operational experience as a scientist and executive in the healthcare and biotechnology industries and as a venture capitalist concentrating on those industries, qualify him to serve as a member of our board of directors.

Anne VanLent has served as a member of our board of directors and chair of the audit committee since August 2016. Ms. VanLent is President of AMV Advisors, providing corporate strategy and financial consulting services to emerging growth life sciences companies. Ms. VanLent had been Executive Vice President and Chief Financial Officer of Barrier Therapeutics, Inc., a publicly traded pharmaceutical company, from May 2002 through April 2008. Ms. VanLent also worked for eight years as Senior Vice President and Chief Financial Officer of The Liposome Company, Inc., a publicly traded biopharmaceutical company. Ms. VanLent currently serves as a director, chair of the Audit Committee, and member of the Nominating and Governance Committee of Trevi Pharmaceuticals, Inc. Until June 2020, she also served as a director, chair of the Audit Committee and member of the Compensation Committee of Vaxart, Inc. as a result of its merger in February 2018 with Aviragen Therapeutics, Inc., where she served as lead director, chair of the Audit Committee and member of the Nominating and Governance Committee. From April 2011 to December 2017 she served as a director, chair of the Audit Committee, and chair of the Nominating and Governance Committee of Ocera Therapeutics, Inc. From April 2013 through June 2017 she served as a director, member of the Audit Committee, and member of the Compliance Committee of Novelion Pharmaceuticals, Inc. From July 2013 to May 2016, Ms. VanLent served as a director, chair of the Audit Committee, and member of the Compensation Committee of Onconova Therapeutics, Inc. and as a director of Integra Life Sciences Holdings, Inc. Ms. VanLent received a B.A. degree in Physics from Mount Holyoke College. Our Board of Directors believes that Ms. VanLent's qualifications to sit on our Board of Directors include her extensive leadership and finance experience, and her extensive experience serving as a board member, audit committee member and audit committee chair of public companies in the life sciences industry.

CORPORATE GOVERNANCE

Code of Business Conduct and Ethics; Corporate Governance Guidelines

We have adopted a written code of business conduct and ethics that applies to our directors, executive officers and employees, as well as corporate governance guidelines. Copies of the code of business conduct and ethics and our corporate governance guidelines are posted on the Corporate Governance section of our website, which is located at www.agtc.com. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website.

Audit Committee

The Company has a standing audit committee consisting of Ms. VanLent, its chairperson, Mr. Hurwitz and Dr. Magovcevic-Liebisch. Among other things, the audit committee assists our board of directors in its oversight

of: the integrity of our financial statements; our compliance with legal and regulatory requirements; the qualifications and independence of our independent registered public accounting firm; and the performance of our independent registered public accounting firm. Our board of directors has determined that each member of the audit committee satisfies the Nasdaq Stock Market independence standards and the independence standards of Rule 10A-3(b)(1) of the Securities Exchange Act. Each of the members of our audit committee meets the requirements for financial literacy under applicable rules and regulations of the SEC and the Nasdaq Stock Market. Our board of directors has also determined that Ms. VanLent qualifies as an "audit committee financial expert," as defined by applicable rules of the Nasdaq Stock Market and the SEC.

ITEM 11. EXECUTIVE COMPENSATION

The compensation of our executive officers is determined by the compensation committee of our board of directors, or the compensation committee, and discussed by the compensation committee throughout the year. Our formal annual compensation review process generally takes place during the first quarter of each fiscal year, after the results of the previous fiscal year are known. Annual variable compensation and discretionary cash bonuses for the completed fiscal year, if any, and long-term equity-based incentive compensation, if any, are awarded by the compensation committee on a discretionary basis, generally during the first fiscal quarter, after a review of the previous fiscal year's results.

Our compensation committee is comprised entirely of non-employee directors, each of whom our board of directors has determined is independent within the meaning of the rules of the Nasdaq Stock Market. The members of the compensation committee have substantial managerial experience and wide contacts in the biotechnology and biopharmaceutical industries and in the broader healthcare industry, upon which they rely in making their determinations. The compensation committee also considers publicly available information concerning the compensation practices of other companies in the biotechnology industry. This information is used by the compensation committee informally and primarily for purposes of comparison to ascertain whether our compensation practices for our executive officers are broadly competitive.

Our Chief Executive Officer makes recommendations with regard to the compensation of our executive officers other than herself, which are reviewed by the compensation committee. Executive officers do not participate in the process of establishing their own annual compensation.

The compensation committee does not have a formal benchmarking policy or a practice of establishing the amount of any element of our executive officers' compensation by reference to a fixed range of percentages or percentiles of the compensation of any peer or comparison group. As a result, the determinations made by the members of our compensation committee are guided to a significant degree by their collective judgment and experience. During fiscal year 2020, the compensation committee retained a compensation consultant, Aon Consulting's Radford Surveys + Consulting, or Radford, to assist the compensation committee in assessing the form and amount of compensation paid to our executives.

Our compensation committee has reviewed our compensation programs and believes that our compensation programs have not encouraged or rewarded excessive or inappropriate risk taking.

Summary Compensation Table for Fiscal Year 2020

The table below sets forth information regarding compensation earned by our named executive officers.

Name	Year	Salary ($)	Stock Awards ($)(1)	Option Awards ($)(2)	Non-equity incentive plan compensation ($)	Other ($)(3)	Total ($)
Susan B. Washer	2020	529,575	—	192,993	253,402	12,283	988,253
President and Chief Executive Officer	2019	514,150	—	274,957	254,504	8,621	1,052,232
Mark S. Shearman, Ph.D.	2020	414,160	100,035 (4)	100,357	186,372(5)	13,391	814,315
Chief Scientific Officer	2019	402,097	—	233,713	128,068	8,763	772,641
Stephen W. Potter	2020	363,448	100,035 (4)	100,357	111,815	11,509	687,164
Chief Business Officer							
Matthew Feinsod, M.D. (6)	2020	425,000 (7)	7,500	258,200	114,000	10,667	815,367
Former Interim Chief Medical Officer	2019	699,996	109,050	—	—	11,200	820,246

(1) Represents the fair value of restricted stock or restricted stock unit awards granted in fiscal years 2020 and 2019 in accordance with Accounting Standards Codification Topic 718, *Compensation—Stock Compensation* ("ASC 718"). See Note 10 of the notes to our financial statements included herein for a discussion of the relevant assumptions used in calculating these amounts.

(2) Represents the grant date fair value of option awards granted in fiscal years 2020 and 2019 in accordance with ASC 718. See Note 10 of the notes to our audited financial statements included herein for a discussion of the relevant assumptions used in calculating these amounts.

(3) Consists of 401(k) matching contributions.

(4) Represents restricted stock units granted to each of Dr. Shearman and Mr. Potter on August 15, 2020 for services as Chief Scientific Officer and Chief Business Officer, respectively.

(5) Includes $41,416 that vested on January 31, 2020 pursuant to the cash bonus granted to Dr. Shearman on January 6, 2020 for his services as Chief Scientific Officer.

(6) Effective August 1, 2019, Dr. Feinsod became our Executive Vice President of Global Strategy and Development and ceased to be an executive officer.

(7) From July 1, 2019 through his appointment as Executive Vice President of Global Strategy and Development effective as of August 1, 2019, Dr. Feinsod's annual base salary was $700,000. For the remainder of fiscal year 2020, Dr. Feinsod's annual base salary was $400,000.

Narrative Disclosure to Summary Compensation Table

We review compensation annually for all of our employees, including our executives. In setting executive base salaries and bonuses and granting equity incentive awards, we consider compensation for comparable positions in the market, the historical compensation levels of our executives, individual performance as compared to our expectations and objectives, our desire to motivate our employees to achieve short- and long-term results that are in the best interests of our stockholders, and a long-term commitment to our company. We do not target a specific competitive position or a specific mix of compensation among base salary, bonus or long-term incentives.

Our compensation committee reviews and discusses management's proposed compensation with the Chief Executive Officer for all executives other than our Chief Executive Officer. Based on those discussions and its discretion, the compensation committee then determines the compensation and benefits of our executive officers.

