# 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, 2018**

**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** | **Name of exchange on which registered** |
|---|---|
| **Common Stock, $.001 par value** | **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 and posted on its corporate Web site, if any, 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 if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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 | ☐ (Do not check if a smaller reporting company) | 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 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 $64.9 million, computed by reference to the closing sale price of the common stock as reported by The Nasdaq Global Market on December 29, 2017, 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 August 31, 2018 was 18,129,148.

**DOCUMENTS INCORPORATED BY REFERENCE**

Certain information required to be provided in Part III of this Annual Report on Form 10-K will be provided by a definitive Proxy Statement for the registrant's Annual Meeting of Stockholders (the "Proxy Statement) to be filed with the Securities and Exchange Commission (the "SEC") on or before October 26, 2018.

**APPLIED GENETIC TECHNOLOGIES CORPORATION**
**ANNUAL REPORT ON FORM 10-K**
**FOR FISCAL YEAR ENDED JUNE 30, 2018**

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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, 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," "plan," "anticipate," "believe," "estimate," "predict," "intend," "potential," "might," "would," "continue" 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," or the "Company" refer to Applied Genetic Technologies Corporation.*

## PART I

### 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 retinoschisis (XLRS), X-linked retinitis pigmentosa (XLRP), and achromatopsia (ACHM) and a preclinical program in optogenetics. In addition to ophthalmology, we have initiated preclinical programs in adrenoleukodystrophy (ALD), which is a disease of the central nervous system (CNS) and several programs in otology. With a number of important clinical milestones on the horizon, we believe we are well positioned to advance multiple programs towards pivotal studies. 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, our collaboration with Biogen, which includes clinical programs in XLRS and XLRP and discovery programs in ALD and two ophthalmology indications, validates our approach and technology, and continues to provide us with cash runway to advance our wholly owned candidates.

**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 XLRS, ACHM, and XLRP. 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 XLRS, ACHM, XLRP, our previous experience in Leber's Congenital Amaurosis Type 2 (LCA2) and in preclinical ophthalmology programs we are developing in collaboration with Biogen.
  - **Seek opportunities for strategic partnerships and acquisitions in ophthalmology gene therapy.** On July 1, 2015, we entered into a broad collaboration and license agreement with Biogen to develop gene-based therapies for our XLRS and XLRP programs and three discovery programs. In February 2017, we entered into a collaboration

agreement with 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. 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 would 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 understanding of our target indications and our robust internal expertise in viral vector design including the identification of novel capsids and the optimization of genes and promoters, physical vector delivery, vector manufacturing, clinical trial design and clinical trial conduct are significant competitive advantages. We intend to continue to devote substantial resources both internally and with others, such as our external research collaborations with Synpromics and the University of Florida, to identify next generation capsids and to develop optimized promoters. We are also expanding our discovery capabilities to further enhance our ability to develop next generation products.

- **Expand our manufacturing capabilities.** We continue to invest in the development and expansion of our internal manufacturing capabilities. Our process development and pilot manufacturing facility is now operational, 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 and robust quality control enhancements that are amenable to characterization of commercial products. We believe 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 orphan indications with high unmet medical need and strong probability of a streamlined clinical, regulatory and commercial pathway.** We will continue to focus on diseases for which the underlying genetic defect is well characterized and can be addressed by correcting or inserting a single gene, for which predictive animal models exist and for which clinical endpoints are objective and are 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 each of these orphan diseases and the strong key opinion leader communities and patient advocacy groups around them, we also believe these markets can be served with a small, targeted commercial infrastructure. Our research in otology is one example of this strategy.

- **Evaluate opportunities to leverage our gene therapy platform to address indications outside of ophthalmology.** We intend to develop and partner selectively to expand the scope of our pipeline and the utilization of our gene therapy platform beyond ophthalmology. The adaptability and scalability of our platform also presents an opportunity for us to selectively form collaborative alliances to expand our capabilities and product candidate offerings into a range of genetically defined diseases and potentially to accelerate the development and commercialization of gene therapy products more broadly.

**Our Focus in Ophthalmology**

Sight is critical to the human experience. Many people fear blindness more than premature death. Consequently, we have 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 four ophthalmology development programs across three targets: XLRS caused by mutations in the RS1 gene, ACHM, caused by mutations in either the CNGB3 gene or the CNGA3 gene, and XLRP caused by mutations in the RPGR gene. These three inherited orphan diseases of the eye are caused by mutations in single genes that significantly affect visual function and currently lack effective medical treatments.

- XLRS is characterized by abnormal splitting of the layers of the retina, resulting in poor visual function in young boys, which can progress to legal blindness in adult men. Additionally, approximately 40% of patients are at risk of vitreous hemorrhage or detachment. Initial safety data from our Phase 1/2 clinical trial were presented in June 2017 and demonstrated that our product candidate was generally well tolerated across treatment groups. According to a published study, the incidence rate for XLRS is between one in 5,000 and one in 20,000 males. Using an incidence rate of 1 in 11,500, we estimate that there are about 35,000 patients in the United States and Europe combined. We have completed

target enrollment of 27 patients and are currently enrolling additional pediatric patients in a high dose group in the Phase 1/2 clinical trial for our XLRS product candidate.

- ACHM is characterized by the absence of cone photoreceptor function, resulting in extremely poor visual acuity, 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% have the form of disease caused by mutations in the CNGB3 gene or the CNGA3 gene. We are currently enrolling patients in Phase 1/2 clinical trials for both our ACHM CNGB3 product candidate and our ACHM CNGA3 product candidate.
- 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 are currently enrolling patients in Phase 1/2 clinical trials for our XLRP product candidate.

In addition to these clinical-stage ophthalmology programs, we have a pre-clinical program in collaboration with Bionic Sight to develop an optogenetic product candidate for patients with advanced retinal disease.

**Recent Corporate Milestones**

In December 2017, we started enrolling patients in a Phase 1/2 trial designed to test the safety and efficacy of our gene-therapy product candidate for the treatment of ACHM caused by mutations in the CNGA3 gene.

In April 2018, we dosed the first patient in our XLRP Phase 1/2 trial and received a corresponding milestone payment of $2.5M from Biogen.

Also in April 2018, we completed our targeted enrollment of 27 patients in our XLRS Phase 1/2 clinical trial.

In June, 2018, we received approval from the Israeli Ministry of Health (MOH) for our ACHM CNGA3 Phase 1/2 clinical trial, allowing us to begin enrolling patients in Israel for that trial.

In July 2018, we dosed the fourth patient in our XLRP Phase 1/2 trial and earned a corresponding milestone payment of $10M, which was received in August, 2018.**Our Strengths**

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

- Product candidates in clinical development, including four ongoing Phase 1/2 clinical trials with sufficient capital to complete enrollment and initial data analysis on all four of these trials;
- 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;
- Biogen collaboration for the development and commercialization of two clinical programs (XLRS and XLRP) and rights for them to develop three additional discovery programs (ALD and two ophthalmology programs);
- A collaboration with Bionic Sight for the development and commercialization of an optogenetic gene therapy and a neuro-prosthetic device with advanced retinal coding;
- Proprietary gene therapy manufacturing system, in which yields recently improved by 10-fold, capable of making significant quantities of high quality viral vectors in accordance with Good Manufacturing Practice (GMP) standards over seven different clinical trials;
- Product candidates which, to date, use recombinant AAV vector technology, a well-studied, versatile and efficient gene therapy approach;
- Initial safety data in our XLRS Phase 1/2 trial showing that our most advanced gene therapy candidate is generally safe and well tolerated across dose groups;
- Technical expertise in analytical techniques, synthetic promoter development, engineered capsids, optimized capsids and specialized formulation and delivery techniques; and
- Recently augmented 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 three key areas: vector design, vector manufacturing and vector 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 18 years conducting research on the best combinations of these elements with the aim of developing safe and effective product candidates.

*Vector Selection Overview*. The success of a gene therapy platform is highly dependent on the vector selected. Our platform is based on the use of a modified version of the 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 are capable of delivering 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. Not only do we engineer these promoters in-house, we also work with a collaborator, Synpromics, that specializes in designing promoter elements to 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 to the 8 or 10 to the 9 of 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 developed a proprietary, high-yield vector manufacturing process using scalable technologies. While our manufacturing method uses the herpes virus as a helper in the first step of a four-step AAV vector manufacturing process, there is no herpes virus in the final product. Our propriety process for AAV manufacturing uses robust cell lines that are well characterized and have been included in multiple regulatory submissions in the United States and Europe. This process is highly efficient and selective, generating more packaged vector with higher fidelity of target sequences than other production systems. We have developed and transferred over 30 robust and quantitative product-specific assays consistent with expected requirements for clinical development and are currently validating the assays as required for regulatory approval. We supplement our deep manufacturing experience with characterization of the resulting candidate technology. We have successfully completed technology transfer for vector manufacturing to three Contract Manufacturing Organizations (CMOs) and multiple partners. Additionally, in the last year we have made several process improvements leading to a ten-fold increase in productivity and conversion to a fully scalable suspension process.

Our manufacturing process has been reviewed by the FDA, the Irish Medicines Board, and the Israeli Ministry of Health and has been authorized for production of product candidates for use in clinical trials in the United States, Europe and Israel. To date we have successfully manufactured clinical trial material for seven different indications using three different manufacturing vendors, to ensure sufficient capacity, and believe we are the only AAV gene therapy company with this level of experience. Our manufacturing process is reproducible and scalable. Our process development facility is operational and we are conducting equipment evaluation runs with multiple vendors to support development of commercial processes for our product candidates.

We own or have licensed 67 issued patents, nine pending patent applications and three allowed patent applications covering our manufacturing technology. We believe that our core competency and intellectual property estate in vector manufacturing differentiate us competitively and 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. We have committed substantial resources to developing an integrated production and testing platform capable of meeting both clinical and commercial needs, including:

- Our propriety platform for AAV production generates high quality rAAV vectors with high packaging fidelity and low empty particles across multiple serotypes.
- Our AAV production system generates high volumetric productivities and has been demonstrated to work in multiple vendors' single-use bioreactor (SUB) systems.
- We have adapted our HSV helper manufacturing system to SUBs, 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 30 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 US, Israel and Europe.

Taken together, we believe the efficiency, productivity, scalability, characterization and regulatory definition of our proprietary rAAV manufacturing platform uniquely position us to accelerate from early phase human clinical trials to late phase, BLA-enabling data in all our clinical programs.

***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.

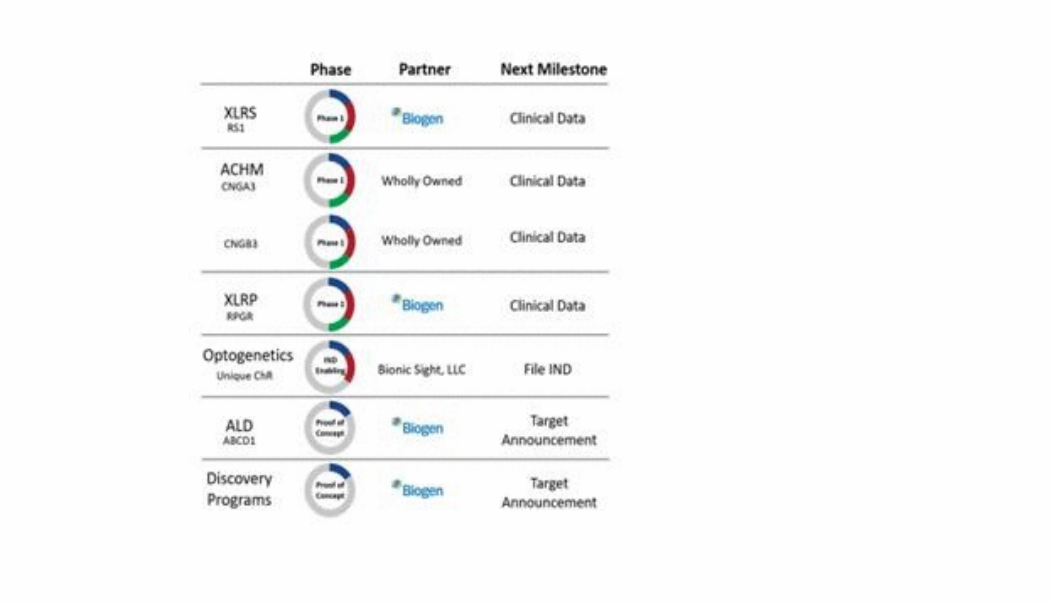
In ophthalmology, the product candidate can best be delivered to cells in the eye by either injecting the product candidate into the vitreous of the eye, an intravitreal injection, or by injecting the product candidate under the retina, a sub-retinal injection. Our ongoing clinical trial for our XLRS product candidate uses intravitreal injection, which we also expect to use as the method of delivery for Bionic Sights' optogenetic product candidate. We are using sub-retinal injection as the method of delivery for our ACHM and XLRP product candidates in our ongoing clinical trials.

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.

Once a product candidate is identified in our ALD discovery program, we expect it will be administered by intrathecal delivery, which is an injection into the cerebrospinal fluid.

**Our Product Candidate Pipeline**

The graphic below summarizes our current gene therapy programs:




Our four most advanced product candidates address ophthalmology indications XLRS, ACHM B3, ACHM A3, and XLRP, 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 four accepted endpoints—visual acuity, visual fields, contrast sensitivity and color vision—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 recent guidance 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 Retinoschisis (XLRS)***

XLRS is an inherited retinal disease, meaning that children are born with the defective gene that causes poor visual function that significantly affects daily living activities. XLRS is specifically caused by mutations in the RS1 gene, which is located on the X chromosome and encodes the retinoschisin, or RS1, protein. Retinoschisin is expressed and secreted primarily from photoreceptor cells and binds strongly and specifically to the surface of photoreceptor and bipolar cells in the retina. Mutated forms of retinoschisin are unable to bind properly, resulting in schisis, or splitting of the layers of the retina, primarily in the macula. The disease begins early in childhood, and affected boys typically have best-corrected visual acuity of 20/60 to 20/120 at initial diagnosis. Complications such as vitreous hemorrhage or retinal detachment occur in up to 40% of patients, especially in older patients. According to a published study, the incidence rate for XLRS is between one in 5,000 and one in 20,000 males. Using a conservative incidence rate of 1 in 11,500 and assuming half the population is male, we estimate that there are about 35,000 persons with XLRS in the United States and Europe combined.

The diagnosis of XLRS is made based on clinical findings and results of imaging studies and Electroretinogram (ERG). ERG is a clinical test that measures the electrical function of the retina. Clinical findings include reduced visual acuity and a characteristic spoke-wheel appearance of the macula when viewed by an ophthalmoscope, which is the instrument commonly used by ophthalmologists and optometrists to view the retina. Images obtained by optical coherence tomography, or OCT, a method of viewing layers of the eye somewhat like a sonogram, show spaces between the layers of the retina within the macula and fovea in most school-age boys with XLRS. These spaces mean that electrical signals cannot move from the photoreceptors to other retinal neurons and on to the brain, resulting in poor vision.

There is currently no approved treatment for XLRS. Management of disease manifestations includes low vision aids such as large-print textbooks, preferential seating in the front of the classroom and use of handouts with high contrast. Surgery may be required to address complications of vitreous hemorrhage or full-thickness retinal detachment, which occur in up to 40% of patients.

Previous anecdotal evidence has indicated that treatment with carbonic anhydrase inhibitors may provide a reduction in the degree of schisis. However, data from our natural history study demonstrated that topical carbonic anhydrase inhibitors provided reduction in the degree of schisis in only some patients. In any case, the absence of controlled clinical trials makes interpretation of these data difficult. In addition, treatment with carbonic anhydrase inhibitors does not address the fundamental genetic defect in persons affected by XLRS. Neither carbonic anhydrase inhibitors nor any other medicinal products have been approved by regulatory agencies for treatment of XLRS.

***Our XLRS product candidate***

Our gene therapy approach involves using an AAV vector to insert a functional copy of the RS1 gene into the patient's retinal cells, thereby inducing those cells to produce the normal retinoschisin protein. Our XLRS product candidate contains the RS1 gene and a promoter that has been shown to work well in primate retinal cells, and is packaged in a proprietary engineered AAV capsid that is able to efficiently enter target cells in the inner layers of the retina after intravitreal injection. Preclinical studies have demonstrated that our XLRS product candidate has relevant activity in an animal model of the disease.

*Clinical development*

We are currently conducting a Phase 1/2 clinical trial of our XLRS product candidate at nine clinical sites that specialize in inherited retinal diseases. The primary endpoint of this clinical trial is safety, and available data thus far have shown that the XLRS product candidate is generally safe and well tolerated. In addition to safety, this trial will measure biologic activity by assessing changes in visual function, visual acuity and quality of life.

The Phase 1/2 clinical trial protocol, is designed as a dose escalating trial to evaluate our product candidate in XLRS patients at three dose levels. In April, 2018, we completed targeted enrollment of 27 patients and expect to provide topline six-month data for these patients across both safety and biologic activity endpoints by the end of 2018, with the primary analysis of the full twelve-month active trial data six months later. We are also currently enrolling additional pediatric patients in the high dose group.

We have also completed a natural history study in persons affected by XLRS. The results from 56 participants with XLRS were evaluated on cross sectional and longitudinal measures of visual acuity, visual field sensitivity, microperimetry, cyst volume and ERG. The data suggest that patients with XLRS show a spectrum of visual function, with baseline visual acuity ranging from 0 to 82 ETDRS letters (average = 55.5; approximately 20/80). Measures of visual function were stable over time and weakly correlated to changes in cyst volume or patient age.

Successful completion of the Phase 1/2 clinical trial and the natural history study will guide us, and our collaborator Biogen, in finalizing the design of a potential pivotal clinical trial. In a pivotal trial, we would expect to enroll between 40 and 75 patients who will be evaluated for changes in visual function, and other parameters identified in the Phase 1/2 trial, over a 12-month period. If successful, we believe the results of this second trial could support submission of a Biologics License Application, or BLA, to the FDA in the United States and a Marketing Authorization Application, or MAA, to the EMA in Europe for our XLRS product candidate.

As a part of our collaboration, Biogen has obtained worldwide commercialization rights for the XLRS program. We will be responsible for the clinical development program through product approval. Biogen will fully reimburse us for the clinical development costs, subject to certain conditions, following the first-in-human study. We have an option to share development costs and profits after the initial clinical trial data are available.

***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, about half, or a total of 13,500 in the United States and Europe combined, have the form of the disease caused by mutations in the CNGB3 gene and approximately 6,800 people have the form of the disease caused by mutations in the CNGA3 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-related ACHM product candidate*

We are currently enrolling patients in a Phase 1/2 clinical trial at five clinical sites that specialize in inherited retinal diseases. An additional site is performing advanced optical testing on patients. The primary endpoint of this clinical trial is safety, and while available data thus far have shown that the ACHM CNGB3 product candidate is generally safe and well tolerated, we did experience initial variability in surgical procedures which we have now resolved. In addition to safety, this trial will measure biologic activity by assessing changes in visual function, visual acuity and quality of life.

The current clinical protocol, is designed as a dose escalating trial to evaluate our product candidate in ACHM patients at three dose levels.

The dosing levels for this trial were modified in November, 2017 to reflect additional preclinical data indicating the potential for efficacy at lower doses and thus increasing safety margins. As of September 6, 2018, we have completed enrollment of 6 patients across these two groups and two patients in a higher dose group.

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

Successful completion of the Phase 1/2 clinical study and the natural history study will guide us in finalizing the design of a potential pivotal clinical trial. In a pivotal trial, we expect that between 40 and 75 patients will be enrolled and evaluated for changes in visual function over a 12-month period following treatment. If successful, we believe the results of this pivotal trial could support our submission of a BLA to the FDA and of an MAA to the EMA for our ACHM-B3 product candidate.

*Clinical development of our CNGA3-related ACHM product candidate*

We are currently enrolling patients in a Phase 1/2 clinical trial at five clinical sites that specialize in inherited retinal diseases. An additional site is performing advanced optical testing on patients. The primary endpoint of this clinical trial is safety, and available data thus far have shown that the ACHM CNGA3 product candidate is generally safe and well tolerated. In addition to safety, this trial will measure biologic activity by assessing changes in visual function, visual acuity and quality of life.

The current clinical protocol, is designed as a dose escalating trial to evaluate our product candidate in ACHM patients at three dose levels. As of September 6, 2018, we have dosed two patients in the low dose group of this trial.

Additionally, we have nearly completed enrollment in a natural history study in persons affected by ACHM caused by CNGA3 mutations and results from the study will be presented in appropriate scientific meetings and publications.

Successful completion of the Phase 1/2 clinical study and the natural history study will guide us in finalizing the design of a potential pivotal clinical trial. In a pivotal trial, we expect that between 40 and 75 patients will be enrolled and evaluated for changes in visual function over a 12-month period following treatment. If successful, we believe the results of this pivotal trial could support our submission of a BLA to the FDA and of an MAA to the EMA for our ACHM-A3 product candidate.

***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 in the United States and Europe. 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 candidates***

Our gene therapy approach to 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 contains an optimized and stabilized RPGR gene and a promoter that has been shown in preclinical studies to drive efficient gene expression in primate rods, cones and restores photoreceptor function in dog and mouse models of XLRP.

*Clinical development*

We are currently enrolling patients in a Phase 1/2 clinical trial at four clinical sites that specialize in inherited retinal diseases. The primary endpoint of this clinical trial is safety, and available data thus far have shown that the XLRP product candidate is generally safe and well tolerated. In addition to safety, this trial will measure biologic activity by assessing changes in visual function, visual acuity and quality of life.

The current clinical protocol, is designed as a dose escalating trial to evaluate our product candidate in XLRP patients at three dose levels. As of September 6, 2018, we have dosed 5 patients across two dose groups.

We are also conducting a natural history study in patients with XLRP caused by RPGR mutations. This study documents the progression of the disease in the absence of treatment and is providing important information about the best methods for measuring visual function and other parameters in these patients, which will guide us in the design and evaluation of subsequent clinical trials in which our product candidate will be tested for safety and efficacy.

Successful completion of the Phase 1/2 clinical study and the natural history study will guide us, and our collaborator Biogen, in finalizing the design of a potential pivotal clinical trial. In a pivotal trial, we expect that between 80 and 120 patients will be enrolled and evaluated for changes in visual function over a 24-month period following treatment. If successful, we believe the results of this pivotal trial could support our submission of a BLA to the FDA and of an MAA to the EMA for our XLRP product candidate.

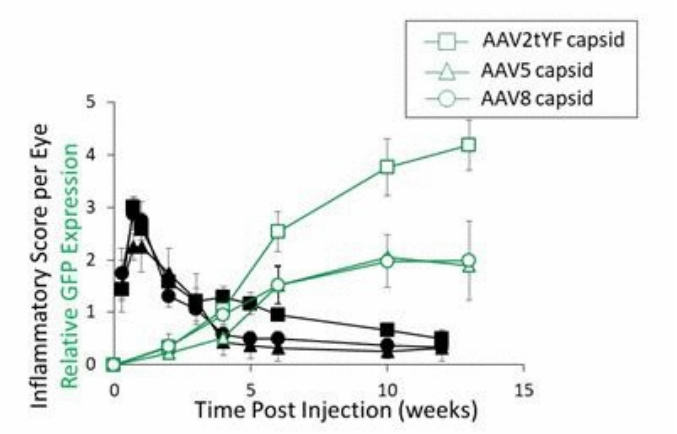
As a part of our collaboration, Biogen obtained worldwide commercialization rights for our XLRP program. We will lead the clinical development program through the completion of first-in-human trials. Biogen will fully reimburse us for the clinical development costs, subject to certain conditions, following the IND-enabling studies. We have an option to share development costs and profits after the initial clinical trial data are available, and an option to co-promote the second of two product candidates (XLRS and XLRP) to be approved in the United States. As of September 6, 2018, we have earned a total of $12.5M in clinical milestones related to the dosing of patients in this trial.

***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 multiple new programs that are in early safety and preclinical proof of concept of stages that are described below.***

**XLRP product candidate differentiation**

Three AAV gene therapy vectors are currently in Phase I/II 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 AAV2tYF, the AAV capsid used in AGTC product candidates, AAV5 and AAV8 capsids in a head-to-head non-human primate (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 AAV2tYF (n=12), AAV5 (n=4) and AAV8 (n=8) revealed that AAV2tYF 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, AAV2tYF 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. 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). Much work has been done to understand similar findings in the liver following AAV administration, but 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, and have been able to eliminate the following as key drivers of inflammation: production methodology (transfection versus HSV), characteristics of the AAV product (full to empty capsid ratio, transgene and process residuals) and capsid serotype (AAV2 versus AAV8) or novel engineered variant (AAV2tYF). 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.

***Advanced Retinal Disease***

As part of our collaboration with Bionic Sight we are developing 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 which 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. These light sensitive proteins open or close in response to light and allow millisecond-scale temporal manipulation of electrical events. Therefore, optogenetics provides a way to manipulate the activity of cells by controlling these proteins with light. A number of studies have been published on the effectiveness of these proteins for treating RP in animal models. Other studies have shown their effectiveness supplemented with a vision-enhancing device known as a retinal prosthesis. The device receives images, processes them, and then converts them into the same patterns of electrical pulses that the brain normally receives from normally functioning photoreceptors.

The candidate treatment being developed by AGTC and Bionic Sight is a recombinant AAV that expresses an optogenetic protein, ChronosFP, in the retinal ganglion cells. Light then activates the ChronosFP to send electrical signals from the retinal ganglion cells to the brain. Bionic Sight is developing a device with a retinal code that can provide light signals to the retinal ganglion cells that will result in an image the brain can recognize and may significantly enhance vision in patients who have received the optogenetic treatment. Our collaborator, Bionic Sight, plans to file an IND for this product candidate for the treatment of RP in the first half of calendar 2019.

**Other opportunities in ophthalmology**

We believe our current gene therapy platform will enable us to develop and test new AAV vectors that carry gene sequences for other inherited diseases in ophthalmology (it is estimated that approximately 290 genes causing inherited retinal disease have been identified), and that by leveraging our work on our lead programs 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. As an example, in January 2016 we announced a research effort in collaboration with the Blue Cone Monochromacy Family Foundation, in blue cone monochromacy (BCM), a rare genetic disease of the retina that almost exclusively affects males. We also believe that there are large-market ophthalmology diseases where AAV vectors may provide benefit, such as wet AMD.

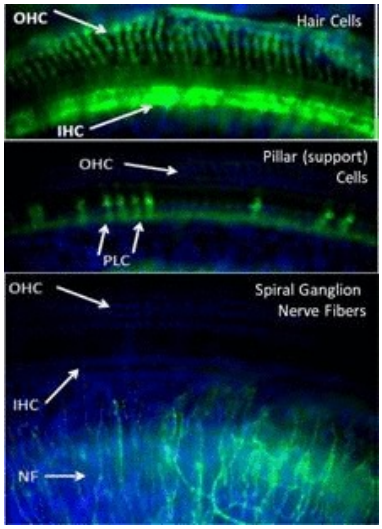
**Central Nervous System**

Our collaboration with Biogen includes options for three discovery programs, two in ophthalmic diseases and one in a non-ophthalmic condition. On February 8, 2016, we announced that Biogen had selected adrenoleukodystrophy (ALD) as the indication for the non-ophthalmic discovery program.

ALD 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. 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. We are working with Biogen on vector design, animal model proof of concept and targeting studies in non-human primates in order to obtain data to support moving a potential product candidate to IND enabling studies.

**Otology**

We previously announced a broad initiative in the area of otology in which we will use our capabilities to develop potential gene therapy product candidates that can address genetic causes of hearing loss. 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 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 that are technically feasible and commercially viable. To augment this exercise, we have been actively screening novel capsid variants in mouse and guinea-pig for their ability to transduce the key cell types of the cochlear – inner and outer hair cells, supporting cells and spiral ganglion neurons. Below are representative examples of immunohistochemical analyses, using GFP as the reporter transgene, from the mouse cochlear, demonstrating cell profiles of interest.




OHC = outer hair cell; IHC = inner hair cell

To supplement these mouse and guinea-pig studies, we have recently demonstrated that AAV vectors can safely be administered to non-human primates without adversely affecting auditory brainstem responses, and transduce the cell types of interest in the cochlea.

In addition to the capsid identification studies, we intend to control the specificity of transgene expression by designing and testing novel synthetic promoters for the cochlear cell types of interest highlighted above. Such promoters, when placed upstream of the transgene of interest, will ensure that the gene replacement occurs in the correct cell, and limit the likelihood of inappropriate gene expression leading to adverse consequences from a safety perspective.

**Strategic collaborations with Biogen and Bionic Sight**

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 July 1, 2015, we entered into a broad collaboration and license agreement with Biogen to develop gene-based therapies for multiple ophthalmic diseases. Biogen made an upfront payment to us in the amount of $124.0 million, which included a $30.0 million equity investment and certain prepaid research and development expenditures. Biogen was granted a license to the XLRS and XLRP programs and the option to license discovery programs for two additional ophthalmic indications and one non-ophthalmic indication at the time of clinical candidate selection. In February 2016, we announced Biogen's selection of adrenoleukodystrophy, or ALD, as the non-ophthalmic indication of the three discovery programs. Under the collaboration, we are eligible to receive upfront and milestone payments exceeding $1 billion. This includes up to $472.5 million collectively for the two lead programs, which also will carry royalties in the high single digit to mid-teen percentages of annual net sales. In addition, Biogen may make payments up to $592.5 million across the discovery programs, along with royalties in the mid-single digits to low teen percentages of annual net sales for the discovery programs.

Since inception of the collaboration agreement, we have earned or received milestone payments from Biogen totaling $17.5 million consisting of:

- a $5.0 million milestone payment for enrollment of the first patient in our XLRS Phase 1/2 trial;
- a $2.5 million milestone payment for dosing the first patient in our XLRP Phase 1/2 trial; and
- a $10 million milestone payment for dosing the forth patient in our XLRP Phase 1/2 trial.

Biogen will also receive an exclusive license to use our proprietary manufacturing technology platform to make AAV vectors for up to six genes, three of which are chosen at our discretion, in exchange for payment of milestones and royalties.