We have an at-will employment agreement with each of Ms. Washer, our President and Chief Executive Officer, Dr. Shearman, our Chief Scientific Officer, and Mr. Potter, our Chief Business Officer. We also have an at-will employment offer letter with Dr. Feinsod, our Executive Vice President of Global Strategy and Development, who served as our Interim Chief Medical Officer until August 1, 2019. For fiscal year 2020, the annual base salaries of Ms. Washer, Dr. Shearman, Mr. Potter and Dr. Feinsod were $529,575, $414,160, $363,448 and $425,000, respectively. In addition, Ms. Washer, Dr. Shearman, Mr. Potter and Dr. Feinsod received bonuses for fiscal year 2020 of $253,402, $144,956, $111,815 and $114,000, respectively, based on the achievement of certain corporate and individual performance goals as determined by the compensation committee. On January 6, 2020, the compensation committee of our board of directors approved an additional bonus payment of $165,664 for Dr. Shearman, of which (i) $41,416 vested on January 31, 2020 and became payable on April 30, 2020 and (ii) $124,248 will vest on December 31, 2020 and be payable on March 31, 2021, subject to Dr. Shearman's continued employment on each such date. For fiscal year 2021, the annual base salaries of Ms. Washer, Dr. Shearman, Mr. Potter and Dr. Feinsod were determined to be $529,575, $422,443, $370,717 and $406,000, respectively.

In fiscal year 2020, our compensation committee retained Radford to assist us with the identification of an appropriate peer group of companies for purposes of benchmarking the competitiveness of our executive compensation. Our compensation committee will evaluate the need for revisions to our executive compensation program to ensure that our program is competitive with the companies with which we compete for executive talent and that it is appropriate for a public company.

Retirement Savings

All of our full-time employees in the United States, including our named executive officers, are eligible to participate in our 401(k) plan. Pursuant to our 401(k) plan, employees may elect to reduce their current compensation by up to the statutorily prescribed annual limit (which is $19,500 in calendar year 2020 and was $19,000 in calendar year 2019), with additional salary deferrals not to exceed $6,500 and $6,000 in calendar years 2020 and 2019, respectively, available to those employees 50 years of age or older, and to have the amount of this reduction contributed to our 401(k) plan. The 401(k) plan permits us to make contributions up to the limits allowed by law on behalf of all eligible employees. Since July 1, 2017, we have been making matching contributions of 100% of the first 4% contributed by employees to our 401(k) plan.

Outstanding Equity Awards at Year End

The following table sets forth information regarding outstanding stock options and other equity awards held by our named executive officers as of June 30, 2020.

	Option Awards					Stock awards	
Name	Number of Securities Underlying Unexercised Options (#) exercisable	Number of Securities Underlying Unexercised Options (#) unexercisable	Option Exercise Price ($)	Option Expiration Date	Option Grant Date	Number of Shares or Units of Stock That Have Not Vested (#)	Market Value of Shares or Units of Stock That Have Not Vested ($)(1)
Susan B. Washer	3,877	—	3.50	11/1/2021	11/1/2011		
	120,686	—	0.35	1/6/2023	1/6/2013		
	140,000	—	4.90	9/18/2023	9/18/2013		
	99,156	—	14.08	4/17/2024	4/17/2014		
	55,780	—	16.00	7/31/2024	7/31/2014		
	80,000	—	18.48	7/21/2025	7/21/2015		
	78,333 (2)	1,667	15.57	7/7/2026	7/7/2016		
	72,916 (2)	27,084	4.90	7/17/2027	7/17/2017		
	47,916 (2)	52,084	4.25	7/25/2028	7/25/2018		
	20,833 (2)	79,167	3.00	8/15/2029	8/15/2019		
Mark S. Shearman, Ph.D.	110,000	—	19.50	6/1/2025	6/1/2015		
	51,000	—	18.48	7/21/2025	7/21/2015		
	48,958 (2)	1,042	15.57	7/7/2026	7/7/2016		
	36,458 (2)	13,542	4.90	7/17/2027	7/17/2017		
	40,729 (2)	44,271	4.25	7/25/2028	7/25/2018		
	10,833 (2)	41,167	3.00	8/15/2029	8/15/2019		
						39,000 (3)	216,060
Stephen W. Potter	131,049	—	24.62	1/29/2025	1/29/2015		
	51,000	—	18.48	7/21/2025	7/21/2015		
	48,958 (2)	1,042	15.57	7/7/2026	7/7/2016		
	36,458 (2)	13,542	4.90	7/17/2027	7/17/2017		
	40,729 (2)	44,271	4.25	7/25/2028	7/25/2018		
	10,833 (2)	41,167	3.00	8/15/2029	8/15/2019		
						39,000 (3)	216,060
Matthew Feinsod, M.D.	22,500	—	16.00	7/31/2024	7/31/2014		
	7,986 (4)	92,014	3.91	7/29/2029	7/29/2019		
						10,000 (5)	55,400

(1) The market values of the awards set forth in this table are based on the number of awards shown multiplied by the closing price of our common stock on June 30, 2020 ($5.54), as reported by the Nasdaq Global Market.

(2) This option becomes exercisable in equal monthly installments over four years from the date of grant.

(3) Represents restricted stock units granted on August 15, 2019 as part of our fiscal year 2020 annual equity awards, of which 50% vested on August 15, 2020 following the achievement of certain trading-based milestones, and the remaining 50% will vest on August 15, 2021 subject to continued employment.

(4) This option becomes exercisable based on the achievement of certain performance milestones. For each milestone met, the option vests as to 16,666.6667 shares of the our common stock in accordance with the following schedule: 25% of the underlying shares for such milestone vest on achievement of such milestone, and thereafter the remaining underlying shares for such milestone vest in equal monthly installments over

three years, resulting in the option being exercisable for 100% of the underlying shares for such milestone on the third anniversary of the achievement of the applicable milestone.

(5) Represents restricted stock awards granted on March 13, 2020 in connection with the achievement of certain milestones as outlined pursuant to an offer letter between us and Dr. Feinsod dated June 26, 2014, which vested in full on September 13, 2020.

Employment Agreements, Severance and Change in Control Arrangements Employment Agreements, Severance and Change in Control Arrangements

Agreement with Ms. Washer

Under the terms of Ms. Washer's employment agreement, if we terminate Ms. Washer's employment without "cause" or if she terminates her employment with us for "good reason" prior to a change of control or during the 12-month period following a "change of control," in each case as those terms are defined in her employment agreement, she will be entitled to receive severance benefits, payable in a single lump sum, as follows:

- An amount equal to the sum of (a) her then current annual base salary and (b) the product of her target bonus in effect immediately prior to the date of termination multiplied by a fraction equal to the quotient of (i) the number of days elapsed as of the termination date during the year in which the termination occurs divided by (ii) 365.
- Ms. Washer will also be entitled to continue to participate in our benefits plans for a period of up to 12 months following the effective date of the termination of her employment on substantially the same terms as were in effect immediately prior to her termination.
- In addition, if Ms. Washer's employment is terminated by us without cause or by Ms. Washer during the 12 months following a change of control for good reason, all unvested equity awards previously granted to her will become fully vested as of the date of the termination of her employment.
- In the event Ms. Washer terminates her employment for good reason other than during the 12-month period following a change of control, each unvested equity award previously granted to her will immediately vest with respect to 50% of the shares that are unvested as of the effective date of the termination of her employment.
- To the extent that the vesting of any unvested awards held by Ms. Washer at the time of the termination of her employment is contingent upon the attainment of any corporate or market performance condition that has not been satisfied as of that date, the condition will be deemed to have been satisfied as of the date of termination
    - at the 100% level, in the case of a termination by us without cause or by Ms. Washer during the 12 months following a change of control for good reason, or
    - at the 50% level, in the case of a termination by Ms. Washer for good reason other than during the 12 months following a change of control for good reason.

Agreements with Dr. Shearman and Mr. Potter

Pursuant to the terms of each of Dr. Shearman's and Mr. Potter's employment agreements, if we terminate Dr. Shearman's or Mr. Potter's, as applicable, employment without "cause" or if he terminates his employment with us for "good reason" prior to a change of control or during the 12-month period following a "change in control," in each case as those terms are defined in his employment agreement, he will be entitled to receive severance benefits, payable in a single lump sum, as follows:

- In the event of Dr. Shearman's or Mr. Potter's, as applicable, termination without cause or for good reason within 12 months of a change in control, an amount equal to the sum of (a) his then current annual base salary and (b) his target bonus in effect immediately prior to the date of termination.

- In the event of Dr. Shearman's or Mr. Potter's, as applicable, termination without cause (other than within 12 months of a change in control), an amount equal to the sum of (a) the product of his then current annual base salary multiplied by 0.75 and (b) the product of his target bonus in effect immediately prior to the date of termination multiplied by a fraction equal to the quotient of (i) the number of days elapsed as of the termination date during the year in which the termination occurs divided by (ii) 365.
- Dr. Shearman or Mr. Potter, as applicable, will also be entitled to continue to participate in our benefits plan for a period of up to (a) 12 months following termination in the event of Dr. Shearman's or Mr. Potter's, as applicable, termination without cause or for good reason, in either case, within 12 months following a change in control or (b) the earlier of nine months or until Dr. Shearman or Mr. Potter, as applicable, obtains other employment that provides the same type of benefit, if Dr. Shearman or Mr. Potter, as applicable, is terminated without cause (other than within 12 months of a change in control).
- In the event of Dr. Shearman's or Mr. Potter's, as applicable, termination for cause or good reason within 12 months of a change in control, Dr. Shearman's or Mr. Potter's, as applicable, options and other awards subject to vesting, including any award the vesting of which is contingent upon the attainment of any Company or market performance conditions, will immediately be deemed fully vested and exercisable.