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 agreement, AGTC made an initial $2.0 million payment to Bionic Sight for an equity interest in that company. This initial investment represents an approximate 5 percent equity interest in Bionic Sight. In addition to the initial investment, AGTC will contribute to ongoing research and development costs through additional payments or other in-kind contributions. These payments and contributions will be made over time, up to the date that Bionic Sight has received both investigational new drug clearance from the FDA and receipt of written approval from an institutional review board to conduct clinical trials from at least one clinical site for that product candidate.

AGTC will receive additional equity, based on the valuation in place at the beginning of the agreement, for these cash and in-kind research and development contributions and will be obligated to purchase additional equity in Bionic Sight for $4.0 million, upon the filing of an IND for the product candidate at a pre-determined valuation. Our collaborator, Bionic Sight, plans to file an IND for this product candidate for the treatment of RP in the first quarter of calendar 2019.

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 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 three 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. We interact frequently with the Powell Gene Therapy Center at UF and have an excellent working relationship with the UF Office of Technology Licensing.

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 a sub-awardee, we expect to receive approximately $3.8 million over five years under this grant. As of June 30, 2018, we have received payments in the aggregate amount of $2.8 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, and the Alpha-1 Foundation. Both organizations are well known for their advocacy of patients' interests in obtaining diagnosis, developing treatments and providing for reimbursement. Both actively support research into treatment, and we have been awarded three research grants totaling $1.6 million from the FFB and one grant of $0.3 million from the Alpha-1 Foundation. 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, ALD Connect, The Alpha-one Project and Global Genes.

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 AAT deficiency. 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 IP 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 August 15, 2018, our patent portfolio included approximately 70 patents and patent applications that we own and approximately 48 patents and patent applications that we have licensed. More specifically, we own nine U.S. patents, seven pending U.S. applications, 32 foreign patents and 22 foreign patent applications. We have licensed six U.S. patents, two pending U.S. applications, 35 foreign patents and two pending foreign patent applications. Of the patents and patent applications that we own or license, 79 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. Two of the

patents and 18 of the patent applications that we own are directed to small cone promoters and uses thereof. A patent issuing from this group could have an expiration date in 2034.

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 upon 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 2018 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 three 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 mutant 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.

  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.

***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***

We continue to collaborate with 4DMT and Synpromics in order to develop optimized capsids to target specific cell populations and novel synthetic promoters, respectively.

**Competition**

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 XLRS, ACHM and XLRP, although at least one company is developing a candidate gene therapy treatment intended to treat each indication. We believe 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, record keeping, 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 and, in some instances, the NIH, through its Recombinant DNA Advisory Committee, or RAC. 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 and its RAC, which makes recommendations to the NIH on gene therapy issues and engages 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.

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 two additional gene therapy approvals, the most recent being 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 them to take additional steps this past year to support the advancement of gene therapy products. In July 2018 they issued a suite of draft guidance documents that provide insight into their expectations for product development including manufacturing, clinical trial design and development in rare diseases. AGTC's review of the draft guidelines 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, two 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, Strimvelis became the second 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 assure 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.

When a gene therapy trial is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, along with the submission of an IND to the FDA, the protocol and related documentation is submitted to and the trial is registered with the NIH Office of Science Policy, or OSP, pursuant to the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules, or NIH Guidelines. Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for research involving recombinant DNA, however many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. The oversight bodies at the initial clinical site(s) (Institutional Review Board (IRB) and Institutional Biosafety Committee (IBC)) are responsible for recommending whether or not the clinical study requires public review by the RAC, a federal advisory committee, which discusses protocols that raise novel or particularly important scientific, safety or ethical considerations. This recommendation is included with the protocol and related documentation submitted to the OSP as part of the trial registration process. The OSP may agree or disagree with that recommendation. Should the oversight bodies recommend public RAC review and the OSP concurs, the protocol will then be reviewed at one of the NIH RAC's quarterly meetings for which information must be submitted at least eight weeks in advance. RAC proceedings and reports are posted to the OBA web site and may be accessed by the public.

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. If the FDA places the clinical trial on a clinical hold within that 30-day time period, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. With gene therapy protocols, if the FDA allows the IND to proceed, and the trial oversight bodies and the OSP decide that full public review of the protocol is warranted, initiation of the protocol will be delayed until after completion of the RAC review process. The FDA may also impose clinical holds on a biological product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an

IND will result in the FDA allowing clinical 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 stopping rules that assure 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 that includes annual examinations and/or annual queries, either in person or by questionnaire, of trial subjects.

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, the NIH 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 recently approved three 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. The NIH and the FDA have a publicly accessible database, the Genetic Modification Clinical Research Information System, which includes information on gene transfer trials and serves as an

electronic tool to facilitate the reporting and analysis of adverse events on these trials. Over the last several years the FDA has issued helpful guidance on development of gene therapy products and has recently shown an increased willingness to work closely with developers, especially with those working in orphan disease areas.

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 product development, laboratory and animal studies, human trials, 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 2019, which becomes effective October 1, 2018, the user fee for an application requiring clinical data, such as a BLA, is $2,588,478. PDUFA also imposes an annual prescription drug program fee ($309,915) 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 assure 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, 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 assure 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 assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

Before approving a BLA, the FDA will inspect the facilities at which the product candidate is manufactured. The FDA will not approve the product candidate unless it determines that the manufacturing processes and facilities are in compliance with GMP requirements and adequate to assure consistent production of the product candidate within required specifications. Additionally, before approving a BLA, the FDA may inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND trial requirements and GCP requirements. To assure GMP and GCP compliance, an applicant must incur significant expenditure of time, money and effort in the areas of training, record keeping, 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.

***Orphan drug designation***

Under the Orphan Drug Act, the FDA may grant orphan 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 more than 200,000 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 product designation must be requested before submitting an NDA or BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product candidate that has orphan 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. 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 defined 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 or 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 demonstrate 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.

Any product candidate submitted to the FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as 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. Drug or biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval, which means that they may be approved on the basis of adequate and well-controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug or biological product candidate receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

Under the provisions of the new 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 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.

We also must comply with the FDA's advertising and promotion requirements, such as the prohibition of preapproval promotion, requirements related to direct-to-consumer advertising, the prohibition on promoting products for uses or in-patient populations that are not described in the product's approved labeling (known as "off-label use"), limitations on industry-sponsored scientific and educational activities, and promotional activities involving the internet. Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant or

manufacturer to administrative or judicial civil or criminal sanctions and adverse publicity. FDA sanctions could include refusal to approve pending applications, withdrawal of an approval, clinical hold, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, mandated corrective advertising or communications with doctors, debarment, restitution, disgorgement of profits, or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

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. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved BLA, including withdrawal of the product from the market. 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.

***United States patent term restoration and marketing exclusivity***

Depending upon 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, 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.

A reference biologic is granted twelve 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 was introduced, which would reduce exclusivity for biological drugs from twelve to seven years. 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 health care 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 $32.2 million, $26.2 million and $39.4 million for the fiscal years ended June 30, 2018, 2017 and 2016, respectively.

**Employees**

As of June 30, 2018, we had 78 full-time employees, 48 of whom have Ph.D., M.D. or other post-graduate degrees. Of these full-time employees, 55 are engaged in research and development activities and 23 are engaged in finance, human resources, facilities and general management.

All of our personnel are co-employees of AGTC and a professional human resource service organization, TriNet HR Corporation, or TriNet. Under our agreement with TriNet, TriNet 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 for these individuals. We reimburse TriNet for these costs and pay TriNet 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 TriNet to be reasonable and customary and believe 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 material with, or furnish it to, the SEC. 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, 2016, these trademarks have been registered in the United States, 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 these 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.

**Implications of being an emerging growth company**

As a company with less than $1.07 billion in revenue during our last fiscal year, we qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. As an emerging growth company, we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include:

- only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure;
- reduced disclosure about our executive compensation arrangements;
- no non-binding advisory votes on executive compensation or golden parachute arrangements; and
- exemption from the auditor attestation requirement in the assessment of our internal controls over financial reporting.

We may take advantage of these exemptions for up to five years from the date of our initial public offering of common stock or such earlier time that we are no longer an emerging growth company. We would cease to be an emerging growth company if we have more than $1.07 billion in annual revenue, we have more than $700 million in market value of our stock held by non-affiliates or we issue more than $1 billion of non-convertible debt over a three-year period. We may choose to take advantage of some, but not all, of the available exemptions.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth 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 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 revenues 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 our collaboration agreement with Biogen, we have incurred losses from operations in each year since our inception in 1999. We reported net losses of $21.3 million and $1.4 million for each of the fiscal years ended June 30, 2018 and 2016, respectively. As of our most recent fiscal year ended June 30, 2018, we had an accumulated deficit of $110.9 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 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 revenues 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 Biogen and Bionic Sight, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our product candidates. We do not anticipate generating revenues 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 revenues 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, such as wet AMD, 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 revenues 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.

***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 XLRS, 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, 2018, and 2017, our cash and cash equivalents and investments amounted to $104.9 million and $138.4 million, respectively. Our research and development expenses were $32.2 million, $26.2 million and $39.4 million for the fiscal years ended June 30, 2018, 2017 and 2016, respectively. We believe that our existing cash and cash equivalents at June 30, 2018 will be sufficient to enable us to advance planned preclinical studies and clinical trials for our lead product candidates for at least the next two years. 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 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 certain 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.

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.

**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 assure safety in the full population for which we seek approval;
- the 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 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 approval from applicable regulatory authorities outside the United States. 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 revenues. 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, manufacturers or manufacturing process;
- warning letters;
- 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;
- 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 revenues 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, three gene therapy products have been approved in the United States and two 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.

Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. For example, the FDA has 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. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the United States National Institutes of Health, or the NIH, are also subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC review process can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and approved its initiation. Conversely, the FDA can put an IND on clinical hold even if the RAC has provided a favorable review of the drug. Also, before a clinical trial can begin at an NIH-funded institution, 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 the requirements for approval of any of our product candidates.

These regulatory review committees and advisory groups and the 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.

***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 previous 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 for our lead product candidates will result in success in our clinical trials of those product candidates.

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 early versions of lentiviral vectors, which integrate with, and thereby alter, the host cell's DNA, have led to several well-publicized adverse events, including reported cases of leukemia. 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 and we have 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, each 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 all patients in our ACHM CNGB3 and ACHM CNGA3 trials in the near-term and a protocol amendment that required 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 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 practices, or GCP requirements or applicable regulatory 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 the FDA's good manufacturing practice, or GMP, requirements or applicable regulatory 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 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.

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, 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 revenues 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 revenues 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 trial 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 or events. These adverse events may occur despite our belief that our AAV vectors have an improved safety profile over prior such treatments.

Known adverse side effects 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 side effects 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 events, 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 events 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 events 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 revenues 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 health care 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 product designation or exclusivity for some of our product candidates. If our competitors are able to obtain orphan product exclusivity for their products that are 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 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 5 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.

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 indication for that time period, except in limited circumstances. The FDA has not defined the meaning of "same drug" specifically for gene therapy products and it is possible that the FDA could conclude that no two gene therapy products could be considered the same. The applicable 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 sufficient quantity of the product to meet the needs of patients with the rare disease or condition, or if a gene 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, record-keeping, and submission of safety and other post-market information. Any regulatory approvals that we receive for our product candidates may also be subject to 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 must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with 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 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 revenues.

In addition, the FDA's policies may change and additional government regulations may be enacted that 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.

**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 have completed the design phase for a cGMP facility at our Florida headquarters to support later stage clinical development. 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. We are currently negotiating with and conducting pilot work at three alternative third-party manufacturers to expand our capacity and mitigate risk. 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 record keeping) 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.

As described above in "Business," we have encountered delays in clinical trial material availability as a result of difficulties in proper testing. 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 other 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 revenues 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, such as our collaboration with Biogen, may be important to our business. If these collaborations are not successful, our business could be adversely affected.***

We have a collaboration with Biogen to develop, seek regulatory approval for and commercialize gene therapy products to treat XLRS and XLRP. The collaboration agreement also provides for discovery programs targeting three indications (including our adrenoleukodystrophy (ALD) program) whereby we will conduct discovery, research and development activities for those additional drug candidates through the stage of clinical candidate designation, after which, Biogen may exercise an option to continue to develop, seek regulatory approval for and commercialize the designated clinical candidate. In addition, under our manufacturing agreement with Biogen, we granted Biogen an exclusive license to use our proprietary technology platform outside of the collaboration to make AAV vectors for up to three available genes and three additional genes that we may approve in our discretion. An unsuccessful outcome in pending and future clinical trials for which Biogen is responsible could be harmful to the public perception and prospects of our gene therapy platform.

Our license relationships with Biogen and any other current collaboration or collaboration 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, 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.

In the event Biogen terminates the collaboration for a material breach by us, we will be restricted from competing with Biogen for two years in the terminated programs with Biogen having the right, in lieu of termination, to elect to maintain the license from us and reduce the royalties and milestones in a manner specified in the agreement.

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 other than those covered by our collaboration with Biogen. 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. 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 revenues 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 revenues.***

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, such as wet AMD, 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 XLRS, ACHM and XLRP. We believe 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, such as wet AMD, 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 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 is 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 (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 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 regulation 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 revenues 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 three gene therapy products approved to date in the United States and only two 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 early versions of lentiviral vectors, which integrate with, and thereby alter, the host cell's DNA, have led to several well-publicized adverse events, including reported cases of leukemia. Although none of our current product candidates utilize lentiviral vectors, our product candidates use a viral delivery system. 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 health care 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. On January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities 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. More recently, President Trump and the Republican majorities in both houses of the U.S. Congress have been seeking to repeal or replace all or portions of the ACA but to date they have 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. The implications of these actions for our and our partners' business and financial condition, if any, are not yet clear.

***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 health care payors, we will not be able to generate significant revenues 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 revenues, 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 requires that companies evaluate and report on their systems of internal control over financial reporting. In addition, our independent registered public accounting firm must report on its evaluation of those controls, effective for the fiscal year 2019.

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 by, among other things, hiring additional employees to provide further support to our finance and accounting team; restructuring our finance team to better align the functional areas to the overall strategy of the company, while at the same time providing more focus for the accounting team in maintaining proper control over the financial reporting process commensurate to support standalone external financial reporting under public company or SEC requirements; providing functional and system training to employees and preparing detailed documentation to clearly define key tasks and actions, as well as the positions responsible for those tasks and actions; engaging a consulting firm to assist in documenting and formalizing our accounting policies and internal control processes and to help strengthen supervisory reviews by our management; designing and implementing monthly manual controls to manage our financial reporting close processes and to help ensure an adequate level of segregation of duties within our finance and accounting function; developing and implementing policies and procedures related to security access, including security access reviews of our key financial systems' users to ensure the appropriateness of their roles and security access levels; and performing testing related to the functioning of these controls, and continuing to monitor these controls and make enhancements as needed.

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 Section 404 of the Sarbanes-Oxley Act of 2002, 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 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 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, health care clearinghouses and health care 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 health care 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 health care 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 health care 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 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 and 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, TriNet HR Corporation, or TriNet. Under the terms of our arrangement, TriNet 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 for these individuals. We reimburse TriNet for these costs, and pay TriNet an administrative fee for its services. If TriNet 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 TriNet 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 revenues 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 loss carryforwards 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 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.

***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 the 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, it would result in damage to 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, or in some cases not 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.

***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 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 and this circumstance 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, and 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 recently 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 20, 2018, the price of our common stock on the Nasdaq Global Market has ranged from $3.25 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 rates, fuel prices, international currency fluctuations 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 which investors perceive to be similar to us, the opportunities in the digital simulation market or the stock market in general, could 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. As a newly public company, we have only limited coverage by securities 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.

***We are an "emerging growth company," and the reduced reporting requirements applicable to emerging growth companies may make our common stock less attractive to investors.***

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act t, reduced disclosure obligations regarding executive compensation and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, changes in rules of U.S. generally accepted accounting principles or their interpretation, the adoption of new guidance or the application of existing guidance to changes in our business could significantly affect our financial position and results of operations. Based on the market value of our common stock at December 31, 2017 and the number of shares of our common stock held by non- affiliates, we expect to continue to be an emerging growth company until June 30, 2019. Even after we no longer qualify as an emerging growth company, we may continue to qualify as a "smaller reporting company" which would allow us to take advantage of many of the same exemptions from disclosure requirements. However, we do expect to be required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act for the fiscal year ending June 30, 2019 because we are an "accelerated filer" as defined in Rule 12b-2 of the Exchange Act. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

***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 at prices and in a manner, 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 a diversion of 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

None.

## ITEM 2. PROPERTIES

*Alachua, Florida*

Our corporate headquarters are located in Alachua, Florida. In January 2016, we moved into a new combined-use facility consisting of approximately 21,000 square feet of laboratory and office space. The initial lease term for this facility is 10 years and we have options to extend the term of the lease for three additional five-year periods. Our prior leased facilities encompassed approximately 7,000 square feet of office and laboratory space. Operating leases associated with the prior facilities expired in December 2015.

*Cambridge, Massachusetts*

In August 2015, we entered into a two-year lease to occupy approximately 3,000 square feet of office and laboratory space in Cambridge, Massachusetts. On July 31, 2017, we entered into a new lease to increase our office and laboratory space in Cambridge by approximately 5,000 square feet to a total of approximately 8,000 square feet and extend the term of the lease for an additional seven years, with an option to further extend the 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.

## 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. The following table sets forth, for the periods indicated, the high and low intraday sales prices of our common stock as reported by The NASDAQ Global Market:

| | 2018 | | 2017 | |
|---|---|---|---|---|
| | High | Low | High | Low |
| First fiscal quarter | $ 5.30 | $ 3.50 | $ 17.00 | $ 8.50 |
| Second fiscal quarter | $ 4.25 | $ 3.25 | $ 10.85 | $ 6.35 |
| Third fiscal quarter | $ 5.51 | $ 3.55 | $ 10.15 | $ 6.08 |
| Fourth fiscal quarter | $ 5.75 | $ 3.60 | $ 7.05 | $ 4.70 |

As of August 31, 2018, a total of 18,129,148 shares of our common stock were outstanding and we had 35 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.

**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."

**Issuer Purchases of Equity Securities**

The following table provides certain information with respect to our purchases of shares of the Company's common stock during the fourth quarter of fiscal 2018

**Issuer Purchases of Equity Securities**

| Period | Total Number of Shares Purchased(a) | Average Price Paid per Share(a) | Total Number of Shares Purchased as Part of Publicly Announced Plan | Approximate Dollar Value of Shares That May Yet Be Purchased Under the Plan |
|---|---|---|---|---|
| April 1, 2018 through April 30, 2018 | 685 | $ 4.00 | — | $ — |
| May 1, 2018 through May 31, 2018 | 4,110 | $ 5.49 | — | $ — |
| June 1, 2018 through June 31, 2018 | 606 | $ 4.55 | — | $ — |
| Total | 5,401 | $ 5.20 | — | |

- These columns reflect the surrender to the Company of an aggregate of 5,401 shares of common stock to
- satisfy tax withholding obligations in the connection with the vesting of restricted stock issued to an employee
- during the fourth quarter of fiscal 2018.

**ITEM 6: SELECTED FINANCIAL DATA**

The following selected financial data should be read in conjunction with our financial statements and related notes in Part II, Item 8 of this Annual Report on Form 10-K and with our "Management's Discussion and Analysis of Financial Condition and Results of Operations" in Part II, Item 7 of this Annual Report on Form 10-K.

Our selected statement of operations data for the fiscal years ended June 30, 2018, 2017, and 2016 and our selected balance sheet data as of June 30, 2018 and 2017 are derived from our audited financial statements included elsewhere in this report. The selected statements of operations data for the fiscal years ended June 30, 2015 and 2014 and the balance sheet data as of June 30, 2016, 2015 and 2014 are derived from our audited financial statements not included in this Annual Report on Form 10-K. Our historical results are not necessarily indicative of results to be expected for any future period. The selected financial data in this section are not intended to replace our financial statements and the related notes.

**Selected Financial Data**

| | Year Ended June 30, | | | | |
|---|---|---|---|---|---|
| | 2018 | 2017 | 2016 | 2015 | 2014 |
| | (in thousands except per share data) | | | | |
| **Statement of Operations Data:** | | | | | |
| Revenue: | | | | | |
| Collaboration Revenue | $ 24,057 | $ 39,282 | $ 46,751 | $ - | $ - |
| Grant revenue | 129 | 191 | 610 | 1,682 | 917 |
| Sponsored research and other revenue | - | - | - | 672 | 212 |
| Total revenue | 24,186 | 39,473 | 47,361 | 2,354 | 1,129 |
| Operating expenses: | | | | | |
| Research and development | 32,181 | 26,217 | 39,376 | 18,118 | 8,503 |
| General and administrative | 14,389 | 11,354 | 10,074 | 8,768 | 5,182 |
| Total operating expenses | 46,570 | 37,571 | 49,450 | 26,886 | 13,685 |
| Income (loss) from operations | (22,384) | 1,902 | (2,089) | (24,532) | (12,556) |
| Other income (expense): | | | | | |
| Investment income, net | 1,301 | 952 | 711 | 216 | 42 |
| Fair value adjustments to warrant liabilities | - | - | - | - | (441) |
| Fair value adjustments to Series B purchase rights | - | - | - | - | (2,904) |
| Other | (125) | (47) | (3) | (2) | (49) |
| Total other income (expense), net | 1,176 | 905 | 708 | 214 | (3,352) |
| Income (loss) before provision for income taxes | (21,208) | 2,807 | (1,381) | (24,318) | (15,908) |
| Provision for income taxes | 72 | 2,400 | - | - | - |
| Income (loss) before equity in net losses of affiliate | (21,280) | 407 | (1,381) | (24,318) | (15,908) |
| Equity in net losses of affiliate | (20) | - | - | - | - |
| Net income (loss) | $ (21,300) | $ 407 | $ (1,381) | $ (24,318) | $ (15,908) |
| Net income (loss) per share, basic (1) | $ (1.18) | $ 0.02 | $ (0.08) | $ (1.50) | $ (4.46) |
| Net income (loss) per share, diluted (1) | $ (1.18) | $ 0.02 | $ (0.08) | $ (1.50) | $ (4.46) |
| Weighted-average shares outstanding, basic | 18,105 | 18,072 | 17,810 | 16,253 | 3,568 |
| Weighted-average shares outstanding, diluted | 18,105 | 18,385 | 17,810 | 16,253 | 3,568 |

| | June 30, | | | | |
|---|---|---|---|---|---|
| | 2018 | 2017 | 2016 | 2015 | 2014 |
| | (in thousands) | | | | |
| **Balance Sheet Data:** | | | | | |
| Cash and cash equivalents | $ 31,065 | $ 30,706 | $ 28,868 | $ 39,187 | $ 8,623 |
| Short and long-term investments | $ 73,840 | $ 107,743 | $ 143,847 | $ 46,083 | $ 64,450 |
| Total assets | $ 118,531 | $ 147,923 | $ 180,797 | $ 90,174 | $ 77,407 |
| Current liabilities | $ 14,395 | $ 28,156 | $ 54,743 | $ 4,642 | $ 2,534 |
| Total stockholders' equity | $ 99,182 | $ 115,329 | $ 109,288 | $ 85,532 | $ 74,873 |

(1) See Note 2 of the notes to the financial statements appearing elsewhere in this annual report on Form 10-K for a description of the method used to calculate basic and diluted net loss per share.

## 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 retinoschisis (XLRS), X-linked retinitis pigmentosa (XLRP), and achromatopsia (ACHM) and a preclinical program in optogenetics. In addition to ophthalmology, we have initiated preclinical programs in adrenoleukodystrophy (ALD) and otology. With a number of important clinical milestones on the horizon, we believe we are well positioned to advance multiple programs towards pivotal studies. In addition to our product pipeline, we have also developed broad technological capabilities through our collaborations with Synpromics Limited (Synpromics), and the University of Florida, which provide us with expertise in vector design and manufacturing as well as synthetic promoter development and optimization. Finally, our partnership with Biogen, which includes our clinical XLRS and XLRP programs, a discovery program in ALD and two ophthalmology programs, validates our approach and technology, and provides us with a significant cash runway to advance our wholly-owned candidates.

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 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. To date, we have funded our operations primarily through the private placement of preferred stock, common stock, convertible notes and warrants to purchase preferred stock, through public offerings of common stock , and through upfront and milestone payments from our partners. 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 other year since inception except for fiscal 2017. Our net loss for the fiscal year ended June 30, 2018 was $21.3 million, compared to net income of $0.4 million and net losses of $1.4 million for each of the fiscal years ended June 30, 2017 and 2016, 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:

- conduct preclinical studies and clinical trials for our XLRS, ACHM and XLRP 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 inherited 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 collaboration, product development and commercialization efforts; and
- continue to operate as a public company.

As of June 30, 2018, we had cash and cash equivalents and investments totaling $104.9 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 at June 30, 2018, will be sufficient to allow us to generate data from our ongoing clinical programs, to move our pre-clinical optogenetic program in collaboration with Bionic Sight into the clinic and to fund our currently planned research and discovery programs for at least the next two years. 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. 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.

**Strategic Collaborations**

***Biogen***

In July 2015, we entered into a collaboration agreement with Biogen, which we refer to as the collaboration agreement, pursuant to which we and Biogen will collaborate to develop, seek regulatory approval for and commercialize gene therapy products to treat XLRS, XLRP, and discovery programs targeting three indications based on our adeno-associated virus vector technologies. The Collaboration Agreement became effective on August 14, 2015.Under the collaboration agreement, we will conduct all development activities through regulatory approval in the United States for the XLRS program (with activities through Phase 1/2 completion being pre-funded under the agreement and any further activities subject to incremental consideration), and all development activities through the completion of the first in human clinical trial for the XLRP program (with activities through filing the IND being pre-funded under the agreement and any further activities subject to incremental consideration). In addition, the collaboration agreement provides for discovery programs targeting three indications whereby we will conduct discovery, research and development activities for those additional drug candidates through the stage of clinical candidate designation, after which, Biogen may exercise an option to continue to develop, seek regulatory approval for and commercialize the designated clinical candidate. In February 2016, we announced Biogen's selection of adrenoleukodystrophy as the non-ophthalmic indication of the discovery programs. Under the terms of the collaboration agreement, we, in part through our participation in joint committees with Biogen, will participate in overseeing the development and commercialization of these specific programs.

Under the collaboration agreement, we received a non-refundable upfront payment of $94.0 million in August 2015. As a result of the upfront payment made by Biogen, we became liable to our various research partner institutions for sub-license payments, which led us to record an expense of $10.6 million. We paid these costs in full during the fiscal year ended June 30, 2016.

During 2018, 2017 and 2016, we recognized revenue of approximately $24.1 million, $39.3 million and $46.8 million, respectively from our collaboration with Biogen. We are also eligible to receive payments of up to $467.5 million based on the successful achievement of future milestones under the two lead programs and up to $592.5 million based on the exercise of the option for and the successful achievement of future milestones under the three discovery programs. Biogen will pay revenue-based royalties for each licensed product at tiered rates ranging from high single digit to mid-teen percentages of annual net sales of the XLRS or XLRP products and at rates ranging from mid-single digit to low-teen percentages of annual net sales for the discovery products. Due to the uncertainty surrounding the achievement of the future milestones, such payments were not considered fixed or determinable at the inception of the collaboration agreement and accordingly, will not be recognized as revenue unless and until they become earned. We achieved the first milestone under the XLRS program in August 2015 for enrollment of the first patient in our Phase 1/2 trial, which triggered a milestone payment from Biogen of $5.0 million and the recording of milestone revenue and sublicense expense of $1.4 million. We achieved the first milestone under the XLRP program in April 2018 for dosing of the first patient in our Phase 1/2 trial, which triggered a milestone payment from Biogen of $2.5 million and the recording of milestone revenue and sublicense expense of $0.6 million. In July 2018, we earned the second milestone under the XLRP program for enrolling the fourth patient in our Phase 1/2 trial, which will trigger a milestone payment from Biogen of $10.0 million and the recording of milestone revenue and sublicense expense of $2.3 million. We are not able to reasonably predict if and when any of the remaining milestones will be achieved.

In addition to the collaboration agreement, on July 1, 2015, we also entered into an equity agreement with Biogen. Under the terms of the equity agreement, Biogen purchased 1,453,957 shares of our common stock, at a purchase price equal to $20.63 per share, for an aggregate cash purchase price of $30.0 million. We received these cash proceeds from Biogen in August 2015. The shares issued to Biogen constitute restricted securities that may not be resold by Biogen other than in a transaction registered under the Securities Act of 1933, as amended, or pursuant to an exemption from such registration requirement.

***Bionic Sight, LLC***

In February 2017, we entered into a collaboration agreement with Bionic Sight to develop a gene therapy treatment to be used with Bionic Sight's innovative neuroprosthetic device and algorithm for retinal coding. Under the agreement, AGTC made an initial $2.0 million payment to Bionic Sight for an equity interest in that company. This initial investment represents an approximate 5 percent equity interest in Bionic Sight. In addition to the initial investment, we will contribute to ongoing research and development support costs through additional payments or other in-kind contributions. These payments and contributions will be made over time, up to the date that Bionic Sight has received both investigational new drug clearance from the 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".) If the IND Trigger is attained, we will receive additional equity, based on the valuation in place at the beginning of the agreement, for its cash and research and development contributions and will be obligated to purchase additional equity in Bionic Sight for $4.0 million upon the filing of the IND for the product candidate at a pre-determined valuation. Our collaborator, Bionic Sight, plans to file an IND for this product candidate in the first half of calendar 2019.

***Synpromics***

In December 2015, we entered into an agreement with Synpromics Limited ("Synpromics") to utilize Synpromics' proprietary technology to develop and optimize synthetic promoters. In the event that results from the research are promising, we have options to license promoters that result from the collaboration efforts.

***4DMT***

In April 2015, we signed an agreement with 4DMT to conduct research on optimized next generation capsids to target specific target cell populations within the human retina using 4DMT's *Directed Evolution* AAV vector discovery platform. If promising capsids result from the research, we have an option to enter into a licensing agreement.

**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 the future, we may generate revenue from a combination of: product sales, 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 any are 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.

We will adopt ASC 606, *Revenue from Contracts with Customers* ("Topic 606") as of July 1, 2018, the first day of our fiscal year 2019. The adoption of Topic 606 may impact the timing and pattern of revenue recognition; however, the quantification of such impact is ongoing. Upon adoption and in accordance with the disclosure requirements under Topic 606, we will report revenue amounts under Topic 606 but will continue to disclose the amount of revenue that would have been recognized under ASC 605, *Revenue Recognition*, during our fiscal year 2019. See Note 2 of the notes to the financial statements appearing elsewhere in this annual report on Form 10-K for additional commentary on the adoption of Topic 606.

We expect in fiscal year 2019 that revenues from our Biogen collaboration associated with amortization of non-refundable upfront fees will decrease under ASC 605 approximately $12.0 million compared to fiscal year 2018. This decrease is primarily due to the forecasted completion of the XLRS service period during fiscal year 2019, reaching the end of the XLRP service period in the first quarter of fiscal year 2018, and to a lesser extent, due to changes in estimates associated with the period of performance under the preclinical programs. We expect in fiscal year 2019 that milestone revenues recognized under the Biogen collaboration will increase under ASC 605 approximately $7.5 million compared to fiscal year 2018. In April 2018, the first patient was dosed in the XLRP Phase 1/2 clinical trial and we earned a $2.5 million milestone payment. In July 2018 the fourth patient was dosed in the XLRP Phase 1/2 clinical trial and we earned a $10.0 million milestone payment. We expect in fiscal year 2019 developmental services revenues from the Biogen collaboration will increase under ASC 605 by approximately $2.0 million compared to fiscal year 2018 due to the initiation of the XLRP Phase 1/2 clinical trial.

***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 revenues 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 as well as any additional clinical trials 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, we could be required to expend significant additional financial resources and time on the completion of clinical development.

From inception through June 30, 2018, we have incurred approximately $169.2 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. Additionally, we expect sublicensing fees to increase during the first quarter of fiscal 2019 due to earning a $10.0 million milestone payment from Biogen. We expect sublicensing fees to be approximately 23% of the Biogen milestone payment.

***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 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 and expense consists primarily of interest earned on cash and cash equivalents and our held-to-maturity investments.

**Critical accounting policies and estimates**

The following discussion of critical accounting policies identifies the accounting policies that require application of management's most difficult, subjective or complex judgments, often as a result of the need to make estimates about the effect of matters that are inherently uncertain and may change in subsequent periods. It is not intended to be a comprehensive list of all of our significant accounting policies, which are more fully described in Note 2 of the notes to the financial statements appearing elsewhere in this annual report on Form 10-K. In many cases, the accounting treatment of a particular transaction is specifically dictated by generally accepted accounting principles, with no need for management's judgment in their application. There are also areas in which the selection of an available alternative policy would not produce a materially different result.

***Revenue recognition***

We have generated revenue primarily through collaboration agreements, sponsored research arrangements with nonprofit organizations for the development and commercialization of product candidates and revenues from federal research and development grant programs. We recognize revenue when amounts are realized or realizable and earned. Revenue is considered realizable and earned when the following criteria are met: (1) persuasive evidence of an arrangement exists; (2) delivery has occurred or services have been rendered; (3) the price is fixed or determinable; and (4) collection of the amounts due are reasonably assured.

We evaluate the terms of sponsored research agreement grants and federal grants to assess our obligations and if our obligations are satisfied by the passage of time, revenue is recognized on a straight-line basis. In situations where the performance of our obligations has been satisfied when the grant is received, revenue is recognized upon receipt of the grant. Certain grants contain refund provisions. We review those refund provisions to determine the likelihood of repayment. If the likelihood of repayment of the grant is determined to be remote, the grant is recognized as revenue. If the probability of repayment is determined to be more than remote, we record the grant as a deferred revenue liability, until such time that the grant requirements have been satisfied.

*Collaboration revenue*

As described above, on July 1, 2015, we entered into a collaboration agreement with Biogen. The terms of this agreement contains multiple elements, or deliverables, which include, among others, (i) licenses, or options to obtain licenses, to our technology, and (ii) research and development activities to be performed on behalf of the collaborative partner. Payments made under this arrangement includes one or more of the following: non-refundable, up-front license fees; option exercise fees; funding of research and/or development efforts; milestone payments; and royalties on future product sales.

Multiple element arrangements are analyzed to determine whether the deliverables within the agreement can be separated or whether they must be accounted for as a single unit of accounting.

Deliverables under an agreement are required to be accounted for as separate units of accounting provided that (i) a delivered item has value to the customer on a stand-alone basis; and (ii) if the agreement includes a general right of return relative to the delivered item, delivery or performance of the undelivered item is considered probable and substantially in the control of the vendor. The allocation of consideration amongst the deliverables under the agreement is derived using a "best estimate of selling price" if vendor specific objective evidence and third-party evidence of fair value is not available. If the delivered element does not have stand-alone value or if the fair value of any of the undelivered elements cannot be determined, the arrangement is then accounted for as a single unit of accounting, and we recognize the consideration received under the arrangement as revenue on a straight-line basis over our estimated period of performance.

We determine the estimated selling price for deliverables within each agreement using vendor-specific objective evidence, or VSOE, of selling price, if available, third-party evidence, or TPE, of selling price if VSOE is not available, or best estimate of selling price, or BESP, if neither VSOE nor TPE are available. Determining the best estimate of selling price for a deliverable requires significant judgment. We use BESP to estimate the selling price related to licenses to our proprietary technology, since we often do not have VSOE or TPE of selling price for these deliverables. In those circumstances where we utilize BESP to determine the estimated selling price of a license to our proprietary technology, we consider market conditions as well as entity-specific factors, including those factors contemplated in negotiating the agreements as well as internally developed models that include assumptions related to the market opportunity, estimated development costs, probability of success and the time needed to commercialize a product candidate pursuant to the license. In validating our best estimate of selling price, we evaluate whether changes in the key assumptions used to determine the best estimate of selling price will have a significant effect on the allocation of arrangement consideration among multiple deliverables.

If the delivered element does not have stand-alone value or if the fair value of any of the undelivered elements cannot be determined, the arrangement is then accounted for as a single unit of accounting, and we recognize the consideration received under the arrangement as revenue on a straight-line basis over our estimated period of performance. Our anticipated periods of performance, typically the terms of our research and development obligations, are subject to estimates by management and may change over the course of the collaboration agreement. Such changes could have a material impact on the amount of revenue we record in future periods.

*Milestone revenue*

We apply the milestone method of accounting to recognize revenue from milestone payments when earned, as evidenced by written acknowledgement from the collaborator or other persuasive evidence that the milestone has been achieved and the payment is non-refundable, provided that the milestone event is substantive. A milestone event is defined as an event (i) that can only be achieved based in whole or in part on either our performance or on the occurrence of a specific outcome resulting from our performance; (ii) for which there is substantive uncertainty at the inception of the arrangement that the event will be achieved; and (iii) that would result in additional payments being due to us. Events for which the occurrence is either contingent solely upon the passage of time or the result of a counterparty's performance are not considered to be milestone events. A milestone event is substantive if all of the following conditions are met: (i) the consideration is commensurate with either our performance to achieve the milestone, or the enhancement of the value to the delivered item(s) as a result of a specific outcome resulting from our performance to achieve the milestone; (ii) the consideration relates solely to past performance; and (iii) the consideration is reasonable relative to all the deliverables and payment terms (including other potential milestone consideration) within the arrangement.

We assess whether a milestone is substantive at the inception of the arrangement. If a milestone is deemed non-substantive, we account for that milestone payment in accordance with the multiple element arrangements guidance and recognize revenue consistent with the related units of accounting for the arrangement over the related performance period.

***Research and development expenses***

Research and development costs 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 are required to 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 service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. The significant estimates in our accrued research and development expenses are related to expenses incurred with respect to academic research centers, CROs, and other vendors in connection with research and development activities for which we have not yet been invoiced.

There may be instances in which our service providers require advance payments at the inception of a contract or in which payments made to these vendors will exceed the level of services provided, resulting in a prepayment of the research and development expense. 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 Standard Codification ("ASC") Topic 718, *Compensation—Stock Compensation* ("ASC 718") generally recognize share-based compensation expense on a straight-line basis over the periods during which the employees and non-employee directors are required to provide service in exchange for the award. In addition, we issue stock options and restricted shares of common stock to non-employees in exchange for consulting services and account for these 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 non-employees are subject to periodic fair value re-measurement over their vesting terms.

For purposes of calculating stock-based compensation, we estimate the fair value of stock options using a Black-Scholes option-pricing model. The determination of the fair value of share-based payment awards utilizing the Black-Scholes model is affected by our stock price and a number of assumptions, including expected volatility, expected life, risk-free interest rate and expected dividends. The expected volatility is primarily based on the historical volatility of peer company data while the expected life of the stock options is based on historical and other economic data trended into the future. The risk-free interest rate assumption is based on observed interest rates appropriate for the expected terms of our stock options. The dividend yield assumption is based on our history and expectation of no dividend payouts. If factors change and we employ different assumptions, stock-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 stock-based compensation expense and the actual factors which become known over time, specifically with respect to anticipated forfeitures, we may change the input factors used in determining stock-based compensation costs for future grants. These changes, if any, may materially impact our results of operations in the period such changes are made.

***Income taxes***

We use the asset and liability method for accounting 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. Any interest and penalties related to uncertain tax positions will be reflected in income tax expense. The Company is subject to examination of its income tax returns in the federal and state income tax jurisdictions in which it operates. On December 28, 2015, the United States Internal Revenue Service, or IRS, notified the Company of an income tax audit for the tax period ending June 30, 2014. As of June 30, 2017, the IRS audit was closed and the Company incurred no penalties or payment liabilities for its income tax positions. For fiscal years ended June 30, 2018 and 2017, we recorded income tax expense related to uncertainties related to state income tax.

The Tax Cut and Jobs Act (the "Tax Act") was enacted on December 22, 2017. The Tax Act contains several key provisions including, among other things, reducing the U.S. federal corporate tax rate from 34% to 21%. We have enacted this reduction in tax rate effective January 1, 2018, and as such is using a blended rate for the fiscal year ended June 30, 2018. In addition, federal net operating losses ("NOLs") generated during future periods will be carried forward indefinitely, but will be subject to an 80% utilization against taxable income. We continue to evaluate the impact of the Tax Act and analyze additional guidance.

For the fiscal year ended June 30, 2018, the Company recorded an income tax provision, related to the Company's Federal alternative minimum tax credit and uncertainties related to state income tax. The Company calculates its alternative minimum taxable income (AMTI) using the alternative minimum tax ("AMT") system. The Company's federal income tax liability is the greater of the tax computed using the regular tax system or the tax under the AMT system. Corporations are exempt from AMT for all prior years in which their annual gross receipts for the 3-year period ending before the current tax year did not exceed $7.5 million. For the fiscal year ending June 30, 2017, the Company no longer qualifies for the small company exclusion. The AMT system limits the use of net operating losses used by taxpayers to offset taxable income.

***Recent Accounting Pronouncements***

In May 2017, the FASB issued Accounting Standards Update ("ASU") No. 2017-09, *Scope of Modification Accounting*, which amends ASC Topic 718, *Compensation – Stock Compensation*. The amendments in this Update provide guidance about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting in Topic 718. The amendments are effective for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years and early adoption is permitted. We are currently in the process of evaluating the impact of adoption of this standard on our financial statements.

In March 2016, the FASB issued ASU No. 2016-09, *Improvements to Employee Share-Based Payment Accounting*, which amends ASC Topic 718, *Compensation – Stock Compensation*. The amendments simplify several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities, forfeitures, and classification on the statement of cash flows. The amendments are effective for fiscal years beginning after December 15, 2016, and interim periods within those fiscal years and early adoption is permitted. We have adopted this standard for our 2018 fiscal year and it did not have a material effect on our balance sheets, results of operations or cash flows.

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)* in order to increase transparency and comparability among organizations by recognizing lease assets and lease liabilities on the balance sheet for those leases previously classified as operating leases under GAAP. The standard requires, in most instances, a lessee to recognize on its balance sheet a liability to make lease payments (the lease liability) and also a right-of-use asset representing its right to use the underlying asset for the lease term. The amendments are effective for fiscal years beginning after December 15, 2018, including interim periods within those periods, using a modified retrospective approach and early adoption is permitted. We are currently in the process of evaluating the impact of adoption of this standard on our financial statements.

In May 2014, the FASB issued ASU No. 2014-09, *Revenue from Contracts with Customers (Topic 606)* which replaces the existing accounting standards for revenue recognition with a single comprehensive five-step model. The core principle is to recognize revenue upon the transfer of goods or services to customers at an amount that reflects the consideration expected to be received. It also requires enhanced disclosures about revenue, provides guidance for transactions that were not previously addressed comprehensively, and improves guidance for multiple-element arrangements. The guidance applies to any entity that either enters into contracts with customers to transfer goods or services or enters into contracts for the transfer of nonfinancial assets unless those contracts are within the scope of other standards. Since its issuance, the FASB has amended several aspects of the new guidance, including provisions that address revenue recognition associated with the licensing of intellectual property. In July 2015, the FASB delayed the effective date of this guidance by one year. The guidance is now effective for public companies for annual periods beginning after December 15, 2017 as well as interim periods within those annual periods using either the full retrospective approach or modified retrospective approach.

We adopted Topic 606 as of July 1, 2018, the first day of our fiscal year 2019. The adoption of Topic 606 may impact the timing and pattern of revenue recognition; however, the quantification of such impact is ongoing. Upon adoption and in accordance with the disclosure requirements under Topic 606, we will report revenue amounts under Topic 606 but we will continue to disclose the amount of revenue that would have been recognized under ASC 605, *Revenue Recognition*, during our fiscal year 2019. See Note 2 of the notes to the financial statements appearing elsewhere in this annual report on Form 10-K for additional commentary on the adoption of Topic 606.

***Emerging growth company status***

The JOBS Act permits an "emerging growth company" such as ours to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. We have chosen to "opt out" of this provision and, as a result, we will comply with new or revised accounting standards as required when they are adopted. This decision to opt out of the extended transition period under the JOBS Act is irrevocable.

**Results of operations**

***Comparison of the fiscal years ended June 30, 2018 and 2017***

*Revenue*

| | Year ended June 30, | | Increase | % Increase |
|---|---|---|---|---|
| | 2018 | 2017 | (Decrease) | (Decrease) |
| | (dollars in thousands) | | | |
| Collaboration revenue | | | | |
| Amortization of upfront fees | $ 18,529 | $ 38,231 | (19,702) | (52)% |
| Development services | 3,028 | 1,051 | 1,977 | 188% |
| Milestone revenue | 2,500 | $ - | 2,500 | n/m |
| Total Collaboration revenue | $ 24,057 | $ 39,282 | $ (15,225) | (39)% |
| Grant revenue | 129 | 191 | (62) | (32)% |
| Total revenue | $ 24,186 | $ 39,473 | $ (15,287) | (39)% |

Total revenue for fiscal year 2018 decreased by $15.3 million to $24.2 million compared to fiscal year 2017 primarily due to the reduction in amortization of upfront fees. Non-refundable upfront fees received under our collaboration with Biogen are amortized to collaboration revenue on a straight-line basis over the estimated service period. Development services revenue primarily consists of reimbursement of Post-Funding Development Activities under the Biogen Collaboration. Amortization revenue decreased by $19.7 million during fiscal 2018 compared to fiscal 2017, primarily due to reaching the end of the XLRP service period during the first quarter of fiscal 2018, and to a lesser extent, due to changes in estimates associated with the period of performance under the XLRS and preclinical programs. Development services revenue increased $2.0 million during fiscal 2018 compared to fiscal 2017, primarily due to activities associated with the Phase 1/ 2 clinical trial for XLRP. In fiscal 2018 the first patient was dosed in the XLRP Phase 1/ 2 clinical trial and we earned a $2.5 million milestone payment. Grant revenue decreased by $62,000 during fiscal 2018 compared to fiscal 2017, largely attributable to reduced research and development activities on grant-funded projects

*Research and development expenses*

The following table summarizes our research and development expenses by product candidate or program for the fiscal year ended June 30, 2018 and 2017:

| | Year Ended June 30, | | Increase | % Increase |
|---|---|---|---|---|
| | 2018 | 2017 | (Decrease) | (Decrease) |
| | (dollars in thousands) | | | |
| External research and development expenses | | | | |
| ACHM | $ 4,202 | $ 3,699 | $ 503 | 14% |
| XLRS | 2,399 | 2,062 | 337 | 16% |
| XLRP | 2,688 | 1,788 | 900 | 50% |
| Research and discovery programs | 6,077 | 3,677 | 2,400 | 65% |
| Total external research and development expenses | 15,366 | 11,226 | 4,140 | 37% |
| Internal research and development expenses | | | | |
| Employee-related costs | 8,897 | 7,808 | 1,089 | 14% |
| Share-based compensation | 2,443 | 2,546 | (103) | (4)% |
| Other | 5,475 | 4,637 | 838 | 18% |
| Total internal research and development expenses | 16,815 | 14,991 | 1,824 | 12% |
| Total research and development expense | $ 32,181 | $ 26,217 | $ 5,964 | 23% |

External research and development costs consist of collaboration, licensing, manufacturing, testing, and other miscellaneous expenses that are directly attributable to our most advanced product candidates and discovery programs. We do not allocate personnel-related costs, including stock-based compensation, costs associated with broad technology platform improvements or other indirect costs, to specific programs, as they are deployed across multiple projects under development and, as such, are separately classified as internal research and development expenses in the table above.

Research and development expenses for fiscal 2018 were $32.2 million, compared to $26.2 million for fiscal 2017, an increase of $6.0 million, or 23%. This increase was primarily attributable to:

- $2.4 million of increased external spending on general research and discovery programs primarily due to increased spending on the optogenetics, otology, and adrenoleukodystrophy (ALD) preclinical programs and increased spending on general research activities in ophthalmology to support ongoing clinical programs;
- $1.1 million of increased employee-related expenses associated with the hiring of additional employees to support clinical trial execution and research and development activities;
- $0.9 million of increased external spending on the XLRP program primarily due to initiating a Phase 1/2 clinical trial in fiscal year 2018 and recording of sublicense fees of $0.6 million associated with achieving a $2.5 million milestone payment from Biogen
- $0.8 million of increased spending on the other R&D activities including, consulting fees, laboratory supplies, travel and depreciation expense;
- $0.5 million of increased external spending on the ACHM B3 and A3 Phase 1/2 clinical trials primarily due to increased clinical trial activities associated with higher patient enrollment and increased manufacturing activities;
- $0.3 million of increased external spending on the XLRS program primarily related to increased patient enrollment in the ongoing Phase 1/2 clinical trial.

*General and administrative expenses*

| | Year Ended June 30, | | Increase | % Increase |
|---|---|---|---|---|
| | 2018 | 2017 | (Decrease) | (Decrease) |
| | (dollars in thousands) | | | |
| Employee-related costs | $ 5,557 | $ 3,952 | $ 1,605 | 41% |
| Share-based compensation | 2,751 | 3,060 | (309) | (10)% |
| Legal and professional fees | 437 | 1,058 | (621) | (59)% |
| Other general and administrative expenses | 5,644 | 3,284 | 2,360 | 72% |
| Total general and administrative expenses | $ 14,389 | $ 11,354 | $ 3,035 | 27% |

General and administrative expenses for the fiscal 2018 were $14.4 million, compared to $11.4 million for fiscal 2017, an increase of $3.0 million, or 27%. The increase was primarily driven by higher employee-related costs and other expenses which resulted from hiring additional employees to support our continued expansion. The increase in other expenses of $2.4 million includes increases in consulting, accounting, state taxes and the provision for uncollectible accounts. The increased expenses were partially offset by lower share-based compensation expenses and legal and professional fees.

*Other income (expense), net*

For fiscal year 2018, other income (expense), net, which was primarily comprised of investment income, increased to $1.2 million from $0.9 million generated in 2017 due largely to modestly better investment performance.

*Provision for income taxes*

Income tax expense was $72,000 for the year ended June 30, 2018 compared to income tax expense of $2.4 million for the year ended June 30, 2017. The fiscal year 2018 income tax expense results from an income tax benefit that was recorded for the AMT paid in the fiscal year 2017 that became refundable under the Tax Act and an increase in the Company's uncertain tax provision liability. The fiscal year 2017 income tax results from the recognition of revenue related to the Biogen agreement for tax purposes, which is accelerated compared to our GAAP revenue, resulting in significantly more taxable income than GAAP net income. While our taxable income is largely offset by the use of NOLs, our income tax expense is primarily due to federal alternative minimum tax expense, the apportionment of income to certain state jurisdictions where we do not have NOLs and the recognition of a reserve for uncertain tax positions. taxable income is largely offset by the use of NOLs, our income tax expense is primarily due to federal alternative minimum tax expense, the apportionment of income to certain state jurisdictions where we do not have NOLs and the recognition of a reserve for uncertain tax positions.

***Comparison of the fiscal years ended June 30, 2017 and 2016***

*Revenue*

| | Year ended June 30, | | Increase | % Increase |
|---|---|---|---|---|
| | 2017 | 2016 | (Decrease) | (Decrease) |
| | (dollars in thousands) | | | |
| Collaboration revenue | | | | |
| Amortization of upfront fees | $ 38,231 | $ 41,751 | (3,520) | (8)% |
| Development services | 1,051 | - | 1,051 | n/m |
| Milestone revenue | - | 5,000 | (5,000) | n/m |
| Total Collaboration revenue | $ 39,282 | $ 46,751 | $ (7,469) | (16)% |
| Grant revenue | 191 | 610 | (419) | (69)% |
| Total revenue | $ 39,473 | $ 47,361 | $ (7,888) | (17)% |

Total revenue for fiscal year 2017 decreased by $7.9 million to $39.5 million compared to fiscal year 2016. This decrease is primarily due to recognizing $5.0 million of milestone revenue in fiscal year 2016 associated with achieving a XLRS patient enrollment milestone under our collaboration arrangement with Biogen, and to a lesser extent, due to reduced revenue associated with the amortization of upfront fees associated with the Biogen collaboration due to certain delays which have extended our estimate of period of performance and reduced research and development activities under grant-funded projects.

*Research and development expenses*

| | Year Ended June 30, | | Increase | % Increase |
|---|---|---|---|---|
| | 2017 | 2016 | (Decrease) | (Decrease) |
| | (dollars in thousands) | | | |
| External research and development expenses | | | | |
| ACHM | $ 3,699 | $ 3,648 | $ 51 | 1% |
| XLRS | 2,062 | 9,237 | (7,175) | (78)% |
| XLRP | 1,788 | 8,604 | (6,816) | (79)% |
| Research and discovery programs | 3,677 | 6,911 | (3,234) | (47)% |
| Total external research and development expenses | 11,226 | 28,400 | (17,174) | (60)% |
| Internal research and development expenses | | | | |
| Employee-related costs | 7,808 | 5,631 | 2,177 | 39% |
| Share-based compensation | 2,546 | 2,147 | 399 | 19% |
| Other | 4,637 | 3,198 | 1,439 | 45% |
| Total internal research and development expenses | 14,991 | 10,976 | 4,015 | 37% |
| Total research and development expense | $ 26,217 | $ 39,376 | $ (13,159) | (33)% |

Research and development expenses for fiscal year 2017 decreased by $13.2 million to $26.2 million compared to fiscal year 2016. This decrease was primarily driven by a $12.0 million reduction in sublicense expenses associated with our collaboration arrangement with Biogen on the XLRS and XLRP programs and a $3 million reduction in licensing and milestones fees paid to 4D Molecular Therapeutics and Synpromics Limited associated with our research and discovery programs. These decreases were partially offset by increased employee-related expenses related to the hiring of additional employees to support clinical trial execution and research and development activities.

*General and administrative expenses*

| | Year Ended June 30, | | Increase | % Increase |
|---|---|---|---|---|
| | 2017 | 2016 | (Decrease) | (Decrease) |
| | (dollars in thousands) | | | |
| Employee-related costs | $ 3,952 | $ 2,827 | $ 1,125 | 40% |
| Share-based compensation | 3,060 | 2,860 | 200 | 7% |
| Legal and professional fees | 1,058 | 1,144 | (86) | (8)% |
| Other | 3,284 | 3,243 | 41 | 1% |
| Total general and administrative expenses | $ 11,354 | $ 10,074 | $ 1,280 | 13% |

General and administrative expenses for fiscal year 2017 increased by $1.3 million to $11.4 million compared to fiscal year 2016. The increase was primarily driven by the hiring of additional employees which resulted in higher share-based compensation and other employee-related costs.

*Other income (expense), net*

For fiscal year 2017 other income (expense), net, which was primarily comprised of investment income, increased to $0.9 million from $0.7 million generated in 2016 due largely to modestly better investment performance.

**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, 2018, we had an accumulated deficit of $110.9 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 fiscal year 2016, we received a non-refundable upfront cash payment of $94.0 million under our collaboration arrangement with Biogen. Contemporaneous with this collaboration arrangement, we also entered into an equity agreement with Biogen under which we received an additional $30.0 million in cash in exchange for 1,453,957 shares of common stock that we issued to Biogen at a purchase price of $20.63 per share. During fiscal year 2017, we made an initial $2.0 million payment to Bionic Sight for an equity interest in that company. 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 24 months. As of June 30, 2018, we had cash and cash equivalents and investments of $104.9 million.

As of June 30, 2018, our cash and cash equivalents were held in bank accounts and money market funds, while our investments consisted of certificates of deposit and corporate and government bonds, none of which mature more than 12 months after the balance sheet date, consistent with our investment policy that seeks to maintain adequate liquidity and preserve capital.

***Cash flows***

The following table sets forth the primary sources and uses of cash for each of the periods set forth below:

| | Year Ended June 30, | | |
|---|---|---|---|
| | 2018 | 2017 | 2016 |
| | (in thousands) | | |
| Net cash provided by (used in): | | | |
| Operating activities | $ (32,520) | $ (31,001) | $ 70,991 |
| Investing activities | 33,015 | 32,811 | (100,804) |
| Financing activities | (136) | 28 | 19,494 |
| Net (decrease) increase in cash and cash equivalents | $ 359 | $ 1,838 | $ (10,319) |

***Operating activities.*** Cash used in operating activities of $32.5 million for the fiscal year ended June 30, 2018 was primarily due to changes in operating assets and liabilities of $18.4 million, partially offset by non-cash items of $7.4 million, including $5.2 million in stock-based compensation expense, and a net loss of $21.3 million. Cash used in operating activities of $31.0 million during the fiscal year ended June 30, 2017 was primarily due to changes in operating assets and liabilities of $38.4 million, partially offset by non-cash items of $7.0 million, including $5.6 million of stock-based compensation expense, and net income of $0.4 million. For both fiscal 2018 and 2017, the changes in operating assets and liabilities was primarily due to the amortization of upfront fees associated with our Biogen collaboration. For fiscal year 2016, net cash provided by operating activities of $71.0 million was primarily associated with the upfront cash proceeds of $104.8 million received in connection with the entry into our collaboration with Biogen, which included an allocation of $10.8 million from the equity agreement, and a milestone payment from Biogen of $5.0 million. These proceeds were partially offset by the impact of our net loss, including the amortization of deferred revenue and payments to certain research partner institutions in the aggregate amount of $12.0 million for sub-license, milestone and other costs which were all associated with the Biogen collaboration, and changes during the period in our working capital accounts.

***Investing activities.*** Cash provided by investing activities of $33.1 million for the fiscal year ended June 30, 2018 was primarily due to maturity of investments of $100.9 million, partially offset by purchases of investments of $67.1 million and the purchase of property and equipment and intellectual property of $0.8 million. Cash provided by investing activities of $32.8 million for the fiscal year ended June 30, 2017 was primarily due to maturity of investments of $103.2 million, partially offset by purchases of investments of $67.5 million, a $2.0 million payment to Bionic Sight for an equity interest in that company and purchases of property and equipment of $0.7 million. For the fiscal year 2016, net cash used in investing activities consisted primarily of cash outflows of $208.2 million related to the purchase of investments and $2.6 million related to the purchase of property and equipment and the acquisition of intellectual property, including leasehold improvements at our new facility in Alachua, Florida. These cash outflows were partially offset by $110.0 million of proceeds from the maturity of investments.

***Financing activities***. Net cash used in financing activities was $136,000 for the fiscal year 2018. Net cash provided by financing was $28,000 for the fiscal year 2017. 2018 net cash used consisted of cash outlays for deferred offering costs and include the proceeds received from the exercise of stock option awards, while 2017included the proceeds received from the exercise of stock options. Net cash provided by financing activities during fiscal year 2016 was $19.5 million and consisted of $19.2 million of cash received in connection with our sale of shares of common stock to Biogen pursuant to the equity agreement executed on July 1, 2015 and $283,000 of cash received in connection with the exercise of stock options.

***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 at June 30, 2018, will be will be sufficient to allow us to generate data from our ongoing clinical programs, to move our pre-clinical optogenetic program in collaboration with Bionic Sight into the clinic and to fund our currently planned research and discovery programs for at least the next two years. 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.

We have based our projections of operating capital requirements on assumptions that may prove to be incorrect and we may use all of our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical products, we are unable to estimate the exact amount of our operating capital requirements. Our future funding requirements will depend on many factors, including, but not limited to:

- the timing and costs our planned clinical trials for our XLRS and ACHM product candidates;
- the timing and costs of our planned preclinical studies of our XLRP product candidate;
- 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 partners;
- the initiation, progress, timing, costs and results of preclinical studies relating to potential applications of our gene therapy platform in other indications;
- our success in scaling our manufacturing method and expanding our manufacturing capabilities;
- the number and characteristics of product candidates that we pursue;
- the outcome, timing and costs of seeking regulatory approvals;
- subject to receipt of marketing approval, revenue received from commercial sales of our product candidates;
- the terms and timing of any future collaborations, licensing, consulting or other arrangements that we may establish;
- the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, defense and enforcement of any patents or other intellectual property rights;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims; and
- the extent to which we in-license or acquire other products and technologies.
- the timing of payments for our additional equity purchase obligations under the Bionic Sight collaboration agreement

***Contractual obligations and commitments***

Our current leased facilities encompass approximately 21,000 square feet of laboratory and office space in Alachua, Florida under a lease arrangement that will expire in December 31, 2025. In addition, we occupy approximately 8,000 square feet of office and laboratory space in Cambridge, Massachusetts. On July 31, 2017, we entered into a new lease to increase our office and laboratory space in Cambridge by approximately 5,000 square feet to a total of approximately 8,000 square feet and extend the term of the lease for an additional seven years, with an option to further extend the lease for one additional three-year term.