Agreement with Dr. Feinsod

Pursuant to the terms of Dr. Feinsod's employment agreement, effective as of August 1, 2019, Dr. Feinsod will be eligible to receive certain severance benefits in connection with a termination of his employment by us without "cause" (as defined in Dr. Feinsod's employment agreement) or by Dr. Feinsod for "good reason" (as defined in Dr. Feinsod's employment agreement), in each case, subject to execution of a mutually acceptable release and settlement agreement. If such a termination occurs, he shall be entitled to receive nine months of his then current base salary, including the amount of any earned bonus. We will also continue to pay our portion of COBRA premiums for the nine-month period. Upon the occurrence of a "change of control" (as defined in Dr. Feinsod's employment agreement) in which Dr. Feinsod is not offered the position of Executive Vice President of Global Strategy and Development of the acquirer, all of Dr. Feinsod's options granted in connection with his appointment as Executive Vice President of Global Strategy and Development shall immediately be deemed fully vested and exercisable.

Director Compensation

Our non-employee directors receive equity-based compensation and cash fees as follows:

- each non-employee director receives an annual cash fee in the amount of $40,000;
- our chairman receives an additional cash fee in the amount of $35,000;
- the chairperson of each of our board committees receives an additional annual cash fee as follows: audit committee chair, $18,000; compensation committee chair, $12,000; and nominating and corporate governance committee chair, $8,000; and
- each other member of a board committee receives an additional annual cash fee as follows: audit committee, $9,000; compensation committee, $6,000; and nominating and corporate governance committee, $4,000.

The cash fees described above are paid quarterly in arrears. Non-employee directors are also reimbursed upon request for travel and other out-of-pocket expenses incurred in connection with their attendance at meetings of the board and of committees on which they serve.

Upon initial election to our board of directors, our non-employee directors are entitled to receive a non-qualified stock option, vesting in equal installments on each of the first three anniversaries of the date of grant, to purchase 25,000 shares of our common stock. In August 2020, the vesting of the initial option grants to directors was revised to one third on the first anniversary of the date of grant and thereafter in 24 equal monthly installments, so that the option is fully-vested on the third anniversary of the date of grant. In addition, each non-employee director remaining in office receives at each annual meeting of the stockholders a non-qualified stock option, vesting on the first anniversary of the date of grant, to purchase 10,000 shares of our common stock. In August 2020, the annual option award made to each non-employee director remaining in office at the annual meeting was increased to 12,000 shares of our common stock and the vesting revised so that the option vests in twelve equal monthly installments. Each such initial or annual stock option is granted with an exercise price equal to the fair value of our common stock on the date of grant.

The table below sets forth information regarding compensation awarded to, earned by or paid to our non-employee directors who served during fiscal year 2020. We do not pay any compensation to our President and Chief Executive Officer in connection with her service on our board of directors. See "Executive Compensation" for a discussion of the compensation of Ms. Washer.

Name	Fees earned or paid in cash ($)(1)	Option awards ($)(2)	Total ($)
Scott Koenig, M.D., Ph.D.	85,000	19,188	104,188
William Aliski, MPA	48,000	19,188	67,188
Ed Hurwitz	53,000	19,188	72,188
Ivana Magovcevic-Liebisch, Ph.D.	61,000	19,188	80,188
James Rosen	46,000	19,188	65,188
Anne VanLent	58,000	19,188	77,188

(1) Represents the amount earned or paid for service as a director during fiscal year 2020.
(2) Represents the grant date fair value of option awards granted in fiscal year 2020 in accordance with ASC 718. See Note 10 of the notes to our audited financial statements included herein for a discussion of the relevant assumptions used in calculating these amounts.

The table below shows the aggregate number of option awards held by each of our current non-employee directors who was serving as of June 30, 2020.

Name	Number of Options Outstanding at June 30, 2020
Scott Koenig, M.D., Ph.D.	72,833
William Aliski, MPA	45,000
Ed Hurwitz	58,263
Ivana Magovcevic-Liebisch, Ph.D.	58,263
James Rosen	58,263
Anne VanLent	54,000

Compensation Committee Interlocks and Insider Participation

The members of our compensation committee for fiscal year 2020 were Dr. Koenig, Dr. Magovcevic-Liebisch and Mr. Rosen. None of our executive officers serves, or in the past has served, as a member of the board of directors or compensation committee, or other committee serving an equivalent function, of any entity that has one or more executive officers who serve as members of our board of directors or our compensation committee. None of the members of our compensation committee is an officer or employee of our company, nor has any of them ever been an officer or employee of our company.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

The table below sets forth certain information with respect to beneficial ownership of our common stock as of September 2, 2020, by:

- each person or entity, or group of affiliated persons or entities, known by us to beneficially own more than 5 percent of our common stock;
- each of our directors and named executive officers; and
- all of our executive officers and directors as a group.

Beneficial ownership is determined in accordance with the rules of the SEC. In computing the number of shares beneficially owned by a person and the percentage ownership of that person, shares of common stock subject to options held by that person that are currently exercisable or exercisable within 60 days of September 2, 2020 are deemed outstanding, but are not deemed outstanding for computing the percentage ownership of any other person. To our knowledge, except as set forth in the footnotes to this table and subject to applicable community property laws, each person named in the table has sole voting and investment power with respect to the shares set forth opposite such person's name. Except as otherwise indicated, the address of each of the persons in this table is c/o Applied Genetic Technologies Corporation, 14193 NW 119th Terrace, Suite 10, Alachua, Florida 32615.

Each stockholder's percentage ownership is determined in accordance with Rule 13d-3 under the Exchange Act and is based on 25,857,883 shares of our common stock outstanding as of September 2, 2020. The number of outstanding shares beneficially owned by each stockholder below was obtained from the most recent publicly filed information, as applicable. Amounts under the heading "Right to Acquire" represent shares that may be acquired upon exercise of outstanding stock options exercisable within 60 days of September 2, 2020.

Name of Beneficial Owner	Shares Outstanding	Right to Acquire	Total	Percentage of Shares Outstanding
Stichting Aescap 2.0 (1)	1,805,761	—	1,805,761	7.0%
Integrated Core Strategies (US) LLC and affiliates (2)	1,511,561	—	1,511,561	5.8%
Entities affiliated with InterWest Partners (3)	1,455,904	—	1,455,904	5.6%
Susan B. Washer (4)	28,408	753,206	781,614	2.9%
Mark S. Shearman, Ph.D. (5)	13,777	316,686	330,463	1.3%
Stephen W. Potter (6)	15,177	337,735	352,912	1.3%
Matthew Feinsod, M.D. (7)	66,052	33,125	99,177	*
William Aliski, MPA (8)	8,500	26,666	35,166	*
Edward Hurwitz (9)	27,472	48,263	75,735	*
Scott Koenig, M.D., Ph.D. (10)	34,246	62,833	97,079	*
Ivana Magovcevic-Liebisch, Ph.D. (11)	3,000	48,263	51,263	*
James Rosen (12)	1,000	48,263	49,263	*
Anne VanLent (13)	—	44,000	44,000	*
All executive officers and directors (10 persons) (14)	134,580	1,843,789	1,978,369	7.1%

* Less than 1%

(1) This information is based on an amendment to Schedule 13G filed with the SEC on January 27, 2020 by Stichting Aescap 2.0, Privium Fund Management B.V., Inspirational Visions B.V. and Patrick Johan Hendrik Krol. Privium Fund Management B.V. is the fund manager of Stichting Aescap 2.0 and Patrick Johan Hendrik Krol is the portfolio manager of Privium Fund Management B.V. and Inspirational Visions B.V. Stichting Aescap 2.0 has sole voting and dispositive power with respect to all of the shares of our

common stock reported as beneficially owned by them and Inspirational Visions B.V. has sole voting and dispositive power with respect to all of the shares of our common stock reported as beneficially owned by them. The address of Inspirational Visions B.V. is Barbara Strozzilaan 101, 1083 HN Amsterdam, The Netherlands. The address of each of Stichting Aescap 2.0, Privium Fund Management B.V. and Patrick Johan Hendrik Krol is Gustav Mahlerplein 3, 1082 MS, Amsterdam, The Netherlands.

(2) This information is based on a Schedule 13G filed with the SEC on June 19, 2020 by Integrated Core Strategies (US) LLC, ICS Opportunities II LLC, ICS Opportunities, Ltd., Millennium International Management LP, Millennium Management LLC, Millennium Group Management LLC and Israel A. Englander. Millennium International Management LP is the investment manager to ICS Opportunities II LLC and ICS Opportunities, Ltd. Millennium Management LLC is the general partner of the managing member of Integrated Core Strategies (US) LLC and the general partner of the 100% owner of ICS Opportunities II LLC and ICS Opportunities, Ltd. Millennium Group Management LLC is the managing member of Millennium Management LLC and the general partner of Millennium International Management LP. The managing member of Millennium Group Management LLC is a trust of which Israel A. Englander serves as the sole voting trustee. Each of Millennium International Management LP, Millennium Management LLC, Millennium Group Management LLC and Israel A. Englander may be deemed to have shared voting and dispositive power over the shares held by Integrated Core Strategies (US) LLC, ICS Opportunities II LLC and ICS Opportunities, Ltd. The address for each of these entities and Mr. Englander is 666 Fifth Avenue, New York, New York 10103.

(3) This information is based on a Schedule 13G filed with the SEC on February 14, 2020 by InterWest Partners VIII, LP, InterWest Investors VIII, LP, InterWest Investors Q VIII, LP, InterWest Management Partners VIII, LLC, Gilbert H. Kliman and Arnold L. Oronsky. InterWest Management Partners VIII, LLC is the general partner of InterWest Partners VIII, LP, InterWest Investors VIII, LP, InterWest Investors Q VIII, LP, and has sole voting and investment control over the shares held by each of them. Gilbert H. Kliman and Arnold L. Oronsky are the managing directors of InterWest Management Partners VIII, LLC. Each of the managing directors share voting and dispositive power over the shares held by the entities affiliated with InterWest Partners and Mr. Oronsky has sole voting a dispositive power over 28,263 shares. The address for these entities is c/o InterWest Partners, 2710 Sand Hill Road, Suite 200, Menlo Park, California 94025.

(4) Excludes 295,293 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(5) Excludes 131,314 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table and 19,500 shares underlying restricted stock units that will vest on August 15, 2021.

(6) Excludes 131,314 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table and 19,500 shares underlying restricted stock units that will vest on August 15, 2021.

(7) Excludes 119,375 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(8) Excludes 18,334 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(9) Includes 1,800 shares held by the Hurwitz/Lichtenfeld Revocable Trust over which Mr. Hurwitz, as a trustee and a beneficiary, may be deemed to exercise voting and investment control. Excludes 10,000 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(10) Excludes 10,000 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(11) Excludes 10,000 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(12) Excludes 10,000 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(13) Excludes 10,000 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(14) Excludes 765,381 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table and 39,000 shares underlying restricted stock units that will vest on August 15, 2021.

We have two equity compensation plans under which awards are currently authorized for issuance, our 2013 Equity and Incentive Plan and our 2013 Employee Stock Purchase Plan. In connection with the consummation of our initial public offering in April 2014, our board of directors terminated any new offerings under our 2001 Stock Option Plan and our 2011 Stock Incentive Plan. Each of our 2013 Equity and Incentive Plan, our 2013 Employee Stock Purchase Plan, our 2001 Stock Option Plan and our 2011 Stock Incentive Plan was approved by our stockholders prior to our initial public offering in 2014. The table below provides information regarding securities authorized for issuance as of June 30, 2020 under our equity compensation plans.

Plan category	Number of securities to be issued upon exercise of outstanding options, warrants and rights, and vesting of outstanding restricted stock units (a)	Weighted average exercise price of outstanding options, warrants and rights (b)	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a)) (c)
Equity compensation plans approved by security holders	4,098,870(1)	$ 7.72(2)	1,426,501(3)
Equity compensation plans not approved by security holders	—	—	—
Total	4,098,870	$ 7.72	1,426,501

(1) Includes 153,000 shares to be issued upon completion of the vesting period for stock-settled restricted stock unit awards, subject to withholding to satisfy the minimum federal, state, local and/or payroll taxes of any kind required by law to be withheld with regard to the settlement of such awards.

(2) The calculation of the weighted average exercise price does not include outstanding restricted stock unit awards.

(3) Includes 1,297,930 shares issuable under our 2013 Equity and Incentive Plan, which may be issued in the form of options, restricted stock, unrestricted stock, performance share awards or other equity-based awards, and 128,571 shares issuable under our 2013 Employee Stock Purchase Plan. This number includes the automatic increase in shares to our 2013 Equity and Incentive Plan by its terms, added July 1 of each fiscal year and calculated as a 4% increase of the number of shares of our common stock issued and outstanding on the immediately preceding June 30 or such lesser number of shares of our common stock as determined by our compensation committee.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

Policies and Procedures for Related-Person Transactions

We have adopted a written related-person transactions policy that sets forth our policies and procedures regarding the identification, review, consideration, approval and oversight of "related-person transactions." For purposes of our policy only, a "related-person transaction" is a past, present or future transaction, arrangement or relationship (or any series of similar transactions, arrangements or relationships) in which we and any "related person" are participants involving an amount that exceeds $120,000.

Transactions involving compensation for services provided to us by an employee, consultant or director are not considered related-person transactions under this policy. A "related person," as determined since the beginning of our last fiscal year, is any executive officer, director or a holder of more than five percent of our common stock, including any of their immediate family members and any entity owned or controlled by such persons.

The policy imposes an affirmative duty upon each director and executive officer to identify any transaction involving them, their affiliates or immediate family members that may be considered a related party transaction before such person engages in the transaction. Under the policy, where a transaction has been identified as a related-person transaction, management must present information regarding the proposed related-person transaction to our audit committee (or, where review by our audit committee would be inappropriate, to another independent body of our board of directors) for review. The presentation must include a description of, among other things, the material facts, the direct and indirect interests of the related persons, the benefits of the transaction to us and whether any alternative transactions are available.

Our audit committee is responsible for reviewing and approving in advance any related-person transactions. In determining whether to approve a related-person transaction, the audit committee will take into account, among other factors it deems appropriate, whether the related-person transaction is on terms no less favorable than terms generally available to an unaffiliated third-person under the same or similar circumstances and the extent of the related person's interest in the transaction.

Director Independence

Our board of directors has determined that, with the exception of Ms. Washer, who is our employee, all of the members of our board of directors are "independent directors" under the applicable rules of the Nasdaq Stock Market. Our board of directors has also determined that each member of our audit committee, compensation committee and nominating and corporate governance committee is an "independent director" under the rules of the Nasdaq Stock Market applicable to such committees.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

Our audit committee engaged Ernst & Young LLP to serve as our independent registered public accounting firm for the fiscal year ended June 30, 2020. The selection of Ernst & Young LLP was ratified by our stockholders at the annual meeting of stockholders for fiscal year 2020.

Audit and Other Fees

The table below summarizes the fees for professional services rendered by Ernst & Young LLP, our independent registered public accounting firm, for fiscal years 2020 and 2019.

	Year Ended June 30,	
Fee category	2020	2019
Audit fees	$668,873	$493,709
Audit-related fees	—	—
Tax fees	—	—
All other fees	2,000	2,000
Total fees	$670,873	$495,709

Audit fees. Audit fees consist of fees and related expenses billed for professional services rendered for the audit of the financial statements and services that are normally provided by our independent registered public accounting firm in connection with statutory and regulatory filings or engagements and include fees for professional services rendered in connection with quarterly and annual reports. The audit fees for fiscal years 2020 and 2019 also include fees and related expenses associated with the issuance of consents by our independent registered public accounting firm to be named in our registration statements and to the use of their audit report in the registration statements.

Audit-related fees. Audit-related fees represent fees for assurance and related services performed by our independent registered public accounting firm that are reasonably related to the performance of the audit or

review of our financial statements, including consultation on accounting standards or accounting for specific transactions.

Tax fees. Tax fees represent fees for professional services performed by our independent registered public accounting firm with respect to tax compliance, tax advice and tax planning and related expenses. The Company engages a separate professional services firm for these services, including assistance with the preparation of federal, state and foreign income tax returns.

All other fees. All other fees represent fees for products and services provided by our independent registered public accounting firm, other than those disclosed above.

Pre-Approval Policies and Procedures

Our audit committee's pre-approval policies or procedures do not allow our management to engage our independent registered public accounting firm to provide any specified services without specific audit committee pre-approval of the engagement for those services. All of the services provided by our independent registered public accounting firm during fiscal year 2020 were pre-approved.

PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a) Documents filed as a part of this Report:

(1) Financial Statements—See Index to Financial Statements and Financial Statement Schedule at Item 8 on page 99 of this Annual Report on Form 10-K.

(2) Financial Statement Schedules—See Index to Financial Statements and Financial Statement Schedule at Item 8 on page 99 of this Annual Report on Form 10-K. All other schedules are omitted because they are not applicable or not required.