*Contingent contractual obligations*

We also have obligations arising under our license agreements to make future payments to third parties that become due and payable on the achievement of certain development, regulatory and commercial milestones (such as the start of a clinical trial, filing of a Biologics License Application, or BLA, approval by the FDA or product launch). We have not included these obligations on our balance sheet or in the table above because the achievement and timing of these milestones is not fixed nor determinable. These obligations include:

- Under each of our various licenses with the University of Florida Research Foundation, or UFRF, covering the AAV construct containing the AAT gene and the method to treat AAT deficiency using this construct, a small cone cell specific promoter, and the use of engineered capsids and under our joint license with UFRF and Johns Hopkins University covering a particular HSV construct and various compositions thereof, we will be required to make payments based upon development, regulatory and commercial milestones for any products covered by the in-licensed intellectual property. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property. We have the right to sublicense our rights under this agreement, and we will be required to pay a percentage of such license income. We are required to make annual maintenance payments under these licenses, which payments are creditable against royalty payments on a year-by-year basis.
- Under our license agreement with the UAB Research Foundation pursuant to which we license a patent covering the use of HSV helpers to produce AAV vectors, we will be required to make payments based upon development and regulatory milestones for any products covered by the in-licensed intellectual property. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property. We have the right to sublicense our rights under this agreement, and we will be required to pay a percentage of such license income. We are required to make annual maintenance payments under this license, which payments are creditable against royalty payments on a year-by-year basis.

If any of our product candidates that utilize technology licensed under these agreements reached commercialization, we will be obligated to make royalty payments ranging from 0.5% to 4.0% of our net sales of the applicable product. We are responsible for a portion of the costs related to the preparation, filing, issuance, prosecution and maintenance of the patents covered by the license agreements.

We enter into contracts in the normal course of business with contract research organizations for preclinical research studies, research supplies and other services and products for operating purposes. These contracts generally provide for termination on notice, and therefore are cancelable contracts.

**Off-balance sheet arrangements**

We did not have during the periods presented, and we do not currently 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

**Interest Rate Sensitivity**

Our financial instruments at June 30, 2018 consisted primarily of cash and cash equivalents and investments totaling $104.9 million. These financial instruments are exposed to the impact of interest rate changes which may result in fluctuations to our interest income. Due to the nature of our investments in money market funds, certificates of deposits, and debt instruments of corporations and U.S. government agencies, all of which generally mature within a two-year period of their purchase date, the estimated fair values of these financial instruments approximate their carrying amounts at June 30, 2018.

We maintain our investment portfolio in accordance with our investment policy. The primary objectives of this investment policy are to maintain adequate liquidity, preserve capital and to meet our operating needs. Although our investments are subject to credit risk, our investment policy specifies credit quality standards for our investments and limits the amount of credit exposure from any single issue, issuer or type of investment. Our investments are also subject to interest rate risk and may decrease in value if market interest rates increase. However, due to the conservative nature of our investments and relatively short investment periods, we believe interest rate risk is mitigated and an immediate 10% increase in interest rates would not have a material effect on the fair market value of our portfolio. We do not own derivative financial instruments. Accordingly, we do not believe that there is any material market risk exposure with respect to derivative or other financial instruments.

**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 sheet of Applied Genetic Technologies Corporation (the Company) as of June 30, 2018, the related statements of operations, stockholders' equity and cash flows for the year then ended, 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, 2018, and the results of its operations and its cash flows for the year then ended in conformity with U.S. generally accepted accounting principles.

**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 audit. 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 audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether 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 audit 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 audit 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 audit 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 audit provides a reasonable basis for our opinion.

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

Tampa, Florida
September 10, 2018

**Report of Independent Registered Public Accounting Firm**

The Board of Directors and Stockholders of Applied Genetic Technologies Corporation

We have audited the accompanying balance sheet of Applied Genetic Technologies Corporation (the Company) as of June 30, 2017, and the related statements of operations, stockholders' equity (deficit) and cash flows for each of the two years in the period ended June 30, 2017. Our audits also included the financial statement schedule of the Company listed in Item 15(a) as of and for the years ended June 30, 2017 and 2016. These financial statements and financial statement schedule are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements and schedule based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Applied Genetic Technologies Corporation as of June 30, 2017, and the results of its operations and its cash flows for each of the two years in the period ended June 30, 2017, in conformity with U.S. generally accepted accounting principles. Also, in our opinion, the related financial statement schedule, when considered in relation to the basic financial statements taken as a whole, presents fairly in all material respects the information set forth therein.

/S/ RSM US LLP
Raleigh, North Carolina
September 13, 2017

**APPLIED GENETIC TECHNOLOGIES CORPORATION**
**BALANCE SHEETS**

| In thousands, except per share data | June 30, 2018 | June 30, 2017 |
|---|---|---|
| **ASSETS** | | |
| **Current assets:** | | |
| Cash and cash equivalents | $ 31,065 | $ 30,706 |
| Investments | 73,840 | 95,994 |
| Grants receivable | 210 | 174 |
| Prepaid and other current assets | 4,009 | 3,361 |
| Total current assets | 109,124 | 130,235 |
| Investments, net of current portion | - | 11,749 |
| Property and equipment, net | 5,254 | 2,661 |
| Intangible assets, net | 968 | 1,219 |
| Investment in Bionic Sight | 1,980 | 2,000 |
| Other assets | 1,206 | 59 |
| **Total assets** | $ 118,532 | $ 147,923 |
| **LIABILITIES AND STOCKHOLDERS' EQUITY** | | |
| **Current liabilities:** | | |
| Accounts payable | $ 945 | $ 998 |
| Accrued and other liabilities | 7,155 | 6,162 |
| Deferred revenue | 6,295 | 20,996 |
| Total current liabilities | 14,395 | 28,156 |
| Deferred revenue, net of current portion | 610 | 4,438 |
| Other long-term liabilities | 4,345 | - |
| **Total liabilities** | 19,350 | 32,594 |
| **Stockholders' equity:** | | |
| Common stock, par value $.001 per share, 150,000 shares authorized; 18,137 and 18,088 shares issued; 18,126 and 18,088 shares outstanding at June 30, 2018 and 2017, respectively | 18 | 18 |
| Additional paid-in capital | 210,139 | 204,937 |
| Shares held in treasury of: 11 and 0 at June 30, 2018 and 2017 respectively | (49) | - |
| Accumulated deficit | (110,926) | (89,626) |
| Total stockholders' equity | 99,182 | 115,329 |
| **Total liabilities and stockholders' equity** | $ 118,532 | $ 147,923 |

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

**APPLIED GENETIC TECHNOLOGIES CORPORATION**
**STATEMENTS OF OPERATIONS**

| In thousands, except per share amounts | Years ended June 30, 2018 | 2017 | 2016 |
|---|---|---|---|
| **Revenue:** | | | |
| Collaboration revenue | $ 24,057 | $ 39,282 | $ 46,751 |
| Grant revenue | 129 | 191 | 610 |
| Total revenue | 24,186 | 39,473 | 47,361 |
| **Operating expenses:** | | | |
| Research and development | 32,181 | 26,217 | 39,376 |
| General and administrative | 14,389 | 11,354 | 10,074 |
| Total operating expenses | 46,570 | 37,571 | 49,450 |
| **Income (loss) from operations** | (22,384) | 1,902 | (2,089) |
| **Other income (expense):** | | | |
| Investment income, net | 1,301 | 952 | 711 |
| Other expense | (125) | (47) | (3) |
| Total other income, net | 1,176 | 905 | 708 |
| **Income (loss) before provision for income taxes** | (21,208) | 2,807 | (1,381) |
| Provision for income taxes | 72 | 2,400 | - |
| **Income (loss) before equity in net losses of affiliate** | (21,280) | 407 | (1,381) |
| Equity in net losses of affiliate | (20) | - | - |
| **Net income (loss)** | $ (21,300) | $ 407 | $ (1,381) |
| **Net income (loss) per share, basic** | $ (1.18) | $ 0.02 | $ (0.08) |
| **Net income (loss) per share, diluted** | $ (1.18) | $ 0.02 | $ (0.08) |
| **Weighted average shares outstanding, basic** | 18,105 | 18,072 | 17,810 |
| **Weighted average shares outstanding, diluted** | 18,105 | 18,385 | 17,810 |

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

**APPLIED GENETIC TECHNOLOGIES CORPORATION**
**STATEMENTS OF STOCKHOLDERS' EQUITY**

| In thousands | Common Stock Outstanding Shares | Common Stock Amount | Treasury Stock Outstanding Shares | Treasury Stock Amount | Additional Paid-in Capital | Accumulated Deficit | Total |
|---|---|---|---|---|---|---|---|
| **Balance, June 30, 2015** | 16,491 | $ 16 | - | $ - | $ 174,168 | $ (88,652) | $ 85,532 |
| Issuance of common stock, net of issuance costs | 1,494 | 2 | - | - | 19,805 | - | 19,807 |
| Share-based compensation expense | - | - | - | - | 5,007 | - | 5,007 |
| Shares issued under employee plans | 59 | - | - | - | 283 | - | 283 |
| Exercise of Warrants | 9 | - | - | - | 40 | - | 40 |
| Net loss | - | - | - | - | - | (1,381) | (1,381) |
| **Balance, June 30, 2016** | 18,053 | $ 18 | - | $ - | $ 199,303 | $ (90,033) | $ 109,288 |
| Share-based compensation expense | - | - | - | - | 5,606 | - | 5,606 |
| Shares issued under employee plans | 31 | - | - | - | 28 | - | 28 |
| Exercise of Warrants | 4 | - | - | - | - | - | - |
| Net income | - | - | - | - | - | 407 | 407 |
| **Balance, June 30, 2017** | 18,088 | 18 | - | $ - | $ 204,937 | $ (89,626) | $ 115,329 |
| Share-based compensation expense | - | - | | - | 5,193 | - | 5,193 |
| Shares issued under employee plans | 49 | - | 11 | (49) | 9 | - | (40) |
| Net loss | - | - | - | - | - | (21,300) | (21,300) |
| **Balance, June 30, 2018** | 18,137 | 18 | 11 | $ (49) | $ 210,139 | $ (110,926) | $ 99,182 |

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

**APPLIED GENETIC TECHNOLOGIES CORPORATION**
**STATEMENTS OF CASH FLOWS**

| In thousands | Years ended June 30, 2018 | 2017 | 2016 |
|---|---|---|---|
| **Cash flows from operating activities** | | | |
| Net income (loss) | $ (21,300) | $ 407 | $ (1,381) |
| Adjustments to reconcile net income (loss) to net cash (used in) operating activities: | | | |
| Share-based compensation expense | 5,193 | 5,606 | 5,007 |
| Share-based collaboration expense | - | - | 636 |
| Depreciation and amortization | 1,175 | 912 | 567 |
| Investment premium accretion | 85 | 402 | 448 |
| Provision for bad debts | 375 | - | - |
| Equity in net losses of affiliate | 20 | - | - |
| Loss on disposal of property, plant and equipment | 1 | 47 | - |
| Loss on disposal of intangible assets | 126 | - | - |
| Changes in operating assets and liabilities: | | | |
| (Increase) decrease in grants receivable | (36) | 780 | 409 |
| Increase in prepaid and other assets | (1,903) | (240) | (1,562) |
| Increase (decrease) in accounts payable | (53) | (333) | 140 |
| Increase (decrease) in deferred revenues | (18,529) | (38,230) | 63,664 |
| Increase (decrease) in accrued and other liabilities | 2,326 | (352) | 3,063 |
| Net cash (used in) provided by operating activities | (32,520) | (31,001) | 70,991 |
| **Cash flows from investing activities** | | | |
| Purchase of property and equipment | (662) | (739) | (2,471) |
| Purchase of and capitalized costs related to intangible assets | (141) | (152) | (121) |
| Investment in Bionic Sight | - | (2,000) | - |
| Maturity of investments | 100,900 | 103,244 | 109,968 |
| Purchase of investments | (67,082) | (67,542) | (208,180) |
| Net cash provided by (used in) investing activities | 33,015 | 32,811 | (100,804) |
| **Cash flows from financing activities** | | | |
| Proceeds from exercise of common stock options | 9 | 28 | 283 |
| Proceeds from issuance of common stock, net of issuance costs | - | - | 19,211 |
| Payments made toward capital lease obligations | (43) | - | - |
| Deferred offering costs | (102) | - | - |
| Net cash (used in) provided by financing activities | (136) | 28 | 19,494 |
| **Net increase(decrease) in cash and cash equivalents** | 359 | 1,838 | (10,319) |
| **Cash and cash equivalents, beginning of year** | 30,706 | 28,868 | 39,187 |
| **Cash and cash equivalents, end of year** | $ 31,065 | $ 30,706 | $ 28,868 |
| **Supplemental disclosure of non-cash financing activities** | | | |
| Cash paid during the year for income taxes | $ 617 | $ 887 | $ - |
| Capital lease obligation related to the purchase of equipment | $ 240 | $ - | $ - |
| Lease incentive obligation related to the purchase of leasehold improvements | $ 2,588 | $ - | $ - |
| Costs related to future offering costs in accounts payable and accrued liabilities | $ 163 | $ - | $ - |
| Issuance of restricted stock for no consideration | $ 49 | $ - | $ - |

The accompanying notes are an integral part of the 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.

In July 2015, the Company entered into a collaboration agreement (the "Collaboration Agreement") with Biogen MA, Inc., a wholly owned subsidiary of Biogen Inc. ("Biogen"), pursuant to which the Company and Biogen will collaborate 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 Collaboration Agreement became effective in August 2015. The Collaboration Agreement and other transactions with Biogen are discussed further in Note 7 to these 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, 2018, the Company had an accumulated deficit of $110.9 million. While the Company expects to continue to generate some revenue from partnering, including under the collaboration with Biogen, 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, and collaborations. At June 30, 2018, the Company had cash and cash equivalents and investments of $104.9 million.

**(2) Summary of Significant Accounting Policies:**

***Basis of Presentation***

The accompanying financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP") and, in the opinion of management, include all adjustments necessary for a fair presentation of the Company's financial position, results of operations, and cash flows for each period presented.

***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 in making decisions regarding resource allocation and assessing performance. To date, we have viewed our operations and managed our business as one segment.

***Use of estimates***

The preparation of financial statements in conformity with GAAP 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 amount of revenues and expenses during the 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 consist 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 as of each balance sheet date. Debt securities are classified as held-to-maturity when the Company 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. Interest on securities classified as held-to-maturity is included in investment income.

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 the entire amortized cost. Once a decline in fair value is determined to be other-than-temporary, an impairment charge is recorded to other income (expense) and a new cost basis in the investment is established.

***Concentrations of Credit Risk***

The Company maintains its cash and cash equivalents and certificates of deposit with two financial institutions that are federally insured. Some of these financial instruments are in excess of 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 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, in line 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 they are 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 single drug.

***Fair value of financial instruments***

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

To the extent that 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 in determining fair value is greatest for 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, consisting of laboratory equipment, furniture and fixtures, computer equipment and leasehold improvements, are recorded at cost. Depreciation is computed using the straight-line method over the estimated useful lives of the assets, which are generally three to ten years. The weighted average useful life is 6.7 years. Leasehold improvements are stated at cost and are amortized over the shorter of the estimated useful lives of the assets or the 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.

***Intangible assets***

Intangible assets primarily include 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, and writes off any costs associated with patents that are no longer being actively pursued or that have no future benefit.

Amortization expense is computed using the straight-line method over the estimated useful lives of the assets, which are generally eight to twenty years. The weighted average amortization period is 12.4 years. 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 written off to expense.

***Impairment of long-lived assets***

The Company reviews its long-lived assets for impairment when impairment indicators are present. If impairment indicators exist, management determines whether impairment in value has occurred by comparing the estimated undiscounted cash flows from future operations with the carrying values of the assets. Management considers several indicators in assessing impairment, including trends and prospects, as well as the effects of obsolescence, demand, competition and other economic factors. No impairment charges were recorded for each of the fiscal years ended June 30, 2018, 2017, and 2016.

***Revenue recognition***

The Company has primarily generated revenue through collaboration agreements, sponsored research arrangements with nonprofit organizations for the development and commercialization of product candidates and revenues from federal research and development grant programs. The Company recognizes revenue when amounts are realized or realizable and earned. Revenue is considered realizable and earned when the following criteria are met: (1) persuasive evidence of an arrangement exists; (2) delivery has occurred or services have been rendered; (3) the price is fixed or determinable; and (4) collection of the amounts due are reasonably assured.

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue in the Company's balance sheets. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as current liabilities. The Company recognizes revenue for reimbursements of research and development costs under collaboration agreements as the services are performed. The Company records these reimbursements as revenue and not as a reduction of research and development expenses, as the Company has the risks and rewards as the principal in the research and development activities.

The Company evaluates the terms of sponsored research agreement grants and federal grants to assess the Company's obligations and if the Company's obligations are satisfied by the passage of time, revenue is recognized on a straight-line basis. In situations where the performance of the Company's obligations has been satisfied when the grant is received, revenue is

recognized upon receipt of the grant. Certain grants contain refund provisions. The Company reviews those refund provisions to determine the likelihood of repayment. If the likelihood of repayment of the grant is determined to be remote, the grant is recognized as revenue. If the probability of repayment is determined to be more than remote, the Company records the grant as a deferred revenue liability, until such time that the grant requirements have been satisfied.

*Collaboration revenue*

On July 1, 2015, the Company entered into a Collaboration Agreement with Biogen. This collaboration is discussed further in Note 7 of notes to the financial statements. The terms of this agreement contains multiple elements, or deliverables, which may include, among others, (i) licenses, or options to obtain licenses, to its technology, and (ii) research and development activities to be performed on behalf of the collaborative partner. Payments made under this arrangement includes one or more of the following: non-refundable, up-front license fees; option exercise fees; funding of research and/or development efforts; milestone payments; and royalties on future product sales.

Multiple element arrangements are analyzed to determine whether the deliverables within the agreement can be separated or whether they must be accounted for as a single unit of accounting. Deliverables under an agreement are required to be accounted for as separate units of accounting provided that (i) a delivered item has value to the customer on a stand-alone basis; and (ii) if the agreement includes a general right of return relative to the delivered item, delivery or performance of the undelivered item is considered probable and substantially in the control of the Company. The allocation of consideration amongst the deliverables under the agreement is derived using a “best estimate of selling price” if vendor specific objective evidence and third-party evidence of fair value is not available. If the delivered element does not have stand-alone value or if the fair value of any of the undelivered elements cannot be determined, the arrangement is then accounted for as a single unit of accounting, and the Company recognizes the consideration received under the arrangement as revenue on a straight-line basis over the estimated period of performance.

The Company determines the estimated selling price for deliverables within each agreement using vendor-specific objective evidence, or VSOE, of selling price, if available, third-party evidence, or TPE, of selling price if VSOE is not available, or best estimate of selling price, or BESP, if neither VSOE nor TPE are available. Determining the best estimate of selling price for a deliverable requires significant judgment. The Company uses BESP to estimate the selling price related to licenses to its proprietary technology, since it often does not have VSOE or TPE of selling price for these deliverables. In those circumstances where it utilizes BESP to determine the estimated selling price of a license to our proprietary technology, the Company considers market conditions as well as entity-specific factors, including those factors contemplated in negotiating the agreements as well as internally developed models that include assumptions related to the market opportunity, estimated development costs, probability of success and the time needed to commercialize a product candidate pursuant to the license. In validating its best estimate of selling price, the Company evaluates whether changes in the key assumptions used to determine the best estimate of selling price will have a significant effect on the allocation of arrangement consideration among multiple deliverables.

If the delivered element does not have stand-alone value or if the fair value of any of the undelivered elements cannot be determined, the arrangement is then accounted for as a single unit of accounting, and the Company recognizes the consideration received under the arrangement as revenue on a straight-line basis over our estimated period of performance. The Company’s anticipated periods of performance, typically the terms of our research and development obligations, are subject to estimates by management and may change over the course of the collaboration agreement. Such changes could have a material impact on the amount of revenue we record in future periods.

*Milestone revenue*

The Company applies the milestone method of accounting to recognize revenue from milestone payments when earned, as evidenced by written acknowledgement from the collaborator or other persuasive evidence that the milestone has been achieved and the payment is non-refundable, provided that the milestone event is substantive. A milestone event is defined as an event (i) that can only be achieved based in whole or in part on either the Company’s performance or on the occurrence of a specific outcome resulting from the Company’s performance; (ii) for which there is substantive uncertainty at the inception of the arrangement that the event will be achieved; and (iii) that would result in additional payments being due to the Company. Events for which the occurrence is either contingent solely upon the passage of time or the result of a counterparty’s performance are not considered to be milestone events. A milestone event is substantive if all of the following conditions are met: (i) the consideration is commensurate with either the Company’s performance to achieve the milestone, or the enhancement of the value to the delivered item(s) as a result of a specific outcome resulting from the Company’s performance to achieve the

milestone; (ii) the consideration relates solely to past performance; and (iii) the consideration is reasonable relative to all the deliverables and payment terms (including other potential milestone consideration) within the arrangement.

The Company assesses whether a milestone is substantive at the inception of the arrangement. If a milestone is deemed non-substantive, the Company accounts for that milestone payment in accordance with the multiple element arrangements guidance and recognizes revenue consistent with the related units of accounting for the arrangement over the related performance period.

During the fiscal years ended June 30, 2018 and June 30, 2016, the Company recognized milestone revenue in the amount of $2.5 and $5.0 million, respectively. No milestone revenues were recognized during the fiscal years ended June 30, 2017.

***Income taxes***

The Company uses the asset and liability method for accounting 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.

The Tax Cut and Jobs Act (the "Tax Act") was enacted on December 22, 2017. The Tax Act contains several key provisions including, among other things, reducing the U.S. federal corporate tax rate from 35% to 21%. In addition, federal net operating losses ("NOLs") will be carried forward indefinitely, but will be subject to an 80% utilization against taxable income. The Company follows the guidance in SEC Staff Accounting Bulletin 118 (SAB 118) which provides additional clarification regarding the application of ASC Topic 740 in situations where the Company does not have the necessary information available, prepared, or analyzed in reasonable detail to complete the accounting for certain income tax effects of the Act for the reporting period in which the Act was enacted. SAB 118 provides for a measurement period beginning in the reporting period that includes the Act's enactment date and ending when the Company has obtained, prepared, and analyzed the information needed in order to complete the accounting requirements but in no circumstances should the measurement period extend beyond one year from the enactment date. The Company has enacted the reduction in tax rate effective January 1, 2018, which resulted in a decrease to the deferred tax asset and a decrease to the valuation allowance. The Company is in the process of evaluating the Tax Act changes in section 162(m), Internal Revenue Code of 1986, regarding deductions for excessive employee compensation. The Company continues to gather and analyze information, including the definition of an employee contract for stock grants not vested as of the enactment date of the Act. It is the intention of the Company to complete the necessary analysis within the measurement period however the Company anticipates that any adjustment would be a decrease to deferred tax asset and valuation allowance with no impact to income tax expense.

For the fiscal year ended June 30, 2018, the Company recorded an income tax provision of $72,000 related to the Company's federal alternative minimum tax credit and uncertainties in state income tax. The Company calculates its alternative minimum taxable income (AMTI) using the alternative minimum tax ("AMT") system. The Company's federal income tax liability is the greater of the tax computed using the regular tax system or the tax under the AMT system. Corporations are exempt from AMT for all prior years in which their annual gross receipts for the 3-year period ending before the current tax year did not exceed $7.5 million. For the fiscal year ending June 30, 2017, the Company no longer qualifies for the small company exclusion. The AMT system limits the use of net operating losses used by taxpayers to offset taxable income. The $791,000 AMT credit at June 30, 2018 was created by AMT paid during the fiscal year ended June 30, 2017, which became refundable under the Tax Act resulting in an income tax benefit in 2018.

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. Any interest and penalties related to uncertain tax positions will be reflected in income tax expense. The Company is subject to examination of its income tax returns in the federal and state income tax jurisdictions in which it operates. On December 28, 2015, the United States Internal Revenue Service, or IRS, notified the Company of an income tax audit for the tax period ending June 30, 2014. As of June 30, 2017, the IRS audit was closed and the Company incurred no penalties or payment liabilities for its income tax positions.

For the year ended June 30, 2018, the Company's tax expense includes an increase in the uncertain tax position liability of $1,009,000 related to uncertainty in how states may tax income. The uncertain tax position liability for the years ended June 30, 2018 and 2017, was $1,959,000 and 950,000. No similar liability was recorded for the year ended June 30, 2016.

***Research and development expenses***

Research and development costs 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 financial statements, the Company is required to estimate its accrued expenses. This process involves reviewing quotations and contracts, identifying services that have been performed on its behalf and estimating the level of service performed and the associated cost incurred for the service when the Company has not yet been invoiced or otherwise notified of the actual cost. The majority of the Company's service providers invoice the Company monthly in arrears for services performed or when contractual milestones are met. The Company makes estimates of its accrued expenses as of each balance sheet date in its financial statements based on facts and circumstances known to it at that time. The significant estimates in the Company's accrued research and development expenses are related to expenses incurred with respect to academic research centers, contract research organizations ("CROs"), and other vendors in connection with research and development activities for which it has not yet been invoiced.

There may be instances in which the Company's service providers require advance payments at the inception of a contract or in which payments made to these vendors will exceed the level of services provided, resulting in a prepayment of the research and development expense. 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 $1.5 million at June 30, 2018 and 2017, respectively, and are included within the Prepaid and other current assets line item on the balance sheets.

***Share-based compensation***

The Company accounts for share-based awards issued to employees in accordance with ASC Topic 718, *Compensation—Stock Compensation* ("ASC 718") and generally recognizes share-based compensation expense on a straight-line basis over the periods during which the employees are required to provide service in exchange for the award. In addition, the Company issues stock options and restricted shares of common stock to non-employees in exchange for consulting services and accounts for these 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 non-employees are subject to periodic fair value re-measurement over their vesting terms.

For purposes of calculating stock-based compensation, the Company estimates the fair value of stock options using a Black-Scholes option-pricing model. The determination of the fair value of share-based payment awards utilizing the Black-Scholes model is affected by the Company's stock price and a number of assumptions, including expected volatility, expected life, risk-free interest rate and expected dividends. The expected volatility is primarily based on the historical volatility of peer company data while the expected life of the stock options is based on historical and other economic data trended into the future. The risk-free interest rate assumption is based on observed interest rates appropriate for the expected terms of the Company's stock options. The dividend yield assumption is based on the Company's history and expectation of no dividend payouts. If factors change and the Company employs different assumptions, stock-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 stock-based compensation expense and the actual factors which become known over time, specifically with respect to anticipated forfeitures, the Company may change the input factors used in determining stock-based compensation costs for future grants. These changes, if any, may materially impact the Company's results of operations in the period such changes are made.

***Net earnings (loss) per share***

Basic net earnings (loss) per share is calculated by dividing net earnings (loss) by the weighted average shares outstanding during the period, without consideration for common stock equivalents. Diluted net earnings (loss) per share is calculated by adjusting weighted average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. For purposes of the diluted net earnings (loss) per share calculations, stock options and warrants are considered to be common stock equivalents if they are dilutive. For fiscal years 2018 and 2016, basic and diluted net loss per share are the same due to stock options and warrants being considered anti-dilutive. If stock options and warrants had been dilutive, their impact would have increased common stock equivalents outstanding for fiscal 2018 by 0.2 million shares at June 30, 2018. Stock options and warrants were dilutive for fiscal 2017 and increased common stock equivalents outstanding by 0.3 million shares.

***Comprehensive income (loss)***

Comprehensive income (loss) consists of net income (loss) and changes in equity during a period from transactions and other equity and circumstances generated from non-owner sources. The Company's net income (loss) equals comprehensive loss for all periods presented.

***New Accounting Pronouncements***

In May 2017, the FASB issued Accounting Standards Update ("ASU") No. 2017-09, *Scope of Modification Accounting*, which amends ASC Topic 718, *Compensation – Stock Compensation*. The amendments in this Update provide guidance about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting in Topic 718. The amendments are effective for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years and early adoption is permitted. The Company is currently in the process of evaluating the impact of adoption of this standard on its financial statements.

In March 2016, the FASB issued ASU No. 2016-09, *Improvements to Employee Share-Based Payment Accounting*, which amends ASC Topic 718, *Compensation – Stock Compensation*. The amendments simplify several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities, forfeitures, and classification on the statement of cash flows. The amendments are effective for fiscal years beginning after December 15, 2016, and interim periods within those fiscal years and early adoption is permitted. The Company has adopted this standard for its 2018 fiscal year and it did not have a material effect on its balance sheets, results of operations or cash flows.

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)* in order to increase transparency and comparability among organizations by recognizing lease assets and lease liabilities on the balance sheet for those leases previously classified as operating leases under GAAP. The standard requires, in most instances, a lessee to recognize on its balance sheet a liability to make lease payments (the lease liability) and also a right-of-use asset representing its right to use the underlying asset for the lease term. The amendments are effective for fiscal years beginning after December 15, 2018, including interim periods within those periods, using a modified retrospective approach and early adoption is permitted. The Company is currently in the process of evaluating the impact of adoption of this standard on its financial statements.