(3) Index to Exhibits.

Exhibit number	Description
3.1	Fifth Amended and Restated Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K filed with the SEC on April 1, 2014)
3.2	Amended and Restated Bylaws of the Company (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K filed with the SEC on April 1, 2014)
4.1	Specimen certificate evidencing shares of common stock (incorporated by reference to Exhibit 4.1 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
4.2^	Description of Securities Registered Pursuant to Section 12 of the Securities Exchange Act of 1934
10.1	Lease Agreement made as of April 10, 2015, by and between Alachua Foundation Park Holding Company, LLC and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.1 to the Company's Annual Report on Form 10-K for the year ended June 30, 2015 (File No. 001-36370))
10.2*	Employment Agreement dated as of August 29, 2019 between Applied Genetic Technologies Corporation and Mark S. Shearman (incorporated by reference to Exhibit 10.2 to the Company's Annual Report on Form 10-K for the year ended June 30, 2019 (File No. 001-36370))
10.3*	Employment Agreement dated as of August 29, 2019 between Applied Genetic Technologies Corporation and Stephen W. Potter (incorporated by reference to Exhibit 10.3 to the Company's Annual Report on Form 10-K for the year ended June 30, 2019 (File No. 001-36370))
10.4*	Employment Agreement dated as of September 26, 2014 between Applied Genetic Technologies Corporation and Susan B. Washer (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, event date September 26, 2014, filed on October 2, 2014 (File No. 001-36370))
10.5†	Collaboration and License Agreement dated as of July 1, 2015 by and between Biogen MA Inc., and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.5 to the Company's Annual Report on Form 10-K for the year ended June 30, 2018 (File No. 001-36370))
10.6†	Manufacturing License and Technology Transfer Agreement dated as of July 1, 2015 by and between Biogen MA Inc., and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.7 to the Company's Annual Report on Form 10-K for the year ended June 30, 2018 (File No. 001-36370))
10.7†	Second Amendment to Non-exclusive License Agreement, made and effective as of June 29, 2015, by and between The UAB Research Foundation, Inc. and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.10 to the Company's Annual Report on Form 10-K for the year ended June 30, 2015 (File No. 001-36370))

Exhibit number	Description
10.8†	Omnibus Amendment to Standard Exclusive License Agreement with Sublicensing Terms, made and effective as of July 1, 2015, by and between the University of Florida Research Foundation, Inc., the University of Florida Board of Trustees, John Hopkins University and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.11 to the Company's Annual Report on Form 10-K for the year ended June 30, 2015 (File No. 001-36370))
10.9†	Omnibus Amendment to Standard Exclusive License Agreement with Know How and Standard Non-Exclusive License Agreement, made and effective as of June 30, 2015, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.12 to the Company's Annual Report on Form 10-K for the year ended June 30, 2015 (File No. 001-36370))
10.10	Lease Agreement made as of September 19, 2011, by and between Thomson-Davis Enterprises, LLC and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.1 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.11†	Exclusive License Agreement with Sublicensing Terms, effective as of September 25, 2001, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.2 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.12†	Restated Amendment to License Agreement made and, effective as of January 31, 2005, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.3 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.13†	First Amendment After Restated Amendment to License Agreement, made and effective as of November 28, 2007, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.4 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.14†	Standard Exclusive License Agreement with Sublicensing Terms, effective as of October 7, 2003, by and between the University of Florida Research Foundation, Inc., Johns Hopkins University and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.5 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.15†	First Amendment to Standard Exclusive License Agreement with Sublicensing Terms, made as of November 2004, by and between the University of Florida Research Foundation, Inc., Johns Hopkins University and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.6 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.16†	Second Amendment to Standard Exclusive License Agreement with Sublicensing Terms, made as of February 25, 2009, by and among Applied Genetic Technologies Corporation, the University of Florida Research Foundation, Inc. and Johns Hopkins University (incorporated by reference to Exhibit 10.7 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.17†	Non-Exclusive License Agreement with Sublicensing Terms, made as of January 19, 2006, by and between The UAB Research Foundation, Inc. and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.8 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.18†	Standard Non-Exclusive License Agreement, effective as of September 18, 2012, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.10 to the Registrant's Registration Statement on Form S-1 (File No. 333-193309))

Exhibit number	Description
10.19†	Standard Exclusive License Agreement with Know How, effective as of November 5, 2012, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.11 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.20*	Applied Genetic Technologies Corporation 2001 Stock Option Plan, as amended (incorporated by reference to Exhibit 10.13 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.21*	Applied Genetic Technologies Corporation 2011 Stock Incentive Plan, as amended, and forms of Incentive Stock Option Agreement and Nonstatutory Stock Option Agreement thereunder (incorporated by reference to Exhibit 10.14 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.22*	Applied Genetic Technologies Corporation 2013 Equity And Incentive Plan (incorporated by reference to Exhibit 10.15 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.23*^	Form of Incentive Stock Option Agreement under the 2013 Equity and Incentive Plan
10.24*^	Form of Non-Statutory Stock Option Agreement under the 2013 Equity and Incentive Plan
10.25*^	Form of Restricted Stock Unit Agreement under the 2013 Equity and Incentive Plan
10.26*^	Form of Restricted Stock Agreement under the 2013 Equity and Incentive Plan
10.27*	Applied Genetic Technologies Corporation 2013 Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.16 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.28	Form of Indemnification Agreement for Directors Associated with an Investment Fund (incorporated by reference to Exhibit 10.23 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.29	Form of Indemnification Agreement for Directors Not Associated with an Investment Fund (incorporated by reference to Exhibit 10.24 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.30†	Second Amendment After Restated Amendment to License Agreement, made and effective as of January 10, 2014, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.25 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.31†	Fourth Amendment to Standard Exclusive License Agreement with Sublicensing Terms, made as of December 17, 2013 by and between the University of Florida Research Foundation, Inc., Johns Hopkins University and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.26 to the Company's Registration Statement on Form S-1 (File No. 333-193309))
10.32†	First Amendment to Non-Exclusive License, made as of March 28, 2014, by and between the UAB Research Foundation, Inc. and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.27 to the Company's Registration Statement on Form S-1 (File No. 333-197385))
10.33*	Employment Letter Agreement dated as of July 26, 2017 between Applied Genetic Technologies Corporation and William A. Sullivan (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed with the SEC on August 2, 2017 (File No. 001-36370))

Exhibit number	Description
10.34*	Employment Letter Agreement dated as of July 29, 2019 between Applied Genetic Technologies Corporation and Matthew Feinsod (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed with the SEC on August 2, 2019 (File No. 001-36370))
10.35*	Employment Agreement dated as of August 29, 2019 between Applied Genetic Technologies Corporation and Brian Krex (incorporated by reference to Exhibit 10.34 to the Company's Annual Report on Form 10-K for the year ended June 30, 2019 (File No. 001-36370))
10.36	Loan and Security Agreement, dated as of June 30, 2020, by and among Applied Genetic Technologies Corporation, the several banks and other financial institutions or entities from time to time parties to the Loan Agreement (collectively, referred to as the "Lenders"), and Hercules Capital, Inc., in its capacity as administrative agent and collateral agent for itself and Lenders (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed with the SEC on July 2, 2020 (File No. 001-36370))
23.1^	Consent of Ernst & Young LLP, Independent Registered Public Accounting Firm
31.1^	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2^	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1^	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.1^	The following interactive Data Files pursuant to Rule 405 of Regulation S-T, formatted in XBRL (eXtensible Business Reporting Language): (a) Balance Sheets as of June 30, 2020 and 2019; (b) Statements of Operations for the years ended June 30, 2020 and 2019; (c) Statements of Stockholders' Equity for the years ended June 30, 2020 and 2019; (d) Statements of Cash Flows for the years ended June 30, 2020 and 2019; and (e) Notes to such Financial Statements.

* Management contract or compensatory plan or arrangement
^ Filed herewith
† We have omitted portions of this exhibit, for which confidential treatment has been granted.

ITEM 16. FORM 10-K SUMMARY

None.

SCHEDULE II—VALUATION AND QUALIFYING ACCOUNTS

In thousands	Beginning of Year	Additions: Charge (Benefit) to Expense	Additions: To (From) Other Accounts	Deductions	End of Year
Deferred Tax Valuation Allowance					
Year ended June 30, 2020	$ 49,778	$ 14,254	$ —	$ —	$64,032
Year ended June 30, 2019	42,214	7,564	—	—	49,778

SIGNATURES

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

APPLIED GENETIC TECHNOLOGIES CORPORATION

By: /s/ Susan B. Washer
Susan B. Washer
President and Chief Executive Officer
Date: September 18, 2020

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

Signature	Title	Date
/s/ Susan B. Washer **Susan B. Washer**	Chief Executive Officer, President and Director (Principal Executive Officer)	September 18, 2020
/s/ William A. Sullivan **William A. Sullivan**	Chief Financial Officer (Principal Financial and Accounting Officer)	September 18, 2020
/s/ Scott Koenig **Scott Koenig**	Director	September 18, 2020
/s/ William Aliski **William Aliski**	Director	September 18, 2020
/s/ Ed Hurwitz **Ed Hurwitz**	Director	September 18, 2020
/s/ Ivana Magovcevic-Liebisch **Ivana Magovcevic-Liebisch**	Director	September 18, 2020
/s/ Anne VanLent **Anne VanLent**	Director	September 18, 2020
/s/ James Rosen **James Rosen**	Director	September 18, 2020

Exhibit 4.2

DESCRIPTION OF CAPITAL STOCK

The following description of the capital stock of Applied Genetic Technologies Corporation (the "Company," "we," "us," and "our") is qualified in its entirety by reference to our Fifth Amended and Restated Certificate of Incorporation (our "certificate of incorporation") and our Amended and Restated Bylaws (our "by-laws"), copies of which are incorporated by reference as exhibits to our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission, and applicable provisions of the Delaware General Corporation Law. We encourage you to read our certificate of incorporation, by-laws and the applicable provisions of the Delaware General Corporation Law for additional information.

Our authorized capital stock consists of 150,000,000 shares of common stock, $0.001 par value per share, and 5,000,000 shares of preferred stock, $0.001 par value per share.

Common Stock

Voting rights. Holders of our common stock are entitled to one vote per share held of record on all matters to be voted upon by our stockholders. The election of directors by our stockholders is determined by a plurality of the votes cast by the stockholders entitled to vote on the election. Other matters subject to a vote by our stockholders are decided by the affirmative vote of our stockholders having a majority in voting power of the votes cast by the stockholders present or represented and voting on such matter. Our common stock does not have cumulative voting rights.

Dividends. Subject to preferences that may be applicable to the holders of any outstanding shares of our preferred stock, the holders of our common stock are entitled to receive such lawful dividends as may be declared by our board of directors.

Liquidation and dissolution. In the event of our liquidation, dissolution or winding up, and subject to the rights of the holders of any outstanding shares of our preferred stock, the holders of shares of our common stock will be entitled to receive pro rata all of our remaining assets available for distribution to our stockholders.

Other rights and restrictions. Our certificate of incorporation does not permit us to redeem shares of our common stock at our election, provide for a sinking fund with respect to our common stock or provide for the granting of preemptive rights to any stockholder. All outstanding shares are fully paid and nonassessable.

Preferred Stock

Our board of directors is authorized, without stockholder approval, from time to time to issue up to 5,000,000 shares of preferred stock in one or more series, each of the series to have such rights and preferences, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, as the board of directors may determine. The rights of the holders of common stock will be subject to, and may be adversely affected by, the rights of holders of any preferred stock that we may issue in the future. The issuance of preferred

stock, while providing desirable flexibility in connection with possible acquisitions and other corporate purposes, could have the effect of making it more difficult for others to acquire, or of discouraging others from attempting to acquire, a majority of our outstanding voting stock.

Anti-Takeover Effects of Provisions of Delaware Law and Our Charter and By-laws

Provisions of Delaware law and our certificate of incorporation and by-laws could make it more difficult to acquire us by means of a tender offer, a proxy contest, open market purchases, removal of incumbent directors and otherwise. These provisions, summarized below, are expected to discourage types of coercive takeover practices and inadequate takeover bids and to encourage persons seeking to acquire control of us to first negotiate with us. We believe that the benefits of increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure us outweigh the disadvantages of discouraging takeover or acquisition proposals because negotiation of these proposals could result in an improvement of their terms.

We must comply with Section 203 of the Delaware General Corporation Law, an anti-takeover law. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a period of three years following the date the person became an interested stockholder, unless the business combination or the transaction in which the person became an interested stockholder is approved in a prescribed manner. Generally, a "business combination" includes a merger, asset or stock sale, or other transaction resulting in a financial benefit to an interested stockholder. An "interested stockholder" includes a person who, together with affiliates and associates, owns, or did own within three years before the determination of interested stockholder status, 15% or more of the corporation's voting stock. The existence of this provision generally will have an anti-takeover effect for transactions not approved in advance by the board of directors, including discouraging attempts that might result in a premium over the market price for the shares of common stock held by stockholders.

Our certificate of incorporation and by-laws require 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. In addition, special meetings of our stockholders may be called only by the board of directors and some of our officers. Our by-laws establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of candidates for election to our board of directors. Our certificate of incorporation and by-laws also provide for our board of directors to be divided into three classes, with each class serving staggered three-year terms. These provisions may have the effect of deterring hostile takeovers or delaying changes in our control or management.

Listing on the Nasdaq Global Market

Our common stock is listed on the Nasdaq Global Market under the symbol "AGTC."

Authorized but Unissued Shares

The authorized but unissued shares of common stock and preferred stock are available for future issuance without stockholder approval, subject to any limitations imposed by the Nasdaq Listing Rules. These additional shares may be used for a variety of corporate finance transactions, acquisitions and employee benefit plans. The existence of authorized but unissued and unreserved common stock and preferred stock could make it more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tender offer, merger or otherwise.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is Computershare Trust Company, N.A.

Exhibit 10.23

INCENTIVE STOCK OPTION

Granted by

Applied Genetic Technologies Corporation (the "Company")

Under the 2013 Equity and Incentive Plan

This Option is and shall be subject in every respect to the provisions of the Company's 2013 Equity and Incentive Plan, as amended from time to time (the "Plan"), which is incorporated herein by reference and made a part hereof. The holder of this Option (the "Holder") hereby accepts this Option subject to all the terms and provisions of the Plan and agrees that (a) in the event of any conflict between the terms hereof and those of the Plan, the latter shall prevail, and (b) all decisions under and interpretations of the Plan by the Board or the Committee shall be final, binding and conclusive upon the Holder and his or her heirs and legal representatives.

1. **Name of Holder:**

2. **Date of Grant:**

3. **Vesting Start Date:**

4. **Maximum number of shares for which this Option is exercisable:**

5. **Exercise (purchase) price per share:**

6. **Method of Exercise.** This Option may be exercised by the delivery of written notice to the Company setting forth the number of shares with respect to which the Option is to be exercised, together with payment by one of the following methods:

   cash, or certified or bank check or other instrument acceptable to the Administrator for an amount equal to the exercise price of the shares being purchased; or

   any of the other methods set forth in the Plan.

7. **Expiration Date of Option:**

8. **Vesting Schedule**:

9. **Termination of Employment.** This Option shall terminate on the earliest to occur of:

   (i) the date of expiration hereof;

   (ii) three (3) months following the Termination Date upon any termination other than for Disability or death; or

(ii) twelve (12) months following the Termination Date upon termination for Disability or death, or if the Holder dies within three (3) months after his or her Termination Date

10. **Incentive Stock Option; Disqualifying Disposition.** Although this Option is intended to qualify as an incentive stock option under the Internal Revenue Code of 1986 (the "Code"), the Company makes no representation as to the tax treatment upon exercise of this Option or sale or other disposition of the shares covered by this Option, and the Holder is advised to consult a personal tax advisor. Upon a Disqualifying Disposition of shares received upon exercise of this Option, the Holder will forfeit the favorable income tax treatment otherwise available with respect to the exercise of this Option. A "Disqualifying Disposition" shall have the meaning specified in Section 421(b) of the Code; as of the date of grant of this Option a Disqualifying Disposition is any disposition (including any sale) of such shares before the later of (a) the second anniversary of the date of grant of this Option and (b) the first anniversary of the date on which the Holder acquired such shares by exercising this Option, *provided* that such holding period requirements terminate upon the death of the Holder. The Holder shall notify the Company in writing immediately upon making a Disqualifying Disposition of any shares of Common Stock received pursuant to the exercise of this Option, and shall provide the Company with any information that the Company shall request concerning any such Disqualifying Disposition.

11. **Notice.** Any notice to be given to the Company hereunder shall be deemed sufficient if addressed to the Company and delivered to the office of the Company, Applied Genetic Technologies Corporation, 14193 NW 119th Terrace, Suite 10, Alachua, FL 32615, attention of the President and CEO, or such other address as the Company may hereafter designate.

Any notice to be given to the Holder hereunder shall be deemed sufficient if addressed to and delivered in person to the Holder at his or her address furnished to the Company or when deposited in the mail, postage prepaid, addressed to the Holder at such address.

IN WITNESS WHEREOF, the parties have executed this Option, or caused this Option to be executed, as of the Date of Grant.