In May 2014, the FASB issued ASU No. 2014-09, Revenue from Contracts with Customers (Topic 606), which replaces the existing accounting standards for revenue recognition with a single comprehensive five-step model. The core principle is to recognize revenue upon the transfer of goods or services to customers at an amount that reflects the consideration expected to be received. It also requires enhanced disclosures about revenue, provides guidance for transactions that were not previously addressed comprehensively, and improves guidance for multiple-element arrangements. The guidance applies to any entity that either enters into contracts with customers to transfer goods or services or enters into contracts for the transfer of nonfinancial assets unless those contracts are within the scope of other standards. Since its issuance, the FASB has amended several aspects of the new guidance, including provisions that address revenue recognition associated with the licensing of intellectual property. In July 2015, the FASB delayed the effective date of this guidance by one year. The guidance is effective for public companies for annual periods beginning after December 15, 2017 as well as interim periods within those annual periods using either the full retrospective approach or modified retrospective approach.

The Company will adopt Topic 606 using the modified retrospective approach, effective July 1, 2018. Under such adoption approach, the Company will present comparative periods under legacy guidance, recognize a cumulative catch-up adjustment to the opening balance of retained earnings as of the date of adoption, as applicable, and apply the new guidance to new and existing contracts that were not completed as of the date of adoption.

The Company has substantially completed its adoption plan which included a review of collaboration agreements, applying the five-step model of the new standard and comparing the results to the Company's current accounting as well as a process of revising its revenue recognition accounting policy and expanding revenue disclosures to reflect the requirements of the amended revenue recognition guidance. These efforts include implementing appropriate changes to controls, processes, and systems to support the appropriate recognition and disclosure of revenue on an ongoing basis. While the assessment and adoption plan are substantially complete, the Company continues to work on finalizing the quantification of the impact of Topic 606 and implementing the necessary changes to processes and internal controls. Therefore, the Company is not able to reasonably quantify the cumulative catch-up to be recorded to opening accumulated deficit or the estimated impact to the timing and pattern of revenue recognition moving forward. The Company will finalize its assessment in the first quarter of the fiscal year commencing July 1, 2018

**(3) Investments:**

Cash in excess of our immediate requirements is invested in accordance with the Company's investment policy that primarily seeks to maintain adequate liquidity and preserve capital.

The following table summarizes the Company's investments by category as of June 30, 2018 and 2017:

| In thousands | June 30, 2018 | June 30, 2017 |
|---|---|---|
| Investments - Current: | | |
| Certificates of deposit | $ 2,106 | $ 3,500 |
| Debt securities - held-to-maturity | 71,734 | 92,494 |
| | $ 73,840 | $ 95,994 |
| Investments - Noncurrent: | | |
| Certificates of deposit | $ - | $ 2,111 |
| Debt securities - held-to-maturity | - | 9,638 |
| | $ - | $ 11,749 |

A summary of the Company's debt securities classified as held-to-maturity is as follows:

| | June 30, 2018 | | | |
|---|---|---|---|---|
| In thousands | Amortized Cost | Gross Unrealized Gains | Gross Unrealized Losses | Fair Value |
| Investments - Current: | | | | |
| U.S. government and agency obligations | $ 69,731 | $ - | $ (60) | $ 69,671 |
| Corporate obligations | 2,003 | - | (1) | 2,002 |
| | $ 71,734 | $ - | $ (61) | $ 71,673 |

| | June 30, 2017 | | | |
|---|---|---|---|---|
| In thousands | Amortized Cost | Gross Unrealized Gains | Gross Unrealized Losses | Fair Value |
| Investments - Current: | | | | |
| U.S. government and agency obligations | $ 92,494 | $ - | $ (147) | $ 92,347 |
| Corporate obligations | - | - | - | - |
| | $ 92,494 | $ - | $ (147) | $ 92,347 |
| | | | | |
| Investments - Noncurrent: | | | | |
| U.S. government and agency obligations | $ 7,552 | $ - | $ (52) | $ 7,500 |
| Corporate obligations | 2,086 | - | (12) | 2,074 |
| | $ 9,638 | $ - | $ (64) | $ 9,574 |

The amortized cost and fair value of held-to-maturity debt securities as of June 30, 2018, by contractual maturity, were as follows:

| In thousands | Amortized Cost | Fair Value |
|---|---|---|
| Due in one year or less | $ 71,734 | $ 71,673 |
| | $ 71,734 | $ 71,673 |

The Company believes that the unrealized losses disclosed above were primarily driven by interest rate changes rather than by unfavorable changes in the credit ratings associated with these securities and as a result, the Company continues to expect to collect the principal and interest due on its debt securities that have an amortized cost in excess of fair value. At each reporting period, the Company evaluates securities for impairment when the fair value of the investment is less than its amortized cost. The Company evaluated the underlying credit quality and credit ratings of the issuers, noting neither a significant deterioration since purchase nor other factors leading to an other-than-temporary impairment. Therefore, the Company believes these losses to be temporary. As of June 30, 2018, the Company did not have any intent to sell any of the securities that were in an unrealized loss position at that date.

**(4) Fair Value Measurements:**

Certain assets and liabilities are measured at fair value in the Company's financial statements or have fair values disclosed in the notes to the financial statements. These assets and liabilities are classified into one of three levels of a hierarchy defined by 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 following methods and assumptions were used to estimate the fair value and determine the fair value hierarchy classification of each class of financial instrument included in the table below:

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

***Certificates of Deposit.*** The Company's certificates of deposit are placed through an account registry service. The fair value measurement of the Company's certificates of deposit is considered Level 2 of the fair value hierarchy as the inputs are based on quoted prices for identical assets in markets that are not active. The carrying amounts of the Company's certificates of deposit reported in the balance sheets approximate fair value.

***Debt securities – held-to-maturity.*** The Company's investments in debt securities classified as held-to-maturity generally include U.S. Treasury Securities, government agency obligations and corporate obligations. U.S. Treasury Securities are valued using quoted market prices. Valuation adjustments are not applied. Accordingly, U.S. Treasury Securities are considered Level 1 of the fair value hierarchy. The fair values of U.S. government agency obligations and corporate obligations are generally determined using recently executed transactions, broker quotes, market price quotations where these are available or other observable market inputs for the same or similar securities. As such, the Company classifies its investments in U.S. government agency obligations and corporate obligations within Level 2 of the hierarchy.

The following fair value hierarchy table presents information about each major category of the Company's financial assets and liabilities measured at fair value on a recurring basis:

| In thousands | Quoted prices in active markets (Level 1) | Significant other observable inputs (Level 2) | Significant unobservable inputs (Level 3) | Total Fair Value | Total Carrying Value |
|---|---|---|---|---|---|
| **June 30, 2018** | | | | | |
| Cash and cash equivalents | $ 31,065 | $ - | $ - | $ 31,065 | $ 31,065 |
| Certificates of deposit | - | 2,100 | - | 2,100 | 2,106 |
| Held-to-maturity investments: | | | | | |
| Corporate obligations | - | 2,002 | - | 2,002 | 2,003 |
| U.S. government and agency obligations | 69,671 | - | - | 69,671 | 69,731 |
| Total assets | $ 100,736 | $ 4,102 | $ - | $ 104,838 | $ 104,905 |
| **June 30, 2017** | | | | | |
| Cash and cash equivalents | $ 30,706 | $ - | $ - | $ 30,706 | $ 30,706 |
| Certificates of deposit | - | 5,601 | - | 5,601 | 5,611 |
| Held-to-maturity investments: | | | | | |
| Corporate obligations | - | 2,074 | - | 2,074 | 2,086 |
| U.S. government and agency obligations | 79,476 | 20,372 | - | 99,848 | 100,046 |
| Total assets | $ 110,182 | $ 28,047 | $ - | $ 138,229 | $ 138,449 |

## (5) Property and Equipment, Net:

Property and equipment consists of the following:

| | June 30, | |
|---|---|---|
| In thousands | 2018 | 2017 |
| Laboratory equipment | $ 2,929 | $ 2,645 |
| Leasehold improvements | 3,835 | 1,198 |
| Office equipment | 1,077 | 470 |
| Property and equipment, gross | 7,841 | 4,313 |
| Less: Accumulated depreciation and amortization | (2,587) | (1,652) |
| Property and equipment, net | $ 5,254 | $ 2,661 |

Depreciation expense of $909,000, $657,000 and $319,000 was recorded for each of the fiscal years ended June 30, 2018, 2017 and 2016, respectively.

## (6) Intangible Assets, Net:

Intangible assets subject to amortization consist of the following:

| | June 30, 2018 | | |
|---|---|---|---|
| In thousands | Cost | Accumulated Amortization | Net of Accumulated Amortization |
| Patents | $ 2,193 | $ (1,357) | $ 836 |
| Licenses | 289 | (182) | 107 |
| Other | 54 | (29) | 25 |
| Intangible assets, net | $ 2,536 | $ (1,568) | $ 968 |

| | June 30, 2017 | | |
|---|---|---|---|
| In thousands | Cost | Accumulated Amortization | Net of Accumulated Amortization |
| Patents | $ 2,135 | $ (1,205) | $ 930 |
| Licenses | 1,240 | (981) | 259 |
| Other | 54 | (24) | 30 |
| Intangible assets, net | $ 3,429 | $ (2,210) | $ 1,219 |

Amortization expense related to intangible assets for the years ended June 30, 2018, 2017 and 2016 was $266,000, $255,000 and $248,000, respectively.

Estimated amortization expense (in thousands) for the next five years and thereafter is as follows:

| Year Ending June 30, | Amount |
|---|---|
| 2019 | $ 161 |
| 2020 | 163 |
| 2021 | 165 |
| 2022 | 149 |
| 2023 | 72 |
| Thereafter | 258 |
| | $ 968 |

## (7) Collaboration Agreements

*Biogen*

On July 1, 2015, the Company entered into a Collaboration Agreement with Biogen, pursuant to which the Company and Biogen will collaborate 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. The Collaboration Agreement became effective on August 14, 2015.

Under the Collaboration Agreement, the Company will conduct all development activities through regulatory approval in the United States for the XLRS program (with activities through Phase 1/2 completion being pre-funded under the agreement and any further activities subject to incremental consideration), and all development activities through the completion of the first in human clinical trial for the XLRP program (with activities through filing the IND being pre-funded under the agreement and any further activities subject to incremental consideration). In addition, the Collaboration Agreement provides for discovery programs targeting three indications whereby the Company will conduct discovery, research and development activities for those additional drug candidates through the stage of clinical candidate designation, after which, Biogen may exercise an option to continue to develop, seek regulatory approval for and commercialize the designated clinical candidate. In February 2016, the Company announced Biogen's selection of adrenoleukodystrophy as the non-ophthalmic indication of the discovery programs. Under the terms of the Collaboration Agreement, the Company, in part through its participation in joint committees with Biogen, will participate in overseeing the development and commercialization of these specific programs.

The Company has granted to Biogen with respect to the XLRS and XLRP programs, and upon exercise of the 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 have 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.

Activities under the Collaboration Agreement were evaluated under ASC 605-25, *Revenue Recognition—Multiple Element Arrangements*, as amended by ASU 2009-13, *Revenue Recognition* ("ASC 605-25"), to determine if they represented a multiple element revenue arrangement. The Collaboration Agreement includes the following significant deliverables:

(1) for each of the XLRS and XLRP programs, exclusive, royalty-bearing licenses, with the right to grant sublicenses, to use adeno-associated virus vector technology and other technology controlled by the Company for the purpose of researching, developing, manufacturing and commercializing licensed products developed under the arrangement (the "License Deliverables");

(2) for each of the discovery programs, exercisable options to obtain exclusive licenses to develop, seek regulatory approval for and commercialize any of the designated clinical candidates under such discovery programs (the "Option Deliverables"); and

(3) the performance obligations to conduct research and development activities through (a) regulatory approval in the United States, in the case of the XLRS program; (b) completion of the first in human clinical trial, in the case of the XLRP program; and (c) the stage of clinical candidate designation, in the case of each of the discovery programs (the "R&D Activity Deliverables").

The R&D Activity Deliverables for each program are further segmented by those that are "Pre-Funded Activities" and those that are "Post-Funding Development Activities". Pre-Funded Activities are those R&D activities for which the Company has primary responsibility and the consideration to be received under the agreement was received at the inception of the arrangement. Post-Funding Development Activities are those activities that may occur after the Pre-Funded Activities and for which the Company is entitled to additional compensation under the agreement from Biogen. Biogen has final decision-making authority for all matters related to the conduct of the Post-Funding Development Activities. Because Biogen is not contractually obligated to continue the programs beyond the Pre-Funded Activities, and due to the uncertain outcome of the discovery, research and development activities, the Post-Funding Development Activities are not considered deliverables at the inception of the arrangement and the associated fees and milestones are not included in the allocable arrangement consideration. The Company has determined that the additional fees it could receive under the arrangement for Post-Funding Development Activities are not priced at a significant and incremental discount.

The Company determined that both the License Deliverables and Option Deliverables do not have stand-alone value and do not meet the criteria to be accounted for as separate units of accounting under ASC 605-25. The factors considered by the Company in making this determination included, among other things, the unique and specialized nature of its proprietary technology and intellectual property, and the development stages of each of the XLRS, XLRP and the discovery programs targeting three indications. Accordingly, the License Deliverables under each of the XLRS and XLRP programs and the Option Deliverables under each of the discovery programs have been combined with the initial, Pre-Funded Activities deliverables associated with each related program and as a result, the Company's separate units of accounting under its collaboration with Biogen, comprise the XLRS program, the XLRP program, and each of the discovery programs.

Under the Collaboration Agreement, the Company received a non-refundable upfront payment of $94.0 million in August 2015 which it recorded as deferred revenue. This upfront payment of $94.0 million was allocated among the separate units of accounting discussed above using the relative selling price method. In addition to the Collaboration Agreement, on July 1, 2015, the Company also entered into an equity agreement with Biogen. Under the terms of this equity agreement, Biogen purchased 1,453,957 shares of the Company's common stock at a price of $20.63 per share, for an aggregate cash purchase price of $30.0 million which the Company also received in August 2015. The shares issued to Biogen represented approximately 8.1% of the Company's outstanding common stock on a post-issuance basis, calculated on the number of shares that were outstanding at June 30, 2015, and constitute restricted securities that may not be resold by Biogen other than in a transaction registered under, or pursuant to an exemption from the registration requirements of, the Securities Act of 1933, as amended.

Accounting standards for multiple element arrangements contain a presumption that separate contracts negotiated or entered into at or near to the same time with the same entity were likely negotiated as a package and should be evaluated as a single agreement. The Company determined that the price of $20.63 paid by Biogen included a premium of $7.45 per share over the fair value of the company's stock price, calculated based upon the stock price on the date of close of the agreement and adjusted for lack of marketability due to restrictions. Accordingly, the total premium of $10.8 million was also recorded as deferred revenue and, together with the $94.0 million, allocated to the separate units of accounting identified above using the relative selling price method as discussed in Note 2 to these financial statements. The Company will record revenue based on the revenue recognition criteria applicable to each separate unit of accounting. For amounts received up-front and initially deferred,

the Company will recognize the deferred revenue on a straight-line basis over the estimated service periods in which it is required to perform the research and development activities associated with each unit of accounting. At the inception of the Collaboration Agreement, the Company initially estimate the service periods to range between 2 and 3 years. However, due to certain delays which have extended our estimated period of performance, the estimated service periods are currently anticipated to be between 2 and 5 from the inception of the Collaboration Agreement.

During the fiscal years ended June 30, 2018, 2017 and 2016, the Company recognized revenue of approximately $24.1 million, $39.3 million and $46.8 million, respectively from its collaboration with Biogen. Below is a summary of the components of the collaboration revenue:

| | Year ended June 30, | |
|---|---|---|
| | 2018 | 2017 |
| | (dollars in thousands) | |
| Amortization of non-refundable upfront fees | $ 18,529 | $ 38,230 |
| Milestone revenue | 2,500 | - |
| Development services | 3,028 | 1,052 |
| Total collaboration revenue | $ 24,057 | $ 39,282 |

During the fiscal years ended June 30, 2018 and 2016, the Company recorded and received $2.5 and $5.0 million, respectively of milestone revenue after having achieved a patient enrollment-based milestone under the Collaboration Agreement. No milestone revenue was recorded in the fiscal year ended June 30, 2017. Developmental services revenue is comprised of reimbursable costs for post-funding development activities that were conducted by the Company.

As a result of the upfront payment of $94.0 million made by Biogen, as discussed above, the Company became liable to various research partner institutions for sub-license and other payments under existing agreements with such institutions. These agreements obligate the Company to pay to each research partner institution, amounts that range from 5% to 10% of certain proceeds received from collaboration and other arrangements, including any milestone payments received under such arrangements. Accordingly, the Company recorded total collaboration costs of approximately $10.6 million associated with the $94 million upfront payment, including $636,000 of expense that was settled during fiscal year 2016 by the issuance of 40,000 shares of the Company's common stock to a research partner institution, pursuant to the terms of the existing agreement with that institution. The remainder of these sub-license and milestone fees were fully paid in cash during the fiscal year ended June 30, 2016. As a result of the achievement of the $2.5 and $5.0 million milestone payments, discussed above, the Company recorded sub-license expense of $1,375,000 in fiscal year 2016 and $562,500 in fiscal year 2018.

The Company is also eligible to receive payments of up to $467.5 million based on the successful achievement of future milestones under its XLRS and XLRP programs. For XLRS, the Company is eligible to receive up to: (i) $40 million in milestone payments based upon the successful achievement of clinical milestones (relating to dosing in specified trials), (ii) $155 million in milestone payments based upon the achievement of regulatory approvals and first commercial sale in specified territories and (iii) $65 million in milestone payments based upon the achievement of worldwide sales targets. For XLRP, the Company is eligible to receive up to: (i) $42.5 million in milestone payments based upon successful achievement of clinical milestones (relating to dosing in specified trials), (ii) $102.5 million in milestone payments based upon the achievement of regulatory approvals and first commercial sale in specified territories and (iii) $62.5 million in milestone payments based upon the achievement of worldwide sales targets. In addition, the Company is eligible to receive payments of up to $592.5 million based on the exercise of the option for and the successful achievement of future milestones under its discovery programs. Each discovery program is categorized as Category A, Category B or Category C depending on the nature of the indication it seeks to address. For Category A, the Company is eligible to receive payments of up to: (i) $20 million based upon the successful achievement of clinical milestones (relating to dosing in specified trials) and (ii) $70 million in milestone payments based upon the achievement of regulatory approvals and first commercial sale in specified territories. For Category B, the Company is eligible to receive payments of up to: (i) $27.5 million based upon the successful achievement of clinical milestones (relating to dosing in specified trials) and (ii) $105 million in milestone payments based upon the achievement of regulatory approvals and first commercial sale in specified territories. For Category C, the Company is eligible to receive payments of up to: (i) $40 million based upon the successful achievement of clinical milestones (relating to dosing in specified trials) and (ii) $140 million in milestone payments based upon the achievement of regulatory approvals and first commercial sale in specified territories. Under certain limited circumstances, if there are discovery products from more than one discovery program in any of Category A, Category B or Category C, then the milestone payments under the applicable category shall be payable for the applicable discovery product from each such discovery program to achieve the specified milestones.

Biogen will also pay revenue-based royalties for each licensed product at tiered rates ranging from high single digit to mid-teen percentages of annual net sales of the XLRS or XLRP products and at rates ranging from mid-single digit to low-teen percentages of annual net sales for the discovery products. The Company has elected to apply the guidance in ASC 605-28 to the milestones. These milestones, if achieved, are substantive as they relate solely to past performance, are commensurate with estimated enhancement of value associated with the achievement of each milestone as a result of the Company's performance and are reasonable when compared to other consideration amounts payable under the Collaboration Agreement; however, there can be no assurance that the Company will achieve the milestones or that the Company will receive the related revenue. Due to the uncertainty surrounding the achievement of the future milestones, such payments were not considered fixed or determinable at the inception of the Collaboration Agreement and accordingly, will not be recognized as revenue unless and until they become earned. The Company is not able to reasonably predict if and when the remaining milestones will be achieved.

*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.

Under the agreement, AGTC made an initial $2.0 million payment to Bionic Sight for an equity interest in that company. This initial investment represents an approximate 5% equity interest in Bionic Sight. In addition to the initial investment, AGTC will contribute to ongoing research and development support costs through additional payments or other in-kind contributions (AGTC Ongoing R&D Support). The AGTC Ongoing R&D Support payments and in-kind contributions will be made over time, up to the date that Bionic Sight has received both IND clearance from the 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".)

If the IND Trigger is attained, AGTC will receive additional equity, 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 will be obligated to purchase additional equity in Bionic Sight for $4.0 million, at a pre-determined valuation. Due to the uncertainty of achieving the IND Trigger, the Company is expensing the AGTC Ongoing R&D Support payments and in-kind contributions made under the collaboration agreement. Such amounts are included as a component of research and development expenses in the Company's financial statements.

The Company recorded its initial $2.0 million investment in Bionic Sight using the equity method of accounting for investments, which is recorded as its own line item on the Company's balance sheet. During fiscal 2018, the Company recorded a reduction of its investment in Bionic Sight of $19,913 and an investment loss on the statement of operations to reflect its equity interest in the net loss of this affiliate. As of June 30, 2018, the amount of the Company's underlying equity in net assets of Bionic Sight is not representative of the amount at which the investment is carried due to retained losses experienced by Bionic Sight prior to the Company's investment.

The ongoing research and development costs and contributions will be recorded as a periodic cost until such time when or if the IND Trigger is achieved.

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.

**(8) Share-based Compensation Plans:**

The Company uses stock options and awards of restricted stock to provide long-term incentives for 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 all of the 128,571 shares previously authorized under this plan

remain available for issuance. As of June 30, 2018, the total number of shares available for issuance under the 2013 Equity and Incentive Plan was 1,105,262.

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 share award; (ii) the date on which the 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 as of the date of grant; (iv) the vesting term; and (v) the duration of the 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.

A summary of the stock option activity is as follows:

| | Years ended June 30, | | | | | |
|---|---|---|---|---|---|---|
| | 2018 | | 2017 | | 2016 | |
| (In thousands, except per share amounts) | Shares | Weighted Average Exercise Price | Shares | Weighted Average Exercise Price | Shares | Weighted Average Exercise Price |
| **Outstanding**, beginning of year | 2,716 | $ 12.95 | 2,037 | $ 13.71 | 1,484 | $ 11.83 |
| Granted | 914 | 4.72 | 842 | 10.92 | 675 | 17.08 |
| Exercised | (18) | 0.35 | (31) | 0.91 | (59) | 4.77 |
| Forfeited | (415) | 9.86 | (101) | 13.97 | (57) | 13.90 |
| Expired | (90) | 15.92 | (31) | 16.17 | (6) | 15.50 |
| **Outstanding**, end of year | 3,107 | $ 10.93 | 2,716 | $ 12.95 | 2,037 | $ 13.71 |
| **Exercisable**, end of year | 1,900 | | 1,431 | | 872 | |
| Weighted average fair value of options granted during the year | $ 3.37 | | $ 7.54 | | $ 11.83 | |

The following table summarizes information about stock options outstanding:

| | June 30, | | | |
|---|---|---|---|---|
| (Number of options in thousands; remaining lives in years) | 2018 | | 2017 | |
| Exercise Price | Number of Options | Weighted Average Contractual Life Remaining | Number of Options | Weighted Average Contractual Life Remaining |
| $0.35 to $4.90 | 1,118 | 5.18 | 475 | 5.57 |
| $4.95 to $10.55 | 404 | 8.06 | 467 | 9.48 |
| $12.00 to $15.90 | 658 | 5.56 | 744 | 8.11 |
| $16.00 to $19.87 | 764 | 6.09 | 864 | 7.83 |
| $20.00 to $24.62 | 163 | 6.38 | 166 | 7.57 |
| | 3,107 | | 2,716 | |

The following table summarizes information about stock options exercisable:

| | June 30, | |
|---|---|---|
| | 2018 | 2017 |
| Exercise Price | Number of Options (in thousands) | |
| $0.35 to $4.90 | 532 | 460 |
| $4.95 to $10.55 | 133 | 24 |
| $12.00 to $15.90 | 477 | 355 |
| $16.00 to $19.87 | 615 | 482 |
| $20.00 to $24.62 | 143 | 110 |
| | 1,900 | 1,431 |

As of June 30, 2018, the aggregate intrinsic value of all outstanding stock options was $0.6 million and for exercisable stock options was $0.6 million. The intrinsic value per option at June 30, 2018 is calculated as the difference between the exercise price of the underlying option and the closing price of the Company's common stock of $3.70 on that date, and applies only to those awards having an exercise price currently below this closing price. The total fair value of options that vested during the fiscal years ended June 30, 2018, 2017, and 2016 was $5.1 million, $6.0 million, and $4.5 million, respectively.

Unrecognized compensation expense related to non-vested employee stock options amounted to $6.6 million as of June 30, 2018. Such compensation expense is expected to be recognized over a weighted-average period of 2.14 years.

In accounting for stock options to non-employees, the fair value of services related to the options granted are generally recorded as an expense as these services are provided to the Company over the relating service periods. The Company re-measures any non-vested, non-employee options to fair value at the end of each reporting period using the Black-Scholes pricing model.

Share-based compensation expense related to stock options awarded to employees, non-employee directors and consultants amounted to $5.0. million, $5.6 million and $4.9 million for the fiscal years ended June 30, 2018, 2017 and 2016, respectively.

Share-based compensation expense related to restricted shares of common stock awarded to employees and consultants amounted to $145,600, $5,000 and $167,000 for the fiscal years ended June 30, 2018, 2017 and 2016, respectively.

Total share-based expense associated with stock options and restricted shares of common stock was allocated as follows:

| | Years ended June 30, | | |
|---|---|---|---|
| In thousands | 2018 | 2017 | 2016 |
| General and administrative | $ 2,821 | $ 3,136 | $ 2,860 |
| Research and development | 2,372 | 2,470 | 2,147 |
| | $ 5,193 | $ 5,606 | $ 5,007 |

The fair value of each option granted is estimated on the grant date using the Black-Scholes stock option pricing model. The following assumptions were made in estimating fair value:

| | Years Ended June 30, | | |
|---|---|---|---|
| Assumption | 2018 | 2017 | 2016 |
| Dividend yield | 0.00% | 0.00% | 0.00% |
| Expected term | 6.00 to 6.50 years | 6.00 to 6.25 years | 6.00 to 6.25 years |
| Risk-free interest rate | 1.83% to 2.87% | 1.08% to 2.11% | 1.41% to 1.86% |
| Expected volatility | 83.53% | 79.18% | 78.85% |

The dividend yield is based upon the assumption that the Company will not declare a dividend over the life of the options. Since adopting ASC 718, the Company has been unable to use historical employee exercise and option expiration data to estimate the expected term assumption for the Black-Scholes grant-date valuation. The Company therefore has utilized the "simplified" method, as prescribed by the SEC's Staff Accounting Bulletin No. 107, *Share-Based Payment*, to estimate on a formula basis the expected term of our stock options 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. As a relatively new public company, the Company does not have sufficient history to estimate the volatility of its common stock price or the expected life of the options. The Company calculates expected volatility based on reported data for similar publicly traded companies for which historical information is available and will continue to do so until the historical volatility of its common stock is sufficient to measure expected volatility for future option grants. The group of similar publicly traded companies was determined based upon companies considered to be direct competition or having been presented by independent parties as a "comparable" company based upon market sector. In determining this group, the Company has excluded "large-cap" entities. 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 statements of operations for the fiscal years ended June 30, 2017, 2016 and 2015 does not reflect tax related effects on stock-based compensation given the Company's historical and anticipated operating losses and offsetting changes in its valuation allowance that fully reserves against potential deferred tax assets.

## (9) Commitments and Contingencies:

### Operating Leases

*Alachua, Florida*

The Company's corporate headquarters are located in Alachua, Florida. In January 2016, the Company moved into a new combined-use facility consisting of approximately 21,000 square feet of laboratory and office space. The initial lease term for this facility is 10 years and the Company has options to extend the term of the lease for three additional five-year periods. The Company's prior leased facilities encompassed approximately 7,000 square feet of office and laboratory space. The operating leases associated with the prior facilities expired in December 2015.

*Cambridge, Massachusetts*

In August 2015, the Company entered into a two-year lease to occupy approximately 3,000 square feet of office and laboratory space in Cambridge, Massachusetts. In July 2017, the Company entered into a new lease to increase our office and laboratory space in Cambridge by approximately 5,000 square feet to a total of approximately 8,000 square feet and extend the term of the lease for an additional seven years, with an option to further extend the lease for one additional three-year term. This additional facility primarily focuses on business development, pharmacology, clinical operations and basic research and development.

For the fiscal years ended June 30, 2018, 2017, and 2016, rent expense under these operating leases amounted to $909,000, $845,000 and $587,000, respectively. Future annual minimum lease payments (in thousands) under these non-cancelable operating leases are as follows:

| Year Ending June 30, | Amount |
|---|---|
| 2019 | $ 1,331 |
| 2020 | 1,353 |
| 2021 | 1,376 |
| 2022 | 1,400 |
| 2023 | 1,425 |
| Thereafter | 4,088 |
| | $ 10,973 |

### 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 its business, principally relating to licensed technology. The Company had seven license agreements with six different entities, including four with the University of Florida Research Foundation. The Company is responsible for all costs related to preparation, filing, issuance, prosecution and maintenance of the underlying patents covered in the license agreements. The Company is required to pay minimum annual royalty and license maintenance for all licenses until such time when the license is terminated by either expiration of underlying patents or voluntary termination by either party per the agreement.

These 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 as 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 they may not be achieved. The Company may terminate its license agreements with zero to ninety days written notice depending upon 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 any copyright or other intellectual property infringement claim by any third party with respect to the Company's products. The term of these indemnification agreements is 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. From time to time, the Company may be involved in 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.