Applied Genetic Technologies Corporation

By: _____

The undersigned Holder hereby acknowledges receipt of a copy of the Plan and this Option, and agrees to the terms of this Option and the Plan.

_____
Holder

Exhibit 10.24

NONSTATUTORY STOCK OPTION

Granted by

Applied Genetic Technologies Corporation (the "Company")

Under the 2013 Equity and Incentive Plan

This Option is and shall be subject in every respect to the provisions of the Company's 2013 Equity and Incentive Plan, as amended from time to time (the "Plan"), which is incorporated herein by reference and made a part hereof. The holder of this Option (the "Holder") hereby accepts this Option subject to all the terms and provisions of the Plan and agrees that (a) in the event of any conflict between the terms hereof and those of the Plan, the latter shall prevail, and (b) all decisions under and interpretations of the Plan by the Board or the Committee shall be final, binding and conclusive upon the Holder and his or her heirs and legal representatives.

1. **Name of Holder:**

2. **Date of Grant:**

3. **Vesting Start Date:**

4. **Maximum number of shares for which this Option is exercisable:**

5. **Exercise (purchase) price per share:**

6. **Method of Exercise.** This Option may be exercised by the delivery of written notice to the Company setting forth the number of shares with respect to which the Option is to be exercised, together with payment by one of the following methods:

   cash, or certified or bank check or other instrument acceptable to the Administrator for an amount equal to the exercise price of the shares being purchased; or

   any of the other methods set forth in the Plan.

7. **Expiration Date of Option:**

8. **Vesting Schedule:**

9. **Termination of Services.** This Option shall terminate on the earliest to occur of:

   (i) the date of expiration hereof;

   (ii) three (3) months following the Termination Date upon any termination other than for Disability or death; or

(ii) twelve (12) months following the Termination Date upon termination for Disability or death, or if the Holder dies within three (3) months after his or her Termination Date

10. **Tax Withholding.** The Company's obligation to deliver shares shall be subject to the Holder's satisfaction of any federal, state and local income and employment tax withholding requirements

11. **Notice.** Any notice to be given to the Company hereunder shall be deemed sufficient if addressed to the Company and delivered to the office of the Company, Applied Genetic Technologies Corporation, 14193 NW 119th Terrace, Suite 10, Alachua, FL 32615, attention of the President and CEO, or such other address as the Company may hereafter designate.

Any notice to be given to the Holder hereunder shall be deemed sufficient if addressed to and delivered in person to the Holder at his or her address furnished to the Company or when deposited in the mail, postage prepaid, addressed to the Holder at such address.

IN WITNESS WHEREOF, the parties have executed this Option, or caused this Option to be executed, as of the Date of Grant.

Applied Genetic Technologies Corporation

By: _____

The undersigned Holder hereby acknowledges receipt of a copy of the Plan and this Option, and agrees to the terms of this Option and the Plan.

_____
Holder

Exhibit 10.25

RESTRICTED STOCK UNIT AGREEMENT
Granted by

Applied Genetic Technologies Corporation

Under the 2013 Equity and Incentive Plan

Applied Genetic Technologies Corporation (the "Company") hereby grants to the person named below (the "Recipient") restricted stock units ("Restricted Stock Units"), with each such unit representing the right to receive one share of Stock, pursuant to the terms set forth below (the "Award"). The Award is and shall be subject in every respect to the provisions of the Company's 2013 Equity and Incentive Plan, as amended from time to time (the "Plan"), which is incorporated herein by reference and made a part hereof. The Recipient hereby accepts this Award subject to all the terms and provisions of the Plan and agrees that (a) in the event of any conflict between the terms hereof and those of the Plan, the latter shall prevail, and (b) all decisions under and interpretations of the Plan by the Board or the Committee shall be final, binding and conclusive upon the Recipient and his or her heirs and legal representatives. Capitalized terms used herein but not defined shall have the meaning set forth in the Plan.

1. **Name of Recipient:**

2. **Date of Grant:**

3. **Maximum Number of Restricted Stock Units:**

4. **Vesting of Restricted Stock Units:**

5. **Payment.** Upon each vesting date, the Recipient shall receive one share of Stock for each vested Restricted Stock Unit; provided, however, that the number of shares issued shall be reduced by the number of shares sufficient to satisfy the minimum tax withholding obligations as set forth in Section 6 below.

6. **Withholding.** Upon the settlement of Restricted Stock Units pursuant to Section 5 above, the Company shall withhold from issuance a number of shares sufficient to satisfy the minimum Federal, state, local and/or payroll taxes of any kind required by law to be withheld with regard to such settlement.

7. **No Rights to Shares or as a Stockholder.** The Recipient shall not have any right in, or with respect to, any of the shares of Stock issuable under the Award (including voting rights) unless and until the Award vests and is settled by issuance of the shares to the Recipient.

8. **Nontransferability.** The Restricted Stock Units are personal to the Recipient and shall not be transferable or assignable, other than by will or the laws of descent and distribution, and any such purported transfer or assignment shall be null and void.

9. **Termination of Employment.** If the Recipient's employment with or service for the Company is terminated, for any reason or no reason, with or without cause, all unvested Restricted Stock Units shall immediately terminate and be of no further force or effect.

10. **Notice.** Any notice to be given to the Company hereunder shall be deemed sufficient if addressed to the Company and delivered to the office of the Company, Applied Genetic Technologies Corporation, 14193 NW 119th Terrace, Alachua, FL 32615, attention of the chief financial officer, or such other address as the Company may hereafter designate.

Any notice to be given to the Recipient hereunder shall be deemed sufficient if addressed to and delivered in person to the Recipient at his or her address furnished to the Company or when deposited in the mail, postage prepaid, addressed to the Recipient at such address.

IN WITNESS WHEREOF, the parties have executed this Award, or caused this Award to be executed, as of the Date of Grant.

Applied Genetic Technologies Corporation

By: _____

The undersigned Recipient hereby acknowledges receipt of a copy of the Plan and this Award, and agrees to the terms of this Award and the Plan.

_____
[Name of Recipient]

Exhibit 10.26

APPLIED GENETIC TECHNOLOGIES CORPORATION

EMPLOYEE'S RESTRICTED STOCK AGREEMENT

1. **Restricted Stock Award**. Applied Genetic Technologies Corporation (the "**Company**") has granted to [●] (the "**Grantee**"), a restricted stock award (the "**Award**"), pursuant to the Company's 2013 Equity and Incentive Plan (the "**Plan**"), of [●] shares (the "**Shares**") of common stock, $0.001 par value ("**Common Stock**"), of the Company, subject to the terms and conditions of this Agreement and the Plan. Except where the context otherwise requires, the term "Company" shall include the parent and all present and future subsidiaries of the Company as defined in Sections 424(e) and 424(f) of the Internal Revenue Code of 1986, as amended or replaced from time to time (the "**Code**"). Capitalized terms used and not otherwise defined herein shall have the meanings ascribed to them in the Plan.

2. **Forfeitable Shares and Vested Shares**. All Shares shall be deemed to be "Forfeitable Shares" until the Company's right of forfeiture, described in Section 4, below, has expired (and the Grantee's right to retain such shares has accrued) in accordance with the vesting schedule set forth in Section 3. Forfeitable Shares shall be subject to forfeiture as described in Section 4, below. "**Vested Shares**" are Shares held by the Grantee as to which the Company's right of forfeiture has expired (and the Grantee's right to retain has accrued) based on the stock vesting schedule. All certificates representing Forfeitable Shares shall remain in the possession of the Company until such shares become Vested Shares in accordance with the terms of this Agreement.

3. **Vested Shares; Vesting Schedule**. The Company's right of Forfeiture shall expire and the Shares shall become Vested Shares in accordance with the following schedule:

4. **Forfeiture of Shares**.

4.1 **Forfeiture**. If for any reason the Grantee ceases to be employed by the Company (including, without limitation, by reason of the Grantee's voluntary resignation or the Company's dismissal of the Grantee for any reason, with or without cause) then all Shares which as of the date of such termination of employment constitute Forfeitable Shares shall be forfeited to the Company without payment of any consideration by the Company. There shall be no further accruals under the vesting schedule, and no further Forfeitable Shares shall become Vested Shares, from and after the date of any such termination of employment.

4.2 **Death or Disability**. The Committee shall have sole authority and discretion to determine whether in the event of the death or Disability of the Grantee, the vesting of the Shares under the Vesting Schedule would be accelerated so that all Shares become Vested Shares, effective as of the date of death or Disability.

4.3 **Forfeiture of Forfeitable Shares**. The Grantee's rights in all Forfeitable Shares shall terminate automatically on the date of the Grantee's termination of employment, and the Company may thereupon cancel the certificate or certificates representing such Forfeitable Shares on its books. In the event that the certificates then being retained by the Company under this Agreement also represent other shares of Common Stock not being forfeited to the Company, the Company shall issue to the Grantee replacement certificates for such other shares.