## (10) Income Taxes:

For the fiscal years ended June 30, 2018 and 2017, the Company recorded the following current and deferred income tax expense or (benefit). For the fiscal year ended June 30, 2018, the federal and state income tax provision (benefit) summarized as follows:

| | June 30, | |
|---|---|---|
| In thousands | 2018 | 2017 |
| **Current provision:** | | |
| Federal | $ (790) | $ 13,706 |
| State | 862 | 1,774 |
| | 72 | 15,480 |
| **Deferred tax liabilities:** | | |
| Federal | $ — | $ (12,949) |
| State | — | (131) |
| | — | (13,080) |
| **Provision for income taxes** | $ 72 | $ 2,400 |

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

| | June 30, | |
|---|---|---|
| In thousands | 2018 | 2017 |
| **Deferred tax assets:** | | |
| Net operating loss carryforwards | $ 13,910 | $ 9,124 |
| Tax credit carryforwards | 23,567 | 19,195 |
| Accruals and other | 4,625 | 11,987 |
| Depreciation and amortization | 112 | - |
| Gross deferred tax assets | 42,214 | 40,306 |
| Deferred tax asset valuation allowance | (42,214) | (40,303) |
| Total deferred tax assets, net of valuation allowance | - | 3 |
| **Deferred tax liabilities:** | | |
| Depreciation and amortization | - | (3) |
| Total deferred tax liabilities | - | (3) |
| Net deferred tax asset (liability) | $ — | $ — |

As of June 30, 2018, the Company had federal and state net operating losses of approximately $24.8 million and $50.2 million, respectively, that may be applied against future taxable income and expire in various years ranging from 2022 to 2038 and federal net operating losses of $28.3 million that do not expire under the Tax Act. As of June 30, 2018, the Company also had research and development tax credits of approximately $23.6 million that may provide future tax benefits and expire from 2027 to 2047.

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on its history of operating losses, the Company has concluded that as of June 30, 2018, it is more likely than not that the benefit of its deferred tax assets will 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 carry forwards as of June 30, 2018, computed based on statutory federal and state rates, are completely offset by valuation allowances established because realization of the deferred tax benefits are not considered more likely than not as of that date. The valuation allowance increased by approximately $1.9 million during the fiscal year ended June 30, 2018, due primarily to the net increase in federal tax credits and net operating losses.

On December 22, 2017, the President of the United States signed into law the Tax Cuts and Jobs Act (H.R. *1*) (the "Tax Act"). The Act includes a number of changes in existing tax law impacting businesses including, among other things, a permanent reduction in the corporate income tax rate from *35%* to *21%,* effective January 1, 2018. The Company's deferred tax assets and valuation reserve decreased by $7.6 million from the impact of the corporate tax rate change from the Tax Reform Act.

The differences between the effective income tax rate reflected in the benefit (provision) for income taxes and the amounts, which would be determined by applying a blended statutory federal income tax rate of 28% at June 30, 2018 and 34% at June 30, 2017 and 2016 is summarized as follows:

| | Years ended June 30, | | |
|---|---|---|---|
| | 2018 | 2017 | 2016 |
| Federal income tax benefit at statutory rate | 28% | 34% | (34)% |
| State income tax, net of federal benefit | 3% | 4% | (4)% |
| Permanent differences- incentive stock compensation | (2)% | 40% | 35% |
| Permanent differences- research expenses | (7)% | 80% | 115% |
| Research and development tax credits | 24% | (240)% | (327)% |
| Tax Act- refundable AMT credit | 4% | 0% | 0% |
| Other | (2)% | 392% | 174% |
| Change in unrecognized tax benefit | (3)% | 34% | 0% |
| Remeasurement of net deferred tax assets | (36)% | 0% | 0% |
| Change in valuation allowance, including remeasurement | (9)% | (259)% | 41% |
| Effective income tax rate | 0% | 85% | 0% |

Under the provisions of the Internal Revenue Code, the Company's net operating loss and tax credit carry forwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. Net operating loss and tax credit carry forwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50 percent, as defined under Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is 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 common stock which have resulted in a change in control as defined by Sections 382 and 383 of the Internal Revenue Code. Subsequent ownership changes may further affect the limitation in future years. A full valuation allowance has been provided against the Company's net operating loss carryforwards and, if an adjustment were to be required, this adjustment would be offset by an adjustment to the deferred tax asset established for the net operating loss carryforwards and the valuation allowance.

For fiscal years through June 30, 2018, the Company generated research credits but has not conducted a study to document the qualified activities. This study may result in an adjustment to the Company's research and development tax credit carry forwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position at June 30, 2018 or 2017. A full valuation allowance has been provided against the Company's research and development tax credits and, if an adjustment were to be required, this adjustment would be offset by an adjustment to the deferred tax asset established for the tax credit carry forwards and the valuation allowance.

The Company files income tax returns in the United States and in multiple states. The federal and state returns are generally subject to tax examinations for the tax years ended June 30, 2014 through June 30, 2018. To the extent the Company has tax attribute carry forwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service, or state authorities, to the extent such attributes are utilized in a future period. On December 28, 2015, the United States Internal Revenue Service, or IRS, notified the Company of an income tax audit for the tax period ending June 30, 2014. As of June 30, 2017, the IRS audit was closed and the Company incurred no penalties or payment liabilities for its income tax positions.

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 year ended June 30, 2018, the Company increased the uncertain tax position reserve by $1,009,000 which includes interest and penalties. A reserve of $950,000 was recorded for the year ended June 30, 2017. No

similar reserve was recorded for the years ended June 30, 2016. The entire amount of the reserve would reduce the annual effective tax rate if recognized. The Company does not anticipate that the amount of unrecognized tax benefits as of June 30, 2018 will significantly change within the next twelve months. The Company's practice is to recognize interest and/or penalties related to uncertain income tax positions in income tax expense. The Company had $348,000 of interest and/or penalties accrued on the Company's balance sheets at June 30, 2018. The Company had no interest and/or penalties accrued on the Company's balance sheet at June 30, 2017. The Company recognized $348,000 of interest and/or penalties in the statement of operations for the year ended June 30, 2018 related to uncertain tax positions. The Company did not recognize any interest and/or penalties in the statement of operations for the years ended June 30, 2017 and 2016 related to uncertain tax positions. The liability for uncertain tax positions as of June 30, 2018 is included in other long-term liabilities on the balance sheet. A reconciliation of the unrecognized tax benefits is summarized below (in thousands):

| | Years Ended June 30, | | |
|---|---|---|---|
| | 2018 | 2017 | 2016 |
| Balance at beginning of period | $ 950 | $ - | $ - |
| Additions related to current period tax positions | $ — | 950 | $ - |
| Additions related to prior period tax positions | 661 | — | - |
| Balance at end of period | $ 1,611 | $ 950 | $ - |

**(11) Accrued Expenses:**

Accrued expenses as of June 30, 2018 and 2017 consisted of the following:

| | June 30, | |
|---|---|---|
| In thousands | 2018 | 2017 |
| Research and development-related | $ 4,164 | $ 2,868 |
| Compensation-related | 2,186 | 1,780 |
| Accrued tax liabilities | - | 1,514 |
| General and administrative- related | 805 | - |
| | $ 7,155 | $ 6,162 |

**(12) Defined Contribution Plan:**

The Company sponsors an employee 401(k) salary deferral plan ("401(k) Plan") that covers substantially all of its employees and is administered through its 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 the eligible salary. The Company's matching contributions to the 401(k) Plan amounted to $302,000, $235,000 and $162,000 for each of the fiscal years ended June 30, 2018, 2017 and 2016, respectively.

## (13) Common Stock, Preferred Stock and Stockholders' Equity:

### Common Stock

In connection with its entry into the Collaboration Agreement with Biogen described in Note 7 of these financial statements, the Company also entered into an equity agreement with Biogen on July 1, 2015. Under the terms of this equity agreement, Biogen purchased 1,453,957 shares of the Company's common stock, at a price of $20.63 per share, for an aggregate cash purchase price of $30.0 million. These cash proceeds were received from Biogen in August 2015. The shares issued to Biogen constitute restricted securities that may not be resold by Biogen other than in a transaction registered under the Securities Act of 1933, as amended, or pursuant to an exemption from such registration requirement.

As of June 30, 2018, there were 150,000,000 shares of $0.001 par value common stock and 5,000,000 shares of preferred stock that were authorized to be issued. As of that date, a total of 18,137,412 and 18,126,425 shares of common stock were issued and outstanding, respectively, while none of the preferred shares were issued and outstanding.

The following shares of common stock were reserved for future issuance as of June 30, 2018:

| In thousands | June 30, 2018 |
|---|---|
| Stock options issued and outstanding | 3,106,710 |
| Authorized for future grant under the 2013 Employee Stock Purchase Plan | 128,571 |
| Authorized for future grant under the 2013 Equity and Incentive Plan | 1,105,262 |
| | 4,340,543 |

## (14) Quarterly Financial Information (Unaudited):

Summarized quarterly information for the two fiscal years ended June 30, 2018 and 2017, respectively, is as follows:

| | Year 2018 by Quarter: | | | |
|---|---|---|---|---|
| In thousands, except per share data | First | Second | Third | Fourth |
| Revenue | $ 10,315 | $ 4,852 | $ 3,603 | $ 5,416 |
| Loss from operations | $ (1,667) | $ (6,242) | $ (7,696) | $ (6,779) |
| Net loss | $ (1,397) | $ (5,190) | $ (8,101) | $ (6,612) |
| Net loss per common share, basic | $ (0.08) | $ (0.29) | $ (0.45) | $ (0.36) |
| Net loss per common share, diluted | $ (0.08) | $ (0.29) | $ (0.45) | $ (0.36) |

| | Year 2017 by Quarter: | | | |
|---|---|---|---|---|
| In thousands, except per share data | First | Second | Third | Fourth |
| Revenue | $ 11,806 | $ 10,933 | $ 8,388 | $ 8,345 |
| Income (loss) from operations | $ 3,625 | $ 2,379 | $ (600) | $ (2,597) |
| Net income (loss) | $ 3,025 | $ 1,779 | $ (1,200) | $ (3,197) |
| Net earnings (loss) per common share, basic | $ 0.17 | $ 0.10 | $ (0.07) | $ (0.18) |
| Net earnings (loss) per common share, diluted | $ 0.16 | $ 0.10 | $ (0.07) | $ (0.18) |

## (15) Subsequent events

In August 2018, the Company achieved the second milestone under the XLRP program, earning a $10.0 million milestone payment from Biogen related to dosing of the fourth patient in the XLRP Phase 1/2 clinical trial. The company owes sublicensing fees of approximately 23% or $2.3 million, to certain collaborators associated with this milestone payment.

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

On December 20, 2017, the Company notified RSM US LLP ("RSM") that RSM was dismissed as the Company's independent registered public accounting firm. RSM's dismissal became effective on December 19, 2017. The decision to change accounting firms was approved by the Audit Committee and by the Board of Directors of the Company (the "Audit Committee").

RSM's reports on the Company's consolidated financial statements as of and for the fiscal years ended June 30, 2017 and June 30, 2016 did not contain an adverse opinion or a disclaimer of opinion and were not qualified or modified as to uncertainty, audit scope or accounting principles. During the Company's two most recent fiscal years ended June 30, 2017 and 2016 and during the period from July 1, 2017 through December 20, 2017, the Company did not have any disagreement with RSM on any matter of accounting principles or practices, financial statement disclosure or auditing scope or procedures, which disagreements, if not resolved to RSM's satisfaction, would have caused RSM to make reference to the subject matter of disagreement in their reports on the Company's consolidated financial statements. In addition, during such periods, there were no "reportable events" as that term is defined in Item 304(a)(1)(v) of Regulation S-K, except as set forth in this paragraph. As disclosed in the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, the Company's Annual Report on Form 10-K for the fiscal year ended June 30, 2017, as amended, and the Company's Annual Report on Form 10-K for the fiscal year ended June 30, 2016, as amended, the Company identified material weaknesses in the Company's internal control over financial reporting related to the design and operation of our closing and financial reporting processes. Specifically, the material weaknesses were due to the fact that the Company did not have the appropriate resources with the appropriate level of experience and technical expertise to oversee the Company's closing and financial reporting processes.

The Company provided RSM with a copy of the disclosures included in its Current Report on Form 8-K filed on December 21, 2017 (the "Current Report"). The Company requested that RSM furnish a letter addressed to the SEC stating whether or not it agrees with the statements made in the Current Report. A copy of RSM's letter dated December 21, 2017 was attached as Exhibit 16.1 to the Current Report.

On December 19, 2017, the Company engaged Ernst & Young LLP ("EY") as the Company's new independent registered public accounting firm for the fiscal year ending June 30, 2018. The engagement of EY was approved by the Audit Committee.

During the years ended June 30, 2017 and 2016 and the subsequent interim period through December 19, 2017, neither the Company nor anyone acting on its behalf consulted with EY regarding either: (i) the application of accounting principles to a specified transaction, either completed or proposed, or the type of audit opinion that might be rendered on the Company's financial statements, and neither a written report nor oral advice was provided to the Company that EY concluded was an important factor considered by the Company in reaching a decision as to the accounting, auditing or financial reporting issue or (ii) any matter that was either the subject of a disagreement (as that term is defined in Item 304(a)(1)(iv) of Regulation S-K and the related instructions to Item 304 of Regulation S-K) or a reportable event (as that term is defined in Item 304(a)(1)(v) of Regulation S-K).

## 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 us, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. In designing and evaluating our disclosure controls and procedures, we recognize that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, as ours are designed to do, and we necessarily were required to apply our judgment in evaluating whether the benefits of the controls and procedures that we adopt outweigh their costs.

As required by Rule 13a-15(b) under the Exchange Act, an evaluation of the effectiveness of our disclosure controls and procedures (as defined in Rule 13a-15(e) under the Exchange Act) as of June 30, 2017 was conducted under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer. As disclosed in the Company's Annual Report on Form 10-K for the fiscal year ended June 30, 2017, the Company had ineffective internal control over financial reporting relating to the design and operation of our closing and financial reporting processes that constituted material weaknesses. This weakness could have resulted in material misstatements to our annual and interim consolidated financial statements that would not be prevented or detected.

As of the end of the period covered by this report, our management evaluated, with the participation of our Chief Executive Officer and Chief Financial Officer, the remediation efforts related to the material weaknesses previously noted along with the effectiveness of our disclosure controls and procedures. Based on this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that, as of the end of June 30, 2018, our disclosure controls and procedures were effective to provide reasonable assurance that the objectives of disclosure controls and procedures are met.

**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 generally accepted accounting principles. Internal control over financial reporting includes maintaining records that in reasonable detail accurately and fairly reflect our transactions; providing reasonable assurance that transactions are recorded as necessary for preparation of our financial statements; providing reasonable assurance that receipts and expenditures of our assets are made in accordance with management's authorization; and providing reasonable assurance that unauthorized acquisition, use or disposition of our assets that could have a material effect on the financial statements would be prevented or detected on a timely basis.

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. Therefore, internal control over financial reporting determined to be effective provides only reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles.

Management assessed the effectiveness of the Company's internal control over financial reporting as of June 30, 2018. 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, 2018. Accordingly, our management has concluded that the financial statements included in this Annual Report on Form 10-K present fairly, in all material respects, our financial position, results of operations and cash flows for the periods presented in conformity with accounting principles generally accepted in the United States.

We are not required to comply with the auditor attestation requirement of Section 404 of the Sarbanes-Oxley Act while we qualify as an "emerging growth company" as defined in the JOBS Act. Subject to certain limitations, we expect to remain an emerging growth company under the JOBS Act for up to five years from April 1, 2014, the date of our initial public offering.

**Remediation of Previously Identified Material Weaknesses**

Management previously identified and disclosed a material weakness in our internal control over financial reporting at June 30, 2017 which related to the design and operation of our closing and financial reporting processes. Specifically, management concluded that this material weakness in our internal control over financial reporting was due to the fact that we did not have the appropriate resources with the appropriate level of experience and technical expertise to oversee our closing and financial reporting processes.

As of June 30, 2018, management sufficiently completed its remediation of these material weaknesses by taking the following actions:

- We hired additional employees during the second half of fiscal year 2018 to provide further support to our finance and accounting team. As of June 30, 2018, the finance and accounting team comprises a total of nine employees, including our Chief Financial Officer and our Controller;
- We restructured our finance team to better align the functional areas to the overall strategy of the company, while at the same time providing more focus for the accounting team in maintaining proper control over the financial reporting process commensurate to support standalone external financial reporting under public company or SEC requirements;
- We provided functional and system training to employees and prepared detailed documentation to clearly define key tasks and actions, as well as the positions responsible for those tasks and actions. During fiscal year 2018, we also engaged the services of a consulting firm to assist in documenting and formalizing our accounting policies and internal control processes and to help strengthen supervisory reviews by our management; and

- We continued to design and implement monthly manual controls to manage our financial reporting close processes and to help ensure an adequate level of segregation of duties within our finance and accounting function. We also designed additional controls around identification, documentation and application of technical accounting guidance with particular emphasis on events outside the ordinary course of business. These controls include the implementation of additional supervision and review activities by qualified personnel, the preparation of formal accounting memoranda to support our conclusions on technical accounting matters, and the development and use of checklists and research tools to assist in compliance with GAAP with regard to complex accounting issues.
- Developed and implemented policies and procedures related to security access, including security access reviews of our key financial systems' users to ensure the appropriateness of their roles and security access levels.
- Performed testing related to the functioning of these controls, and continue to monitor these controls and make enhancements as needed.

We have completed the documentation and review of the corrective actions described above and our management has concluded that the design and operation of our closing and financial reporting processes is effective and therefore that this previously identified material weakness has been fully remediated as of June 30, 2018.

**Changes in Internal Control over Financial Reporting**

Except for those remedial actions described above, there was no change in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the period covered by this report that has materially affected, or is 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 information regarding directors, executive officers and corporate governance included in our definitive proxy statement to be filed with the Securities and Exchange Commission pursuant to Regulation 14A no later than 120 days after the end of our fiscal year in connection with our fiscal 2018 Annual Meeting of Stockholders (the "Proxy Statement") is incorporated herein by reference.

We are required under Item 405 of Regulation S-K to provide information concerning delinquent filers of reports under Section 16 of the Securities and Exchange Act of 1934, as amended. This information will be set forth under the caption "Section 16(a) Beneficial Ownership Reporting Compliance" in our Proxy Statement, and is incorporated herein by reference.

## ITEM 11. EXECUTIVE COMPENSATION

The information regarding executive compensation included in the Proxy Statement is incorporated herein by reference.

## ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

The information regarding security ownership of certain beneficial owners and management and related stockholder matters included in the Proxy Statement is incorporated herein by reference.

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 following table provides information regarding securities authorized for issuance as of June 30, 2018 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 | 3,106,710 | $ 10.93 | 1,233,833 | (1) |
| Equity compensation plans not approved by security holders | — | $ — | — | |
| Total | 3,106,710 | $ 10.93 | 1,233,833 | |

(1) Includes 1,105,262 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 added 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 DIRECTORS INDEPENDENCE

The information regarding certain relationships and related transactions, and director independence included in the Proxy Statement is incorporated herein by reference.

## ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

The information regarding principal accounting fees and services included in the Proxy Statement is incorporated herein by reference.

## 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 76 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 76 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)) |
| 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 ending June 30, 2015 (File No. 001-36370)) |
| 10.2* | Employment Agreement dated as of May 27, 2015 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 ending June 30, 2015 (File No. 001-36370)) |
| 10.3* | Employment Agreement dated as of January 29, 2015 between Applied Genetic Technologies Corporation and Stephen W. Potter (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q for the quarter ended March 31, 2015 (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 |
| 10.6 | Common Stock Purchase Agreement dated as of July 1, 2015 by and between Biogen MA Inc., and Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 10.8 to the Company's Annual Report on Form 10-K for the year ending June 30, 2015 (File No. 001-36370)) |
| 10.7†** | Manufacturing License and Technology Transfer Agreement dated as of July 1, 2015 by and between Biogen MA Inc., and Applied Genetic Technologies Corporation |
| 10.8† | 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 ending June 30, 2015 (File No. 001-36370)) |
| 10.9† | 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 ending June 30, 2015 (File No. 001-36370)) |
| 10.10† | 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 ending June 30, 2015 (File No. 001-36370)) |
| 10.11 | 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)) |
| 10.12† | 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)) |

| | |
|---|---|
| 10.13† | 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)) |
| 10.14† | 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)) |
| 10.15† | 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)) |
| 10.16† | 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)) |
| 10.17† | 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)) |
| 10.18† | 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)) |
| 10.19† | 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)) |
| 10.20† | 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)) |
| 10.21 | Amended and Restated Investor Rights Agreement, dated as of November 15, 2012 (incorporated by reference to Exhibit 10.12 to the Company's Registration Statement on Form S-1 (File No. 333-193309)) |
| 10.22* | 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)) |
| 10.23* | 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)) |
| 10.24* | 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)) |
| 10.25* | 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)) |
| 10.26 | Form of Applied Genetic Technologies Corporation Warrant to Purchase Shares of Series A-1 Preferred Stock (incorporated by reference to Exhibit 10.17 to the Company's Registration Statement on Form S-1 (File No. 333-193309)) |
| 10.27 | Form of Applied Genetic Technologies Corporation Warrant to Purchase Shares of Series B-1 Preferred Stock (incorporated by reference to Exhibit 10.18 to the Company's Registration Statement on Form S-1 (File No. 333-193309)) |
| 10.28 | Warrant to Purchase Shares of Series A-1 Preferred Stock of Applied Genetic Technologies Corporation issued to Silicon Valley Bank and effective on September 23, 2005 (incorporated by reference to Exhibit 10.19 to the Company's Registration Statement on Form S-1 (File No. 333-193309)) |

| | |
|---|---|
| 10.29 | Warrant to Purchase Shares of Series A-1 Preferred Stock of Applied Genetic Technologies Corporation issued to Silicon Valley Bank and effective on June 30, 2006 (incorporated by reference to Exhibit 10.20 to the Company's Registration Statement on Form S-1 (File No. 333-193309)) |
| 10.30 | Warrant to Purchase Shares of Series A-1 Preferred Stock of Applied Genetic Technologies Corporation issued to Square 1 Bank on July 6, 2010 (incorporated by reference to Exhibit 10.21 to the Company's Registration Statement on Form S-1 (File No. 333-193309)) |
| 10.31 | Warrant to Purchase Shares of Series B-1 Preferred Stock of Applied Genetic Technologies Corporation issued to Square 1 Bank on August 31, 2012 (incorporated by reference to Exhibit 10.22 to the Company's Registration Statement on Form S-1 (File No. 333-193309)) |
| 10.32 | 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)) |
| 10.33 | 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)) |
| 10.34† | 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)) |
| 10.35† | 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)) |
| 10.36† | 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)) |
| 10.37* | 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) |
| 10.38* | Separation Agreement dated as of July 27, 2017 between Applied Genetic Technologies Corporation and Larry Bullock (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K filed with the SEC on August 2, 2017) |
| 14.1 | Applied Genetic Technologies Corporation Code of Ethics (incorporated by reference to Exhibit 14.1 to the Company's Current Report on Form 8-K filed with the SEC on July 3, 2018) |
| 16.1 | Letter to Securities and Exchange Commission from RSM US LLP, dated December 21, 2017 (incorporated by reference to Exhibit 16.1 to the Company's Current Report on Form 8-K filed with the SEC on December 21, 2017) |
| 23.1** | Consent of Ernst & Young LLP, Independent Registered Public Accounting Firm |
| 23.2** | Consent of RSM US 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.INS** | XBRL Instance Document |
| 10.SCH** | XBRL Taxonomy Extension Schema Document |

| | |
|---|---|
| 101.CAL** | XBRL Taxonomy Extension Calculation Linkbase Document |
| 101.DEF** | XBRL Taxonomy Extension Definition Linkbase Document |
| 101.LAB** | XBRL Taxonomy Extension Label Linkbase Document |
| 101.PRE** | XBRL Taxonomy Extension Presentation Linkbase Document |

* 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 Period | Additions: Charge (Benefit) to Expenses | Additions: To (from) Other Accounts | Deductions | End of Period |
|---|---|---|---|---|---|
| **Deferred Tax Valuation Allowance** | | | | | |
| Year 2018 | $ 40,303 | $ 1,911 | $ — | $ — | $ 42,214 |
| Year 2017 | $ 37,412 | $ 2,891 | $ — | $ — | $ 40,303 |
| Year 2016 | $ 36,845 | $ 567 | $ — | $ — | $ 37,412 |

**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 10, 2018

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<br>**Susan B. Washer** | Chief Executive Officer, President and Director (Principal Executive Officer) | September 10, 2018 |
| /s/William A. Sullivan<br>**William A. Sullivan** | Chief Financial Officer (Principal Financial and Accounting Officer) | September 10, 2018 |
| /s/ Scott Koenig<br>**Scott Koenig** | Director | September 10, 2018 |
| | | September 10, 2018 |
| /s/ Ed Hurwitz<br>**Ed Hurwitz** | Director | September 10, 2018 |
| /s/ Ivana Magovcevic-Liebisch<br>**Ivana Magovcevic-Liebisch** | Director | September 10, 2018 |
| /s/ Anne VanLent<br>**Anne VanLent** | Director | September 10, 2018 |
| /s/ James Rosen<br>**James Rosen** | Director | September 10, 2018 |

**Exhibit 10.5**

***EXECUTION VERSION***

***CONFIDENTIAL***

# COLLABORATION AND LICENSE AGREEMENT

**by and between**

**BIOGEN MA INC.**

**and**

**APPLIED GENETIC TECHNOLOGIES CORPORATION**

**July 1, 2015**

CONFIDENTIAL MATERIALS OMITTED AND FILED SEPARATELY WITH THE SECURITIES AND EXCHANGE COMMISSION. TRIPLE ASTERISKS [***] DENOTE OMISSIONS.

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CONFIDENTIAL MATERIALS OMITTED AND FILED SEPARATELY WITH THE SECURITIES AND EXCHANGE COMMISSION. TRIPLE ASTERISKS [***] DENOTE OMISSIONS.

**COLLABORATION AND LICENSE AGREEMENT**

This Collaboration and License Agreement (the "**Agreement**") is entered into as of July 1, 2015 (the "**Execution Date**"), by and between Biogen MA Inc., a corporation organized and existing under the laws of the Commonwealth of Massachusetts and having a principal place of business at 250 Binney Street, Cambridge, MA 02142 ("**Biogen**") and Applied Genetic Technologies Corporation, a corporation organized and existing under the laws of Delaware and having a principal place of business at 11801 Research Drive, Suite D, Alachua, FL 32615 ("**AGTC**"). Biogen and AGTC may each be referred to herein individually as a "**Party**" and collectively as the "**Parties**."

WHEREAS, AGTC is a biotechnology company that has developed technology relating to the identification, research, development and manufacture of gene therapy products in ophthalmology and other indications;

WHEREAS, Biogen has extensive experience and expertise in the development and commercialization of pharmaceutical products, and desires to acquire an exclusive license and exclusive option rights in the Territory (as defined below) to AGTC's patents, patent applications, technology, know-how and scientific and technical information in gene therapy;

WHEREAS, Biogen and AGTC wish to engage in collaborative development activities under the Initial Licensed Programs (as defined below) regarding potential Licensed Products (as defined below);

WHEREAS, Biogen and AGTC wish to collaborate on the conduct of the Discovery Programs (as defined below) to engage in research regarding potential Discovery Products (as defined below);

WHEREAS, subject to the terms of this Agreement, AGTC wishes to grant to Biogen, and Biogen wishes to receive from AGTC, an exclusive license in the Field (as defined below) in the Territory to research, develop, manufacture and commercialize Licensed Products (as defined below); and

WHEREAS, subject to the terms of this Agreement, AGTC wishes to grant to Biogen, and Biogen wishes to receive from AGTC, an exclusive option to receive an exclusive license in the Field in the Territory to research, develop, manufacture and commercialize Discovery Products (as defined below);

NOW THEREFORE, in consideration of the mutual promises and covenants set forth below and other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the Parties hereby agree as follows:

**1. DEFINITIONS**.

Capitalized terms not otherwise defined herein shall have the following meanings:

**1.1.** "**1934 Act**" has the meaning set forth in Section 14.5.

**1.2.** "**AAV**" means adeno-associated virus.

**1.3.** "**AAV Product**" means any product containing a recombinant AAV or AAV-based vector that delivers one or more transgenes or portions thereof to a human or animal subject.

**1.4.** "**Abandoned Program**" means any Discovery Program (i) which is a Terminated Discovery Program and for which Biogen does not elect to reinstate as a Discovery Program under Section 4.4.3, (ii) for which Biogen does not exercise the Option pursuant to Section 4.7, (iii) the ALD/[***] Discovery Program in the event the Non-Ophthalmology Discovery Program is designated in accordance with Section 4.4.4 or (iii) for which Biogen terminates for convenience under Section 16.6.1.

**1.5.** "[***]" has the meaning set forth in Section 1.234.

**1.6.** "[***]" has the meaning set forth in Section 1.26.

**1.7.** "[***] **Program**" means the existing Development program of AGTC or its Affiliates with respect to AAV Products targeting the [***].

**1.8.** "**Additional Biogen Activities**" has the meaning set forth in Section 3.1.3.

**1.9.** "**Additional Clinical Trial**" has the meaning set forth in Section 3.2.2(c).

**1.10.** "**Additional Taxes**" has the meaning set forth in Section 6.8.2.

**1.11.** "**Administration Costs**" has the meaning set forth in Exhibit C.

**1.12.** "**Affiliate**" means, as of any point in time and for so long as such relationship continues to exist with respect to any Person, any other Person that controls, is controlled by or is under common control with such Person. A Person shall be regarded as in control of another Person if it (a) owns or controls more than fifty percent (50%) of the equity securities of the subject Person entitled to vote in the election of directors (or, in the case of a Person that is not a corporation, for the election of the corresponding managing authority); provided, however, that in such circumstance, the term "Affiliate" shall not include subsidiaries or other entities in which a Person owns a majority of the ordinary voting power necessary to elect a majority of the board of directors or other governing board, but is subject to a contractual or other restriction that causes such Person to be unable to elect such majority, until such time as such restriction is no longer in effect; or (b) possesses, directly or indirectly, the power to direct or cause the direction of the management or policies of an such Person (whether through ownership of securities or other ownership interests, by contract or otherwise).

**1.13.** "**Agreement**" has the meaning set forth in the preamble.

**1.14.** "**AGTC**" has the meaning set forth in the preamble.

**1.15.** "**AGTC Assays**" means proprietary assays, and Know-How describing and Patent Rights Covering the same, that are Controlled by AGTC or any of its Affiliates as of the Execution Date or that come into the Control of AGTC or any of its Affiliates during the Term that are (a) useful for determining [***] or the like (b) actually used in a Collaboration Program and (c) disclosed to Biogen. Notwithstanding the foregoing, Schedule 1.23 sets forth the AGTC Assays as of the Execution Date, and will be updated as set forth in Section 1.23. For clarity, actual use in a Collaboration Program for purposes of this definition includes (i) a good faith belief by AGTC that a specific assay would be necessary or useful in such Collaboration Program and (ii) disclosure by AGTC in a discussion between the Parties regarding the potential use of such assay in such Collaboration Program.

**1.16.** "**AGTC Customer-Facing FTE**" has the meaning set forth in Exhibit B.

**1.17.** "**AGTC Improved Know-How**" means any Know-How, other than Joint Know-How, that is conceived, discovered, invented, created, made or reduced to practice or tangible medium by or on behalf of AGTC or any of its Affiliates or Sublicensees in the course of conducting activities under this Agreement, that constitutes an improvement or enhancement to any Biogen Technology used in such Collaboration Program.

**1.18.** "**AGTC Improved Patent Right**" means any Patent Right, other than a Joint Patent Right, that claims or discloses any AGTC Improved Know-How that is Invented by or on behalf of AGTC or any of its Affiliates or Sublicensees in the course of conducting activities under this Agreement.

**1.19.** "**AGTC Improved Technology**" means the AGTC Improved Know-How and the AGTC Improved Patent Rights.

**1.20.** "**AGTC Indemnified Party**" has the meaning set forth in Section 17.2.

**1.21.** "**AGTC Know-How**" means any Know-How, other than Joint Know-How, (a) that (i) AGTC or any of its Affiliates Control as of the Execution Date, (ii) is conceived, discovered, invented, created, made or reduced to practice or tangible medium by or on behalf of AGTC or any of its Affiliates or Sublicensees in the course of conducting activities under this Agreement, other than Program Data for an Initial Licensed Product and, after the Option Exercise Date, for a Discovery Product, or (iii) otherwise comes into the Control of AGTC or any of its Affiliates during the Term, provided that, in the case of any Know-How under this clause (iii) that comes into the Control of AGTC or its Affiliates through a license to Third Party IP Rights, Biogen has elected to take a sublicense to such Third Party IP Rights under Section 13.6.2(a) and (b) that is necessary or useful for the Development, Manufacture, Commercialization or use of any Licensed Product. For clarity, AGTC Know-How includes all AGTC Improved Know-How.

**1.22.** "**AGTC Patent Right**" means any Patent Right, other than a Joint Patent Right, (a) that (i) AGTC or any of its Affiliates Control as of the Execution Date or (ii) comes into the Control of AGTC or any of its Affiliates during the Term, provided that, in the case of any Patent Right under this clause (ii) that comes into the Control of AGTC or its Affiliates through a license to Third Party IP Rights, Biogen has elected to take a sublicense to such Third Party IP Rights under Section 13.6.2(a) and (b) claims or discloses any AGTC Know-How. Schedule 1.22-1 sets forth the AGTC Patent Rights as of the Execution Date, and will be updated on or prior to the Schedule Revision Date to include additional AGTC Patent Rights filed between the Execution Date and the Schedule Revision Date, if any. Schedule 1.22-2, which will be prepared by AGTC and attached hereto in accordance with Section 4.7, will set forth the AGTC Patent Rights which to AGTC's Knowledge Cover a Discovery Product as of the Option Exercise Date for such Discovery Product. Schedule 1.22-1 (and Schedule 1.22-2, as applicable) shall be updated by the Patent Representatives on a semi-annual basis to include additional Patent Rights, if any, that become AGTC Patent Rights after the Schedule Revision Date. Any AGTC Patent Right that is not listed on Schedule 1.22-1 or Schedule 1.22-2, but is otherwise described in this Section 1.22, shall still be considered an AGTC Patent Right hereunder. For purposes of clarity, AGTC Improved Patent Rights are included in the AGTC Patent Rights.

**1.23.** "**AGTC Platform**" means (a) the [***] Manufacturing Technology, (b) the Capsid Optimization Technology, (c) AGTC Assays and (d) the Promoter Technology. Schedule 1.23 further describes the AGTC Platform as of the Execution Date, and will be updated on or prior to the Schedule Revision Date to include additional technologies and assays that become part of the AGTC Platform after the Execution Date, if any. In addition, Schedule 1.23 shall be updated by the Patent Representatives on a semi-annual basis to include additional technologies and assays, if any, that become part of the AGTC Platform after the Schedule Revision Date.

**1.24.** "**AGTC Technology**" means the AGTC Know-How and the AGTC Patent Rights.

**1.25.** "**AGTC Third Party Agreement**" means any agreement between AGTC (or any of its Affiliates) and any Third Party pursuant to which AGTC has acquired, or, during the Term, acquires, Control of any of the AGTC Technology, including the Existing License Agreements.

**1.26.** "**ALD/[***] Discovery Program**" means a program of Pre-Funded Discovery Activities through Clinical Candidate Designation with respect to AAV Products that deliver [***] for the diagnosis, treatment or prevention of adrenoleukodystroyphy or [***], conducted by either or both Parties in accordance with the ALD/[***] Discovery Program Development Plan and the terms of this Agreement.

**1.27.** "**ALD/[***] Discovery Program Development Plan**" means the written plan for Pre-Funded Discovery Activities for the ALD/[***] Discovery Program to be conducted pursuant to this Agreement, as such written plan may be amended, modified or updated by the JDC in accordance with the terms of this Agreement. The initial ALD/[***] Discovery Program Development Plan is attached hereto as Exhibit A-4.

**1.28.** "**Alliance Manager**" has the meaning set forth in Section 2.3.1.

**1.29.** "**Antitrust Laws**" has the meaning set forth in Section 16.1.

**1.30.** "**Audited Party**" has the meaning set forth in Section 9.3.1.

**1.31.** "**Auditing Party**" has the meaning set forth in Section 9.3.1.

**1.32.** "**Available Program Notice**" has the meaning set forth in Section 4.4.2.

**1.33.** "**Available Programs**" has the meaning set forth in Section 4.4.2.

**1.34.** "**Bankruptcy Code**" means the United States Bankruptcy Code (11 U.S.C. §101 et seq.), as amended from time to time, or any successor statute.

**1.35.** "**Biogen**" has the meaning set forth in the preamble.

**1.36.** "**Biogen Customer-Facing FTE**" has the meaning set forth in Exhibit B.

**1.37.** "**Biogen Indemnified Party**" has the meaning set forth in Section 17.3.

**1.38.** "**Biogen Know-How**" means any Know-How, other than Joint Know-How, that (a) (i) is conceived, discovered, invented, created, made or reduced to practice or tangible medium by or on behalf of Biogen or any of its Affiliates or Sublicensees in the course of conducting activities under this Agreement, (ii) relates to one or more Licensed Products or the Development of any of the foregoing and (iii) is necessary or useful for AGTC to perform AGTC's obligations under this Agreement in accordance with any Development Plan or (b) is not Know-How defined in the foregoing subsection (a) and is Controlled by Biogen as of the Execution Date or otherwise comes into the Control of Biogen during the Term and that Biogen uses, subject to the provisions of Section 2.1.2(b)(vi), in the Development, Manufacture or Commercialization or use of the Licensed Products. For purposes of clarity, Biogen Platform Improvement Know-How is included in the Biogen Know-How.

**1.39.** "**Biogen Patent Challenge**" has the meaning set forth in Section 16.5.2.

**1.40.** "**Biogen Patent Right**" means any Patent Right, other than a Joint Patent Right, that (a) Biogen Controls as of the Execution Date or that comes into the Control of Biogen during the Term, and (b) claims or discloses any Biogen Know-How. Schedule 1.40 sets forth the Biogen Patent Rights as of the Execution Date, and will be updated on or prior to the Schedule Revision Date to include additional Biogen Patent Rights filed between the Execution Date and the Schedule Revision Date, if any. Schedule 1.40 shall be updated by the Patent Representatives on a semi-annual basis to include additional Patent Rights, if any, that become Biogen Patent Rights after the Schedule Revision Date, provided that any Biogen Patent Right that is not listed on Schedule 1.40, but is otherwise described in this Section 1.40 shall still be considered a Biogen Patent Right hereunder.

**1.41.** "**Biogen Platform Improvement Know-How**" means any Know-How, other than Joint Know-How, that is conceived, discovered, invented, created, made or reduced to practice or tangible medium by or on behalf of Biogen or any of its Affiliates or Sublicensees in the course of conducting activities under this Agreement, that constitutes an improvement or enhancement to the AGTC Platform.

**1.42.** "**Biogen Platform Improvement Patent Right**" means any Patent Right, other than a Joint Patent Right, that claims or discloses any Biogen Platform Improvement Know-How that is Invented by or on behalf of Biogen or any of its Affiliates or Sublicensees in the course of conducting activities under this Agreement.

**1.43.** "**Biogen Platform Improvement Technology**" means the Biogen Platform Improvement Know-How and the Biogen Platform Improvement Patent Rights.

**1.44.** "**Biogen Product**" has the meaning set forth in Section 8.1.3.

**1.45.** "**Biogen Technology**" means the Biogen Know-How and the Biogen Patent Rights.

**1.46.** "**BLA**" means a Biologics License Application (as defined in 21 C.F.R. 600 et. seq.), NDA, MAA or substantially similar application or submission filed with a Regulatory Authority in a country or group of countries, and any amendments thereto.

**1.47.** "**Budget**" means (a) a Development Budget or (b) a Commercialization Budget, as applicable.

**1.48.** "**Business Day**" means a day other than a Saturday, Sunday or bank or other public holiday in New York, New York.

**1.49.** "**Calendar Quarter**" means the respective periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 or December 31, for so long as this Agreement is in effect.

**1.50.** "**Calendar Year**" means any calendar year ending on December 31.

**1.51.** "**Capsid Optimization Know-How**" means all proprietary Know-How, other than Joint Know-How, Controlled by AGTC or any of its Affiliates as of the Execution Date or that comes into the Control of AGTC or any of its Affiliates during the Term that (a) relates to design, optimization, generation or selection of AAV capsids, where the AAV vector containing any such AAV capsid demonstrates improved efficacy of AAV based gene therapy, (b) is actually used in a Collaboration Program and (c) is actually disclosed to Biogen. For clarity, actual use in a Collaboration Program for purposes of this definition includes (i) a good faith belief

by AGTC that certain Know-How would be necessary or useful in such Collaboration Program and (ii) disclosure by AGTC in a discussion between the Parties regarding the potential use of such Know-How in such Collaboration Program.

**1.52.** "**Capsid Optimization Patent Rights**" means all Patent Rights, other than Joint Patent Rights, Controlled by AGTC or any of its Affiliates as of the Execution Date or that comes into the Control of AGTC or any of its Affiliates during the Term that claim or disclose any Capsid Optimization Know-How. Notwithstanding the foregoing, **Schedule 1.23** sets forth the Capsid Optimization Patent Rights as of the Execution Date, and will be updated as set forth in Section 1.23.

**1.53.** "**Capsid Optimization Technology**" means the Capsid Optimization Know-How and the Capsid Optimization Patent Rights.

**1.54.** "**cGMP**" means current Good Manufacturing Practices as specified in the United States Code of Federal Regulations, ICH Guideline Q7A, or equivalent laws, rules, or regulations of an applicable Regulatory Authority at the time of manufacture.

**1.55.** "**Change of Control**" means, with respect to a Party, (a) a merger or consolidation of such Party with a Third Party that results in the voting securities of such Party outstanding immediately prior thereto, or any securities into which such voting securities have been converted or exchanged, ceasing to represent more than fifty percent (50%) of the combined voting power of the surviving entity or the parent of the surviving entity immediately after such merger or consolidation, or (b) a transaction or series of related transactions in which a Third Party, together with its Affiliates, becomes the beneficial owner of more than fifty percent (50%) of the combined voting power of the outstanding securities of such Party, or (c) the sale or other transfer to a Third Party of all or substantially all of such Party's business or assets relating to one or more Collaboration Programs.

**1.56.** "**Clinical Candidate Designation**" means, with respect to any Discovery Program, the satisfaction by a clinical candidate in such Discovery Program of each of the criteria set forth on Schedule 1.56, as such Schedule may be amended by mutual agreement of the Parties.

**1.57.** "**Clinical Study Report**" means a report containing the results of a Clinical Trial of a pharmaceutical product that is consistent in content and format with applicable Law and regulatory guidance and with the guidelines of the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use (ICH) on Structure and Content of Clinical Study Reports.

**1.58.** "**Clinical Trial**" means a human clinical study conducted on sufficient numbers of human subjects that is designed to (a) establish that a pharmaceutical product is reasonably safe for continued testing, (b) investigate the safety and efficacy of the pharmaceutical product for its intended use, and to define warnings, precautions and adverse reactions that may be associated with the pharmaceutical product in the dosage range to be prescribed or (c) support Regulatory Approval of such pharmaceutical product or label expansion of such pharmaceutical product. Without limiting the foregoing, Clinical Trial includes any FIH Trial or Pivotal Trial.

**1.59.** "[***]" has the meaning set forth in Section 1.7.

**1.60.** "**Collaboration Program**" means (a) the XLRS Program, (b) the XLRP Program or (c) any Discovery Program, but excluding any Abandoned Program.

**1.61.** "**Combination Product**" means (a) any single product in finished form containing as active ingredients both a Licensed Product and one or more other pharmaceutically active compounds or substances (including, for the avoidance of doubt, a transgene other than the transgene identified in the definition of such Licensed Product), whether co-formulated or co-packaged (i.e., within a single box or sales unit); or (b) any Licensed Product sold in combination with one or more other products (such as devices) or services for a single invoice price; or (c) any Licensed Product sold where the sale of the Licensed Product is only available with the purchase of other products or services (such other pharmaceutically active compounds or substances, or such other products or services referred to in clauses (a) through (c) hereof, the "**Other Components**").

**1.62.** "**[***] Discovery Product**" has the meaning set forth in Section 4.7.

**1.63.** "**Commercial FTE Rate**" means a rate to be agreed upon by the JCC in accordance with Section 2.2.2(b)(ii), which rate shall be updated for each Calendar Year to a rate as agreed by the Parties, commencing on January 1, 2017, provided that, if the Parties cannot come to agreement with respect to such rate in any given year, such rate shall be updated for such year in accordance with the Consumer Price Index – All Urban Consumers, US City Average, All Items, 1982-84 = 100, published by the United States Department of Labor, Bureau of Labor Statistics (or its successor equivalent index) over the twelve (12) month period preceding January 1 of the applicable Calendar Year.

**1.64.** "**Commercialization Budget**" means the written budget set forth in any Commercialization Plan for Commercialization activities with respect to the applicable Cost Share Product, as such written plan may be amended, modified or updated in accordance with the terms of this Agreement.

**1.65.** "**Commercialization Plan**" means, with respect to any Cost Share Product, the written plan for Commercialization activities for such Cost Share Product to be conducted pursuant to this Agreement, which shall include, at a minimum, Commercialization activities, Commercialization Budgets and associated timelines, as such written plan may be amended, modified or updated in accordance with the terms of this Agreement.

**1.66.** "**Commercialize**" or "**Commercializing**" means to market, advertise, promote, distribute, offer for sale, sell, have sold, import, lease, export or otherwise commercialize a product, to conduct activities, other than Development and Manufacturing, in preparation for the foregoing activities, and to conduct post-approval studies. When used as a noun, "Commercialization" shall mean any and all activities involved in Commercializing.

**1.67.** "**Commercially Reasonable Efforts**" means, with respect to each Party, the efforts and resources typically used by biotechnology or biopharmaceutical companies similar in size and scope to such Party and its Affiliates to perform the obligation at issue, which efforts shall not be less than those efforts made with respect to other products at a similar stage of development or in a similar stage of product life, with similar developmental risk profiles, of similar market and commercial potential, taking into account the competitiveness of the market place, the proprietary position of the products, the regulatory structure involved, Regulatory Authority-approved labeling, product profile, the profitability of the applicable products (taking into account payments under this Agreement), issues of safety and efficacy, the likely timing of the product's entry into the market, the likelihood of receiving Regulatory Approval and other relevant scientific, technical and commercial factors.

**1.68.** "**Competing Program**" has the meaning set forth in Section 5.8.3(a).

**1.69.** "**Competitive Infringement**" has the meaning set forth in Section 13.5.4.

**1.70.** "**Competitive Product**" means, with respect to a Licensed Product, any AAV Product that is Commercialized or used in the same indication and targeting at least one of the same genes as such Licensed Product.

**1.71.** "**Confidential Information**" means, with respect to each Party, all Know-How or other information, including proprietary information (whether or not patentable) regarding or embodying such Party's technology, products, business information or objectives, that is communicated in any way or form by or on behalf of the Disclosing Party to the Receiving Party or its permitted recipients, on or after the Effective Date of this Agreement, whether or not such Know-How or other information is identified as confidential at the time of disclosure, provided that Know-How or other information not identified as confidential by or on behalf of the Disclosing Party shall be deemed to be Confidential Information of the Disclosing Party if the Receiving Party knows, or should have had a reasonable expectation, that such Know-How or other information communicated by or on behalf of the Disclosing Party is Confidential Information of the Disclosing Party. The terms and conditions of this Agreement shall be considered Confidential Information of both Parties. Notwithstanding any provision of this Section 1.71 to the contrary, Confidential Information does not include any (a) Joint Know-How or (b) Know-How or information that: (i) was already known by the Receiving Party (other than under an obligation of confidentiality to the Disclosing Party) at the time of disclosure by or on behalf of the Disclosing Party; (ii) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party; (iii) became generally available to the public or otherwise part of the public domain after its disclosure to the Receiving Party, other than through any act or omission of the Receiving Party in breach of its obligations under this Agreement; (iv) was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation not to disclose such information to the Receiving Party; or (v) was independently discovered or developed by or on behalf of the Receiving Party without the use of or access to any Confidential Information belonging to the Disclosing Party.

**1.72.** "**Continuing Party**" has the meaning set forth in Section 13.4.2(c).

**1.73.** "**Control**" or "**Controlled**" means with respect to any intellectual property right (including any Patent Right, Know-How or other data, information or Materials), possession of the ability (whether by sole or joint ownership, license or otherwise, other than pursuant to the license grants under this Agreement) to grant, without violating the terms of any agreement with a Third Party, a license, access or other right in, to or under such intellectual property right. Notwithstanding anything in this Agreement to the contrary, a Party shall be deemed to not Control any Patent Rights or Know-How that are owned or controlled by a Third Party described in the definition of "Change of Control", or such Third Party's Affiliates, (a) prior to the closing of such Change of Control, except to the extent that any such Patent Rights or Know-How were developed prior to such Change of Control through the use of such Party's technology, or (b) after such Change of Control to the extent that such Patent Rights or Know-How are developed or conceived by such Third Party or its Affiliates (other than such Party) after such Change of Control without using or incorporating or having access to such Party's technology.

**1.74.** "**Control Limitation Agreement**" means any written agreement or arrangement, other than an Existing License Agreement, executed by AGTC that has not been disclosed to Biogen in Schedule 15.2 which provides for the use or license of Technology by AGTC that would, but for limitations included in such agreement or arrangement on the ability of AGTC to use or grant a license or sublicense to or under such Technology, constitute AGTC Technology or that otherwise restricts AGTC's ability to use or license any Technology that would, but for such restrictions, constitute AGTC Technology.

**1.75.** "**Co-Promotion Agreement**" has the meaning set forth in Section 8.1.4(a).

**1.76.** "**Co-Promotion Option**" has the meaning set forth in Section 8.1.4(a).

**1.77.** "**Co-Promotion Product**" has the meaning set forth in Section 8.1.4(a).

**1.78.** "**Cost of Goods Sold**" means, as to each Licensed Product or Material, the fully burdened cost of such Licensed Product in final therapeutic form or Material. The fully burdened cost of each Licensed Product or Material will be determined in accordance with U.S. GAAP as applied by the Party performing or contracting for each stage of the Manufacturing process and will include direct labor, material, product testing costs and allocable overhead.

**1.79.** "**Cost of Sales**" has the meaning set forth in Exhibit C.

**1.80.** "**Cost Share Option**" has the meaning set forth in Section 6.2.2.

**1.81.** "**Cost Share Product**" has the meaning set forth in Section 6.3.

**1.82.** "**Cover**," "**Covering**" or "**Covers**" means, as to a product and Patent Rights, that, in the absence of a license granted under, or ownership of, such Patent Rights, the making, using, selling, offering for sale or importation of such product would infringe such Patent Rights or, as to a pending claim included in such Patent Rights, the making, using, selling, offering for sale or importation of such product would infringe such Patent Rights if such pending claim were to issue in an issued patent without modification.

**1.83.** "**Customer-Facing Activities**" has the meaning set forth in Exhibit B.

**1.84.** "**Customer-Facing Activities Plan**" has the meaning set forth in Exhibit B.

**1.85.** "**Customer-Facing FTE**" has the meaning set forth in Exhibit B.

**1.86.** "**Data Package**" means, with respect to any Discovery Program, all data, research reports and other information that reasonably demonstrates satisfaction of the criteria set forth on Schedule 1.56, which criteria may be amended from time to time by the JDC, subject to Biogen's final decision-making authority as set forth in Section 2.1.4(c).

**1.87.** "**Declining Party**" has the meaning set forth in Section 13.4.2(c).

**1.88.** "**Designation Notice**" has the meaning set forth in Section 4.4.2.

**1.89.** "**Develop**" or "**Developing**" means to discover, research or otherwise develop a product, including conducting non-clinical and clinical research and development activities such as toxicology, pharmacology and other discovery efforts, test method development and stability testing, process development, formulation development, delivery system development, quality assurance and quality control development, statistical analysis, clinical studies (including pre-approval studies), regulatory affairs, pharmacovigilance and Regulatory Approval and clinical study regulatory activities (including regulatory activities directed to obtaining pricing and reimbursement approvals). When used as a noun, "Development" shall mean any and all activities involved in Developing.

**1.90.** "**Development Budget**" means the written budget set forth in any Development Plan, as such written budget may be amended, modified or updated by the JDC in accordance with the terms of this Agreement.

**1.91.** "**Development Costs**" means, as to each Licensed Product (or, if applicable, any Discovery Program product candidate), the FTE Costs and other direct costs actually incurred, excluding capital expenditures, to obtain the authorization or have the ability to Manufacture, formulate, fill, ship and/or sell such Licensed Product (or, if applicable, such Discovery Program product candidate) in the Field in commercial quantities in the Territory. Development Costs shall include but are not limited to the cost of studies on the toxicological, pharmacokinetic, metabolic or clinical aspects of such Licensed Product (or, if applicable, such Discovery Program product candidate) conducted internally or by individual investigators, or consultants necessary for the purpose of obtaining or maintaining Regulatory Approval of such Licensed Product (or, if applicable, such Discovery Program product candidate) in the Field by a Regulatory Authority in a country of the Territory, and costs for preparing, submitting, reviewing or developing data or information for the purpose of a submission to a Regulatory Authority to obtain or maintain Regulatory Approval of such Licensed Product (or, if applicable, such Discovery Program product candidate) in the Field in a country of the Territory as well as costs of assay development and process development scale-up and recovery (including plant costs). Development Costs shall include expenses for compensation, benefits and travel and other employee-related expenses, as well as data management, statistical designs and studies, document preparation, and other expenses associated with the clinical testing program. Development Costs that are to be paid solely by one but not both of the Parties as set forth in Section 3.2 or Section 4.6 shall not be included in the determination of Operating Profits (Losses) for the purposes of Exhibit C. For clarity, "Development Costs" include any costs incurred in connection with the Pre-Funded Activities to the extent that such costs otherwise meet the definition of "Development Costs" hereunder, including any such costs intended to be covered by the R&D Pre-Funding.

**1.92.** "**Development Plan**" means (a) the XLRS Development Plan, (b) the XLRP Development Plan or (c) any Discovery Program Development Plan, which shall include at a minimum, Development activities, Development Budgets and associated timelines for the applicable Collaboration Program for at least the next three (3) years, or, if Biogen's internal development plans for similarly situated products are a shorter time period, such shorter time period. Development Plans shall be updated annually on a rolling basis pursuant to Section 2.1.2(b)(i).

**1.93.** "**Disclosing Party**" has the meaning set forth in Section 14.1.

**1.94.** "**Discovery Event Milestone Payment**" has the meaning set forth in Section 6.5.2.

**1.95.** "**Discovery Product**" shall mean any AAV Product (a) that is generated by, is derived from or is the subject of a Discovery Program for which Biogen has exercised the Option in accordance with Section 4.7 and (b) with respect to which, absent the license granted to Biogen in Section 5.1, the, Development, Manufacture, Commercialization or use by Biogen as contemplated under this Agreement would infringe a Valid Claim of the AGTC Patent Rights or the Joint Patent Rights or misappropriate AGTC Know-How or Joint Know-How. For clarity, "Discovery Product" includes any AAV Product that is generated by, is derived from or is the subject of a Substitute Discovery Program that otherwise meets the definition of a "Discovery Product", but excludes any product generated by or subject to an Abandoned Program.

**1.96.** "**Discovery Program**" means (a) the [***] Discovery Program, (b) the ALD/[***] Discovery Program, (c) the Non-Ophthalmology Discovery Program, if applicable, (d) the [***], (e) the [***] or (f) any Substitute Discovery Program, but excluding any Abandoned Program.

**1.97.** "**Discovery Program Development Plan**" means (a) the [***] Discovery Program Development Plan, (b) the ALD/[***] Discovery Program Development Plan, (c) any Development Plan for the Non-Ophthalmology Discovery Program approved by the JDC pursuant to Section 2.1.2(b)(ii), (d) the [***], (e) [***] or (f) any Development Plan for a Substitute Discovery Program approved by the JDC pursuant to Section 2.1.2(b)(ii).

**1.98.** "**Discovery Program Substitution** Date" has the meaning set forth in Section 4.4.2.

**1.99.** "**Distribution Costs**" has the meaning set forth in Exhibit C.

**1.100.** "**Distributor**" means any Third Party which purchases its requirements for Licensed Product in a country from Biogen or its Affiliates or Sublicensees and is appointed as a distributor to distribute, market and resell such Licensed Product in such country, even if such Third Party is granted ancillary rights to develop, package or obtain regulatory approvals of such Licensed Product in order to distribute, market or sell such Licensed Product in such country.

**1.101.** "**Dollar**" means the United States Dollar.

**1.102.** "[***]" means [***].

**1.103.** "[***]" means the [***] and the [***].

**1.104.** "[***]" means a program of Pre-Funded Discovery Activities through Clinical Candidate Designation with respect to AAV Products that deliver [***], which [***] shall be designated by the JDC in accordance with Section 2.1.2(b)(iii), for the diagnosis, treatment or prevention of [***], conducted by either or both Parties in accordance with the [***] and the terms of this Agreement.

**1.105.** "[***]" means a program of Pre-Funded Discovery Activities through Clinical Candidate Designation with respect to AAV Products that deliver [***], which [***] shall be designated by the JDC in accordance with Section 2.1.2(b)(iii), for the diagnosis, treatment or prevention of [***], conducted by either or both Parties in accordance with the [***] and the terms of this Agreement.

**1.106.** "[***] **Development Plan**" means the written plan for Pre-Funded Discovery Activities for the [***] to be conducted pursuant to this Agreement, as such written plan may be amended, modified or updated by the JDC in accordance with the terms of this Agreement. The initial [***] Development Plan shall be prepared and approved by the JDC in accordance with Section 2.1.2(b)(ii) and shall be attached hereto as, and shall supersede and replace, Exhibit A-5.

**1.107.** "[***] **Development Plan**" means the written plan for Pre-Funded Discovery Activities for the [***] to be conducted pursuant to this Agreement, as such written plan may be amended, modified or updated by the JDC in accordance with the terms of this Agreement. The initial [***] Development Plan shall be prepared and approved by the JDC in accordance with Section 2.1.2(b)(ii) and shall be attached hereto as, and shall supersede and replace, Exhibit A-6.

**1.108.** "[***]" means [***].

**1.109.** "**Effect**" has the meaning set forth in Section 1.179.

**1.110.** "**Effective Date**" means the later of (a) the Execution Date or (b) the HSR Clearance Date.

**1.111.** "**Event Milestone Payment**" has the meaning set forth in Section 6.4.1.

**1.112.** "**Execution Date**" has the meaning set forth in the preamble.

**1.113.** "**Existing License Agreements**" means those certain license agreements as may be amended from time to time listed on Schedule 15.2.7 (as such Schedule may be updated in accordance with Section 15.2).

**1.114.** "**Existing Licensors**" means the licensors under the Existing License Agreements.

**1.115.** "**FD&C Act**" means the United States Federal Food, Drug, and Cosmetic Act, as amended, and the rules and regulations promulgated thereunder.

**1.116.** "**FDA**" means the United States Food and Drug Administration or any successor agency thereto.

**1.117.** "**Field**" means the diagnosis, treatment or prevention of disease in humans or animals in any and all indications.

**1.118.** "**FIH Trial**" means, with respect to a Licensed Product, the first Clinical Trial of such Licensed Product.

**1.119.** "**FIH Trial Completion**" means, with respect to a Licensed Product, the earliest of (a) [***] after database lock for the FIH Trial for such Licensed Product after the last visit of the last subject in such FIH Trial for measuring data for the primary endpoint for such FIH Trial, (b) (i) with respect to the XLRS Product, [***] after the initial dosing of the last subject in the FIH Trial for such Licensed Product or (ii) with respect to the XLRP Product, such reasonable similar time period as mutually agreed by the Parties in writing prior to the start of such FIH Trial or (c) [***] of the FIH Trial for such Licensed Product, if applicable.

**1.120.** "**First Commercial Sale**" means, with respect to any Licensed Product and with respect to any country of the Territory, the first sale of such Licensed Product by Biogen or an Affiliate or Sublicensee of Biogen to a Third Party in such country after such Licensed Product has been granted Regulatory Approval by the appropriate Regulatory Authority(ies) for Commercialization in such country.