4.4 **Nontransferability of Shares**. No Shares may be transferred, assigned, pledged or hypothecated in any way (whether by operation of law or otherwise) or otherwise disposed of prior to their becoming Vested Shares. Upon any attempt to transfer, assign, pledge, hypothecate or otherwise dispose of any Forfeitable Shares, or upon the levy of any attachment or similar process upon Forfeitable Shares, the Company shall have a right of Forfeiture with respect to such Forfeitable Shares. Notwithstanding the foregoing, the Grantee may transfer any Shares either during his or her lifetime or on death by will or intestacy to one or more members of his or her immediate family or to a trust the beneficiaries of which are exclusively the undersigned and/or a member or members of his or her immediate family; provided, however, that prior to any such transfer each transferee shall execute an agreement, satisfactory to the Company, pursuant to which each transferee shall agree to receive and hold such Shares subject to the provisions hereof (including, without limitation, the Company's right of forfeiture with respect to any Shares so transferred that constitute Forfeitable Shares), and there shall be no further transfer except in accordance with the provisions hereof. For the purposes of this paragraph, "immediate family" shall mean spouse, lineal descendent, father, mother, brother or sister of the transferor.

5. **No Special Employment Rights**. Nothing contained in the Plan or this Agreement shall confer upon the Grantee any right with respect to the continuation of his or her employment by the Company or interfere in any way with the right of the Company at any time to terminate such employment or to increase or decrease the Grantee's compensation.

6. **Rights as a Shareholder**. The Grantee shall have the rights of a shareholder with respect to all of the Forfeitable Shares and the Vested Shares held by the Grantee (including, without limitation, any rights to vote and to receive dividends or non-cash distributions with respect to such shares) unless and until the Company exercises its right of Forfeiture as to any or all of the Forfeitable Shares in accordance with Section 4.

7. **Availability of Tax Election: Withholding**.

(a) Grantee acknowledges that the Company has advised the Grantee of the possibility of making an election under Section 83(b) of the Code with respect to the Award of the Shares and has recommended that the Grantee consult a qualified tax advisor regarding the desirability of making such an election in light of the Grantee's individual circumstances.

(b) Grantee shall, no later than the date as of which the value of any Shares first becomes includable in the gross income of the Grantee for Federal income tax purposes, pay to the Company, or make arrangements satisfactory to the Committee regarding payment of any Federal, state, local and/or payroll taxes of any kind required by law to be withheld with respect to such income. The Company and its Affiliates shall, to the extent permitted by law, have the right to deduct any such taxes from any payment of any kind otherwise due to the participant.

(c) Grantee may elect to have such tax withholding obligation satisfied, in whole or in part, by (i) authorizing the Company to withhold from the Vested Shares a number of shares with an aggregate Fair Market Value (as defined in the Plan, and determined of the date the withholding is effected) not greater than that which would satisfy the minimum statutory withholding amount due with respect to such Award, or (ii) delivering to the Company a number of Shares with an aggregate Fair Market Value (as of the date the withholding is effected) that would satisfy the statutory minimum withholding amount due. In the event that the amount of any such tax that is due with respect to such Award exceeds the statutory minimum amount, the Grantee shall be responsible for, and make provision for the timely payment of, any such excess amount.

8. **Miscellaneous**.

8.1 By accepting this Award, Grantee agrees that, if so requested by the Company or by the underwriters managing any underwritten offering of the Company's securities, the recipient will not, without the prior written consent of the Company or such underwriters, as the case may be, sell, make any short sale of, loan, grant any option for the purchase of, or otherwise dispose of any shares subject to any such Award during the Lock-up Period, as defined below. The "Lock-Up Period" shall mean a period of time not exceeding 180 days or, if greater, such number of days as shall have been agreed to by each director and executive officer of the Company in a substantially similar lock-up agreement by which each such director and executive officer is bound. If requested by the Company or such underwriters, the Grantee will enter into an agreement with such underwriters consistent with the foregoing.

8.2 Any certificate representing Shares shall be subject to a legend in substantially the following form:

"THE SHARES OF STOCK EVIDENCED BY THIS CERTIFICATE ARE SUBJECT TO AND ARE TRANSFERABLE ONLY IN ACCORDANCE WITH THAT CERTAIN RESTRICTED STOCK AGREEMENT DATED [●]. ANY ATTEMPTED TRANSFER OF THE SHARES OF STOCK EVIDENCED BY THIS CERTIFICATE IN VIOLATION OF SUCH AGREEMENT SHALL BE NULL AND VOID AND WITHOUT EFFECT. A COPY OF THE AGREEMENT MAY BE OBTAINED FREE OF CHARGE FROM THE SECRETARY OF THE COMPANY."

8.3 Grantee hereby agrees to execute and deliver to the Secretary of the Company a stock power (endorsed in blank) hereto covering this Award and authorizes the Secretary to deliver to the Company for cancellation any and all Shares that are forfeited or withheld under the provisions of this Agreement.

8.4 Except as provided herein, this Agreement may not be amended or otherwise modified unless evidenced in writing and signed by the Company and the Grantee.

8.5 All notices under this Agreement shall be mailed or delivered by hand to the parties at their respective addresses set forth beneath their names below or at such other address as may be designated in writing by either of the parties to one another.

8.6 This Agreement shall be governed by and construed in accordance with the laws of The Commonwealth of Massachusetts, without regard to its principles of conflicts of laws.

8.7 This Agreement is and shall be subject in every respect to the provisions of the Plan, as amended from time to time, which is incorporated herein by reference and made a part hereof.

8.8 This Agreement is executed in two (2) counterpart originals, one (1) to be retained by the Grantee and one (1) to be retained by the Company.

Date of Grant:

APPLIED GENETIC TECHNOLOGIES CORPORATION

By: _____

Title:

GRANTEE'S ACCEPTANCE

The undersigned hereby accepts the grant of the Restricted Stock Award described in this Agreement and agrees to the terms and conditions thereof. The undersigned hereby acknowledges receipt of a copy of the Company's 2013 Equity and Incentive Plan.

GRANTEE

Name:

Address:

Social Security Number:

STOCK POWER

FOR VALUE RECEIVED, the undersigned hereby sells, assigns and transfers to the Company a total of [●] shares of the Common Stock of the Company represented by stock certificate number [●] to be delivered herewith, and does hereby irrevocably constitute and appoint [●] as attorney to transfer said shares on the books of the Company with full power of substitution in the premises.

Dated: _____

_____

Name:

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

(1) Registration Statement (Form S-8 No. 333-198979) pertaining to the Applied Genetic Technologies Corporation 2001 Stock Option Plan, 2011 Stock Incentive Plan, 2013 Equity and Incentive Plan and the 2013 Employee Stock Purchase Plan,

(2) Registration Statement (Form S-3 No. 333-225286) of Applied Genetic Technologies Corporation, and

(3) Registration Statement (Form S-8 No. 333-233955) pertaining to the Applied Genetic Technologies Corporation 2013 Equity and Incentive Plan;

of our report dated September 18, 2020, with respect to the financial statements and financial statement schedule of Applied Genetic Technologies Corporation included in this Annual Report (Form 10-K) of Applied Genetic Technologies Corporation for the year ended June 30, 2020.

/s/ Ernst & Young LLP

Tampa, Florida
September 18, 2020

Exhibit 31.1

CERTIFICATION

I, Susan B. Washer, certify that:

1. I have reviewed this Annual Report on Form 10-K of Applied Genetic Technologies Corporation;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) 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.

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: September 18, 2020

By: /s/ Susan B. Washer
Susan B. Washer
Chief Executive Officer and President
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION

I, William A. Sullivan, certify that:

1. I have reviewed this Annual Report on Form 10-K of Applied Genetic Technologies Corporation;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) 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.

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: September 18, 2020

By: /s/ William A. Sullivan
William A. Sullivan
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of Applied Genetic Technologies Corporation (the "Company") for the year ended June 30, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers of the Company hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his or her knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: September 18, 2020

By: /s/ Susan B. Washer
Susan B. Washer
Chief Executive Officer and President
(Principal Executive Officer)

Date: September 18, 2020

By: /s/ William A. Sullivan
William A. Sullivan
Chief Financial Officer
(Principal Financial Officer)

A signed original of this written statement required by Section 906, or other document authenticating, acknowledging, or otherwise adopting the signature that appears in typed form within the electronic version of this written statement required by Section 906, has been provided to Applied Genetic Technologies Corporation and will be retained by Applied Genetic Technologies Corporation and furnished to the Securities and Exchange Commission or its staff upon request.