**1.121.** "**FTE Costs**" means costs actually incurred by a Party in accordance with the applicable FTE Rate.

**1.122.** "**FTE Rate**" means the R&D FTE Rate or the Commercial FTE Rate, as applicable.

**1.123.** "**GAAP**" means United States generally accepted accounting principles, consistently applied.

**1.124.** "**GCP**" means good clinical practices, which are the then current standards for Clinical Trials for pharmaceuticals, as set forth in the FD&C Act or other applicable Law, and such standards of good clinical practice as are required by the Regulatory Authorities of the European Union and other organizations and Governmental Authorities in countries for which the applicable Licensed Product is intended to be developed, to the extent such standards are not less stringent than United States GCP.

**1.125.** "**Gene Therapy Product**" means any product containing a virus-based vector that delivers one or more transgenes to a human or animal subject.

**1.126.** "**GLP**" means the then-current good laboratory practice standards promulgated or endorsed by the FDA as defined in 21 C.F.R. Part 58 or the successor thereto, or comparable regulatory standards in jurisdictions outside the United States.

**1.127.** "**Governmental Authority**" means any court, agency, department, authority or other instrumentality of any national, state, county, city or other political subdivision.

**1.128.** "**Gross Sales**" has the meaning set forth in Section 1.184.

**1.129.** "[***]" means [***].

**1.130.** "[***] **Agreements**" means the License Agreement, dated [***], as amended [***], by and between AGTC and [***], as may be further amended from time to time, and the License Agreement, dated [***], as amended [***], by and between AGTC and [***], as may be further amended from time to time.

**1.131.** "[***] **Biological Material(s)**" has the meaning set forth in Section 11.4.1(a).

**1.132.** "[***] **Claims**" has the meaning set forth in Section 17.5.4.

**1.133.** "[***] **Indemnitees**" has the meaning set forth in Section 17.5.4.

**1.134.** "[***] **Product**" has the meaning set forth in Section 11.4.1(a).

**1.135.** "[***] **Virus**" has the meaning set forth in Section 11.4.1(a).

**1.136.** "**HSR Act**" means the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and the rules and regulations promulgated thereunder.

**1.137.** "**HSR Clearance Date**" means the earliest date on which the Parties have actual knowledge that all applicable waiting periods under the HSR Act with respect to the transactions contemplated hereunder have expired or have been terminated.

**1.138.** "**HSR Filing**" means a filing by Biogen and AGTC with the United States Federal Trade Commission and the Antitrust Division of the United States Department of Justice of a Notification and Report Form for Certain Mergers and Acquisitions (as that term is defined in the HSR Act) with respect to the matters set forth in this Agreement, together with all required documentary attachments thereto.

**1.139.** "[***] **Manufacturing Know-How**" means all proprietary Know-How, other than Joint Know-How, Controlled by AGTC or any of its Affiliates as of the Execution Date or that comes into the Control of AGTC or any of its Affiliates during the Term that (a) relates to the production, manufacture, or expression of recombinant AAV using an [***] helper virus, (b) is actually used in a Collaboration Program and (c) is disclosed to Biogen. For clarity, actual use in a Collaboration Program for purposes of this definition includes (i) a good faith belief by AGTC that certain Know-How would be necessary or useful in such Collaboration Program and (ii) disclosure by AGTC in a discussion between the Parties regarding the potential use of such Know-How in such Collaboration Program.

**1.140.** "[***] **Manufacturing Patent Rights**" means all Patent Rights, other than Joint Patent Rights, Controlled by AGTC or any of its Affiliates as of the Execution Date or that comes into the Control of AGTC or any of its Affiliates during the Term that claim or disclose any [***] Manufacturing Know-How. Notwithstanding the foregoing, Schedule 1.23 sets forth the [***] Manufacturing Patent Rights as of the Execution Date, and will be updated as set forth in Section 1.23.

**1.141.** "[***] **Manufacturing Technology**" means the [***] Manufacturing Know-How and the [***] Manufacturing Patent Rights.

**1.142.** "**IND**" means an Investigational New Drug Application submitted under the FD&C Act, or an analogous application or filing with any analogous agency or Regulatory Authority outside of the United States under any analogous foreign Law for the purposes of obtaining permission to conduct human clinical studies.

**1.143.** "**Indemnified Party**" has the meaning set forth in Section 17.4.

**1.144.** "**Indemnifying Party**" has the meaning set forth in Section 17.4.

**1.145.** "**Initial Licensed Product**" means (a) the XLRS Product or (b) the XLRP Product.

**1.146.** "**Initial Licensed Program**" means (a) the XLRS Program or (b) the XLRP Program.

**1.147.** "**Insolvency Event**" has the meaning set forth in Section 16.7.

**1.148.** "**Insolvent Party**" has the meaning set forth in Section 16.7.

**1.149.** "**Invented**" means the act of invention by inventors, in accordance with statutes and regulations regarding inventorship as established under United States patent law, including case law, rules and guidelines associated therewith. "**Invent**" or "**Invents**" have correlative meanings.

**1.150.** "**JHU**" means Johns Hopkins University.

**1.151.** "**JHU Inventors**" has the meaning set forth in Section 17.5.3.

**1.152.** "**Joint Commercialization Committee**" or "**JCC**" has the meaning set forth in Section 2.2.1.

**1.153.** "**Joint Development Committee**" or "**JDC**" has the meaning set forth in Section 2.1.1.

**1.154.** "**Joint Improved Know-How**" means Joint Know-How that constitutes an improvement or enhancement to any Biogen Technology.

**1.155.** "**Joint Improved Patent Right**" means any Patent Right that claims or discloses any Joint Improved Know-How that is Invented jointly by or on behalf of (i) on the one hand, AGTC or any of its Affiliates or Sublicensees and (ii) on the other hand, Biogen or any of its Affiliates or Sublicensees, in each case, in the course of conducting activities under this Agreement.

**1.156.** "**Joint Improved Technology**" means the Joint Improved Know-How and the Joint Improved Patent Rights.

**1.157.** "**Joint Know-How**" means Know-How that is conceived, discovered, invented, created, made or reduced to practice or tangible medium jointly by or on behalf of (i) on the one hand, AGTC or any of its Affiliates or Sublicensees and (ii) on the other hand, Biogen or any of its Affiliates or Sublicensees, in each case, in the course of conducting activities under this Agreement.

**1.158.** "**Joint Patent Right**" means any Patent Right that claims or discloses Know-How that is Invented jointly by or on behalf of (i) on the one hand, AGTC or any of its Affiliates or Sublicensees and (ii) on the other hand, Biogen or any of its Affiliates or Sublicensees, in each case, in the course of conducting activities under this Agreement.

**1.159.** "**Joint Platform Improvement Know-How**" means Joint Know-How that constitutes an improvement or enhancement to the AGTC Platform.

**1.160.** "**Joint Platform Improvement Patent Right**" means any Patent Right that claims or discloses any Joint Platform Improvement Know-How that is Invented jointly by or on behalf of (i) on the one hand, AGTC or any of its Affiliates or Sublicensees and (ii) on the other hand, Biogen or any of its Affiliates or Sublicensees, in each case, in the course of conducting activities under this Agreement.

**1.161.** "**Joint Platform Improvement Technology**" means the Joint Platform Improvement Know-How and the Joint Platform Improvement Patent Rights.

**1.162.** "**Joint Technology**" means the Joint Know-How and the Joint Patent Rights.

**1.163.** "**Know-How**" means intellectual property, data, results, pre-clinical and clinical protocols and study data, chemical structures, chemical sequences, information, inventions, know-how, formulas, trade secrets, techniques, methods, processes, procedures and developments, whether or not patentable; except that Know-How does not include Patent Rights claiming any of the foregoing. For clarity, "Know-How" does not include any Materials.

**1.164.** "**Knowledge**" means, with respect to AGTC, the then, actual knowledge, after inquiry of patent counsel, but without any other duty of inquiry, of the Chief Executive Officer, Chief Financial Officer, Chief Medical Officer, Chief Business Officer, Chief Scientific Officer and Senior Director – Process Development, Senior Director – Research and Pre-Clinical Studies and any other person performing substantially the same functions as any of the foregoing.

**1.165.** "**Law**" means any law, statute, rule, regulation, order, judgment or ordinance of any Governmental Authority.

**1.166.** "**Liability**" has the meaning set forth in Section 17.2.

**1.167.** "**Licensed Activities**" has the meaning set forth in Section 13.6.1.

**1.168.** "**Licensed Product**" means (a) any XLRS Product, (b) any XLRP Product or (c) any Discovery Product.

**1.169.** "**Licensed Program**" means (a) the XLRS Program, (b) the XLRP Program or (c) any Discovery Program for which Biogen has exercised the Option in accordance with Section 4.7.

**1.170.** "**Limited Milestone Payment**" has the meaning set forth in Section 6.2.1.

**1.171.** "**MAA**" means a Marketing Authorization Application for the applicable Licensed Product under the centralized European procedure.

**1.172.** "**Major EU Market Countries**" means the following countries: [***].

**1.173.** "**Major Market Countries**" means the following countries: [***].

**1.174.** "**Manufacture**" or "**Manufacturing**" means activities directed to making, producing, manufacturing, processing, filling, finishing, packaging, labeling, quality control testing and quality assurance release, shipping or storage of a product.

**1.175.** "**Manufacturing Precedent Period**" has the meaning set forth in Section 2.1.4(a)(v).

**1.176.** "**Manufacturing Technology Agreement**" means the Manufacturing License and Technology Transfer Agreement, executed by the Parties on even date herewith.

**1.177.** "**Marketing Application**" means an application, submitted to a Regulatory Authority in any jurisdiction, for Regulatory Approval required in order to Commercialize a product as a drug, including a BLA.

**1.178.** "**Marketing Costs**" has the meaning set forth in Exhibit C.

**1.179.** "**Material Adverse Event**" means, with respect to AGTC, an event, occurrence, development or change (a) that occurs after the Execution Date and prior to or on the HSR Clearance Date (each event, occurrence, development or change that satisfies the criteria in this clause (a), an "**Effect**") and (b) that when taken together with all other Effects, has or would reasonably be expected to have a material adverse effect on the AGTC Technology taken as a whole, the Parties' practice of the AGTC Technology taken as a whole and as contemplated by this Agreement or the Development, Manufacture or Commercialization of Licensed Products as contemplated by this Agreement, except for any Effect resulting from (1) any change in applicable Law or the interpretation thereof other than any change in regulations promulgated by the FDA or any other Regulatory Authority or any change in the interpretation of any regulation promulgated by the FDA or any other Regulatory Authority, (2) any event or change affecting the pharmaceutical industry as a whole or the gene therapy industry in particular that does not have a disproportionate effect on the practice of the AGTC Technology taken as a whole and as contemplated by this Agreement or the Development, Manufacture or Commercialization of Licensed Products as contemplated by this Agreement, (3) any event or change affecting Biogen, provided that such event or change is not caused by AGTC or (4) announcement of entry into this Agreement.

**1.180.** "**Materials**" means any biological or chemical materials in each case, that are necessary or useful to exploit the licenses granted to Biogen under this Agreement including, but not limited to, cell lines (*e.g.*, parental cell lines and any non-commercially available cell lines or cell-based assays, for example, the [***] and [***] cell lines [***], appropriate rep-cap-, gene of interest- and any other related [***] seed stocks, any material-, platform- or product-specific reference materials including any platform or product-specific assay controls and reagents that are not readily available as standard commercial items.

**1.181.** "**Medical Education Costs**" has the meaning set forth in Exhibit C.

**1.182.** "**Milestone/Royalty Option**" has the meaning set forth in Section 6.2.2.

**1.183.** "**NDA**" means a New Drug Application (as more fully described in 21 C.F.R. Parts 314 et seq. or its successor regulation).

**1.184.** "**Net Sales**" means, with respect to a Licensed Product in a country in the Territory, the gross amount invoiced by Biogen, its Affiliates or Sublicensees for the sale or other disposition of such Licensed Product in such country to Third Parties (including Distributors, wholesalers and end-users) ("**Gross Sales**"), less the following deductions (such deductions, collectively, "**Sales Returns and Allowances**"):

(a) sales returns and allowances actually paid, granted or accrued on the Licensed Product, including trade, quantity, prompt pay and cash discounts and any other adjustments, including those granted on account of price adjustments or billing errors;

(b) credits or allowances given or made for rejection, recall, return or wastage replacement of, and for uncollectible amounts on, Licensed Product or for rebates or retroactive price reductions (including Medicare, Medicaid, managed care and similar types of rebates and chargebacks);

(c) taxes, duties or other governmental charges levied on or measured by the billing amount for Licensed Product, as adjusted for rebates and refunds, including without limitation pharmaceutical excise taxes (such as those imposed on a Licensed Product by the United States Patient Protection and Affordable Care Act of 2010 and other comparable laws), but which shall not include any tax, duty, or other charge imposed on or measured by net income (however denominated) or any franchise taxes, branch profits taxes, or similar tax; and

(d) charges for freight, customs and insurance directly related to the distribution of the Licensed Product and wholesaler and Distributor administration fees; and

(e) other future similar deductions, taken in the ordinary course of business or in accordance with GAAP and Biogen's standard practices;

to the extent such deductions: (i) are reasonable and customary, (ii) included in the gross invoiced sales price for the Licensed Product or otherwise directly paid, allowed, accrued, or incurred by such Party, its Affiliates or Sublicensees with respect to the sale of such Licensed Product (iii) applicable and in accordance with standard allocation procedures, (iv) have not already been deducted or excluded, (v) are incurred in the ordinary course of business in type and amount consistent with good industry practice, and (vi) except with respect to the uncollectible amounts and pharmaceutical excise taxes described in subsections (b) and (c) above, are determined in accordance with, and as recorded in revenues under, GAAP. Net Sales shall not be imputed to transfers of Licensed Product without consideration or for nominal consideration for use in any Clinical Trial, or for any bona fide charitable, compassionate use or indigent patient program purpose where Licensed Products are sold at or below Cost of Goods Sold or as a sample. For the avoidance of doubt, in the case of any transfer of any Licensed Product between or among Biogen and its Affiliates or Sublicensees for resale, Net Sales shall be determined based on the sale made by such Affiliate or Sublicensee to a Third Party.

Notwithstanding the foregoing, in the event a Licensed Product is sold as a component of a Combination Product in any country in the Territory in any Calendar Quarter, Net Sales shall be calculated by multiplying the Net Sales of the Combination Product (calculated in the same manner as set forth above as if the Combination Product were a Licensed Product) in such country during such Calendar Quarter by the fraction A/(A+B), where A is the average Net Sales of the Licensed Product when sold separately in such country during such Calendar Quarter and B is the average Net Sales of the Other Components included in the Combination Product (calculated in the same manner as set forth above as if the Other Components were Licensed Product) when sold separately in such country during such Calendar Quarter. In the event that no separate sales of the Licensed Product or any Other Components included in a Combination Product are made by Biogen, its Affiliates or Sublicensees in a country during a Calendar Quarter in which such Combination Product is sold in such country, the average Net Sales in the above described equation shall be replaced with reasonable good faith estimate of the fair market value, as mutually determined by the Parties, of the Licensed Product and each of the Other Components included in such Combination Product.

**1.185.** "**Non-Disclosing Party**" has the meaning set forth in Section 14.6.4.

**1.186.** "**Non-Ophthalmology Discovery Program**" has the meaning set forth in Section 4.4.4.

**1.187.** "**Obligated Party**" has the meaning set forth in Section 3.3.2.

**1.188.** "**Operating Profit or Loss**" or "**Operating Profits (Losses)**" has the meaning set forth in Exhibit C.

**1.189.** "**Option**" has the meaning set forth in Section 4.7.

**1.190.** "**Option Exercise Date**" has the meaning set forth in Section 4.7.

**1.191.** "**Option Exercise Period**" has the meaning set forth in Section 4.7.

**1.192.** "**Option Fee**" shall have the meaning set forth in Section 6.5.1.

**1.193.** "**Orphan Drug Designation**" means a grant by the FDA of a request for designation under Section 526 of the Federal Food, Drug, and Cosmetic Act as amended by section 2 of the Orphan Drug Act (sections 525-528 (21 U.S.C. 360aa-360dd)) in the United States or any analogous grant by a Regulatory Authority in any other country in the Territory.

**1.194.** "**Orphan Drug Exclusivity**" means, with respect to a Licensed Product, a grant of a period of marketing exclusivity by a Regulatory Authority for such Licensed Product in connection with an Orphan Drug Designation for such Licensed Product.

**1.195.** "**Other Components**" shall have the meaning set forth in Section 1.61.

**1.196.** "**Other Operating Income/Expense**" has the meaning set forth in Exhibit C.

**1.197.** "**Out-of-Pocket Costs**" means, with respect to a Party, costs and expenses paid by such Party to Third Parties (or payable to Third Parties and accrued in accordance with GAAP), other than Affiliates or employees of such Party.

**1.198.** "**Party**" or "**Parties**" has the meaning set forth in the preamble.

**1.199.** "**Patent Costs**" has the meaning set forth in Exhibit C.

**1.200.** "**Patent Representative**" has the meaning set forth in Section 13.3.1(a).

**1.201.** "**Patent Rights**" means the rights and interests in and to issued patents and pending patent applications in any country, jurisdiction or region (including inventor's certificates and utility models), including all provisionals, non-provisionals, substitutions, continuations, continuations-in-part, divisionals, renewals and all patents granted thereon, and all reissues, reexaminations, extensions, confirmations, revalidations, registrations and patents of addition thereof, including supplementary protection certificates, PCTs, pediatric exclusivity periods and any foreign equivalents to any of the foregoing.

**1.202.** "[***] **Agreement**" means the [***] License Agreement, dated [***], as amended June 30, 2015, by and between AGTC and [***], as may be further amended from time to time.

**1.203.** "**Person**" means an individual, sole proprietorship, partnership, limited partnership, limited liability partnership, corporation, limited liability company, business trust, joint stock company, trust, incorporated association, joint venture or similar entity or organization, including a government or political subdivision or department or agency of a government.

**1.204.** "**Pivotal Trial**" means a human Clinical Trial of a Licensed Product which is intended to be sufficient for obtaining Regulatory Approval, or is according to 21 C.F.R. §312.21(c), as amended, or its equivalent, as appropriate, in foreign jurisdictions.

**1.205.** "**Post-Funding Development Activities**" means, with respect to each Initial Licensed Program, any activities related to the Development of an Initial Licensed Product in such Initial Licensed Program under the applicable Development Plan that are not Pre-Funded Activities.

**1.206.** "**Preclinical Studies**" means any preclinical pharmacokinetic, toxicology or other study relating to one or more Licensed Products.

**1.207.** "**Pre-Funded Activities**" means (a) with respect to the XLRS Program, any Development activities conducted under the XLRS Development Plan for the XLRS Product [***] and (b) with respect to the XLRP Program, any Development activities conducted under the XLRP Development Plan for a XLRP Product [***], in each case ((a) and (b)), that are designated under the applicable Development Budget to be funded by the R&D Pre-Funding.

**1.208.** "**Pre-Funded Discovery Activities**" means, with respect to any of the [***] Discovery Program, the ALD/[***] Discovery Program, the Non-Ophthalmology Discovery Program, if applicable, the [***], the [***] or any Substitute Discovery Program, any Development activities that take place under and pursuant to a Discovery Program Development Plan for such Discovery Program in accordance with this Agreement.

**1.209.** "[***] **Program**" means, at any time, a research program being conducted solely by or on behalf of AGTC in an indication or involving a gene target, for which program AGTC has not (i) already granted rights to or entered into a fully executed term sheet (which may be a non-binding term sheet) contemplating the grant of rights to a Third Party that would preclude the granting of rights to Biogen for such program as a Discovery Program to the extent contemplated by this Agreement or (ii) begun [***] for purposes of selecting a candidate comprising a [***] to use for such indication or involving such gene target.

**1.210.** "**Price Approval**" means, in any country where a Governmental Authority authorizes reimbursement for, or approves or determines pricing for, pharmaceutical products, receipt (or, if required to make such authorization, approval or determination effective, publication) of such reimbursement authorization or pricing approval or determination (as the case may be).

**1.211.** "**Product-Specific Know-How**" means all Know-How Controlled by a Party or an Affiliate thereof, or jointly by the Parties or one of each of their respective Affiliates, as of the Execution Date or during the Term that relates exclusively to the composition, formulation or use of a Licensed Product or methods of manufacture exclusively related to a Licensed Product, but excluding Joint Platform Improvement Know-How and Biogen Platform Improvement Know-How.

**1.212.** "**Product-Specific Patent Right**" means all Patent Rights Controlled by a Party or an Affiliate thereof, or jointly by the Parties or their respective Affiliates, as of the Execution Date or during the Term that exclusively Covers the composition, formulation or use of a Licensed Product or methods of manufacture exclusively related to a Licensed Product, but excluding Joint Platform Improvement Patent Rights and Biogen Platform Improvement Patent Rights. Notwithstanding the foregoing, Schedule 1.212 sets forth the AGTC Patent Rights that are Product-Specific Patent Rights as of the Execution Date, and will be updated (i) on or prior to the Schedule Revision Date to include additional AGTC Patent Rights filed between the Execution Date and the Schedule Revision Date that are Product-Specific Patent Rights as of the Effective Date, if any and (ii) on or prior to the Option Exercise Date for each Discovery Program with respect to the Discovery Product for which the Option is being exercised. Schedule 1.212 shall be updated by the Patent Representatives on a semi-annual basis to include additional AGTC Patent Rights that are Product-Specific Patent Rights, if any, that become AGTC Patent Rights after the Schedule Revision Date, provided that any AGTC Patent Right that is not listed on Schedule 1.212, but is otherwise described in this Section 1.212 and confirmed by the Patent Representatives to be a Product-Specific Patent Right pursuant to Section 13.3, shall still be considered a Product-Specific Patent Right hereunder.

**1.213.** "**Product-Specific Technology**" means the Product-Specific Know-How and the Product-Specific Patent Rights.

**1.214.** "**Program Data**" means all preclinical and clinical data and results, including all study databases, generated by either Party or both Parties or their respective Affiliates, Subcontractors or agents in the course of performance of their activities pursuant to the Licensed Programs (including under any Development Plan). For clarity, any such data with respect to a Discovery Program generated prior to the Option Exercise Date in the course of performance of activities pursuant to such Discovery Program (including under the applicable Discovery Program Development Plan) shall be deemed "Program Data" as of the Option Exercise Date with respect to such Discovery Program.

**1.215.** "**Promoter Know-How**" means all proprietary Know-How, other than Joint Know-How, Controlled by AGTC or any of its Affiliates as of the Execution Date or that comes into the Control of AGTC or any of its Affiliates during the Term that (a) relates to the design, selection or sequence of nucleic acid signaling sequences, inverted terminal repeats, long terminal repeats, introns, or microRNA target sequences that the foregoing, when contained in an AAV vector, demonstrate targeted expression of a recombinant AAV comprising a transgene to a specific cell type or increased expression in a variety of cell types, (b) is actually used in a Collaboration Program and (c) is disclosed to Biogen. For clarity, actual use in a Collaboration Program for purposes of this definition includes (i) a good faith belief by AGTC that certain Know-How would be necessary or useful in such Collaboration Program and (ii) disclosure by AGTC in a discussion between the Parties regarding the potential use of such Know-How in such Collaboration Program.

**1.216.** "**Promoter Patent Rights**" means Patent Rights, other than Joint Patent Rights, Controlled by AGTC or any of its Affiliates as of the Execution Date or that comes into the Control of AGTC or any of its Affiliates during the Term that claim or disclose any Promoter Know-How. Notwithstanding the foregoing, Schedule 1.23 sets forth the Promoter Patent Rights as of the Execution Date, and will be updated as set forth in Section 1.23.

**1.217.** "**Promoter Technology**" means Promoter Know-How and Promoter Patent Rights.

**1.218.** "**R&D FTE Rate**" means [***], which rate shall be updated for each Calendar Year to a rate as agreed by the Parties, commencing on January 1, 2017, provided that, if the Parties cannot come to agreement with respect to such rate in any given year, such rate shall be updated for such year in accordance with the Consumer Price Index – All Urban Consumers, US City Average, All Items, 1982-84 = 100, published by the United States Department of Labor, Bureau of Labor Statistics (or its successor equivalent index) over the twelve (12) month period preceding January 1 of the applicable Calendar Year.

**1.219.** "**R&D Pre-Funding**" has the meaning set forth in Section 6.1.

**1.220.** "**Receiving Party**" has the meaning set forth in Section 14.1.

**1.221.** "**Regulatory Approval**" means the technical, medical and scientific licenses, registrations, authorizations and approvals (including approvals of BLAs, supplements and amendments, pre- and post- approvals, pricing and third party reimbursement approvals, and labeling approvals) of any Regulatory Authority, necessary for the commercial manufacture, distribution, marketing, promotion, offer for sale, use, import, export or sale of a pharmaceutical product in a regulatory jurisdiction. For the sake of clarity, Regulatory Approval shall not be achieved for a Licensed Product in a country until all applicable Price Approvals and other Third Party reimbursement approvals have also been obtained by Biogen or its designee for such Licensed Product in such country.

**1.222.** "**Regulatory Authority**" means, with respect to a country in the Territory, any national (*e.g.*, the FDA), supra-national (*e.g.*, the European Commission, the Council of the European Union, or the European Medicines Agency), regional, state or local regulatory agency, department, bureau, commission, council or other Governmental Authority involved in the granting of a Regulatory Approval for pharmaceutical products in such country or countries.

**1.223.** "**Research Period**" means, with respect to any Discovery Program, the period (a) starting (i) on the Effective Date, if such Discovery Program is the [***] Discovery Program, the ALD/[***] Discovery Program, the Non-Ophthalmology Discovery Program, if applicable, the [***] or [***] or (ii) on the Discovery Program Substitution Date, if such Discovery Program is a Substitute Discovery Program and (b) ending on the earlier of the date of Clinical Candidate Designation for such Discovery Program or the date that such Discovery Program becomes an Abandoned Program.

**1.224.** "**Residual Knowledge**" means knowledge, techniques, experience and Know-How that are (a) included in any Confidential Information owned or Controlled by the Disclosing Party and (b) retained in the unaided memory of any employee or representative of the Receiving Party as part of a body of knowledge that is not limited to such Confidential Information, after having authorized access to such Confidential Information, provided that such employee or representative has not accessed any written or electronic records or other embodiments of any Confidential Information of the Disclosing Party for use of such Confidential Information outside of this Agreement. A person's memory will be considered to be unaided if the person (i) has not made any effort to memorize or assist the recollection of the Confidential Information for the purpose of retaining and subsequently using or disclosing it, (ii) is not relying on the external records, documents or embodiments of the Disclosing Party's Confidential Information, or notes taken on the foregoing and (iii) is not knowingly disclosing what such person knows to be the Confidential Information of the Disclosing Party. In no event, however, will Residual Knowledge include any knowledge, techniques, experience and Know-How to the extent (at any time, for such time) within the scope of any Patent Right owned or Controlled by the Disclosing Party.

**1.225.** "**ROW Territory**" means all countries in the Territory other than the United States.

**1.226.** "**Royalty Term**" means with respect to any particular Licensed Product in any particular country in the Territory, the period of time beginning on the First Commercial Sale of such Licensed Product in such country and extending until the latest of (a) the expiration of the last to expire of any Valid Claim included in any AGTC Patent Right or Joint Patent Right in such country which Valid Claim Covers the manufacture, use, sale, offer for sale or importation of such Licensed Product in such country; (b) [***].

**1.227.** "[***]" has the meaning set forth in Section [***].

**1.228.** "**RS-1**" has the meaning set forth in Section 1.263.

**1.229.** "**Sales Costs**" has the meaning set forth in Exhibit C.

**1.230.** "**Sales Milestone Payment**" has the meaning set forth in Section 6.4.2.

**1.231.** "**Sales Returns and Allowances**" has the meaning set forth in Section 1.184.

**1.232.** "**Schedule Revision Date**" means the earlier of (a) the fifth (5th) day following the HSR Clearance Date and (b) the day on or after the HSR Clearance Date on which AGTC provides to Biogen either (i) AGTC's supplemental or additional schedules (if any) pursuant to the proviso in the first sentence of Section 15.2, the agreed-upon updated schedules of AGTC Patent Rights, the AGTC Platform and the Product-Specific Patent Rights of AGTC, if any, and a notice that no further supplemental, additional or updated schedules will be provided or (ii) instead of providing any such supplemental, additional or updated schedules, a notice that no further supplemental, additional or updated schedules will be provided.

**1.233.** "**Specification**" means a list of tests, references to analytical procedures, and appropriate acceptance criteria which are numerical limits, ranges, or other criteria for the tests described, which establishes the set of criteria to which a drug substance, drug product, or materials at other stages of its Manufacture or with respect to other drug substances, drug products or materials should conform to be considered acceptable for its intended use.

**1.234.** "[***] Discovery Program" means a program of Pre-Funded Discovery Activities through Clinical Candidate Designation with respect to AAV Products that deliver [***] for the diagnosis, treatment or prevention of [***] disease, conducted by either or both Parties in accordance with the [***] Discovery Program Development Plan and the terms of this Agreement.

**1.235**. "[***] Discovery Program Development Plan" means the written plan for Pre-Funded Discovery Activities for the [***] Discovery Program to be conducted pursuant to this Agreement, as such written plan may be amended, modified or updated by the JDC in accordance with the terms of this Agreement. The initial [***] Discovery Program Development Plan is attached hereto as Exhibit A-3.

**1.236.** "[***]" has the meaning set forth in Section [***].

**1.237.** "**Subcontractor**" has the meaning set forth in Section 3.4.

**1.238.** "**Sublicensee**" means (i) with respect to Biogen or its Affiliate, a Third Party, other than a Distributor, to whom Biogen or its Affiliate has, directly or through multiple tiers, granted a right under the AGTC Technology or the Joint Technology to make, use, develop, sell, offer for sale or import a Licensed Product in a country or otherwise exercise its rights or perform its obligations under this Agreement or any Development Plan, and (ii) with respect to AGTC or its Affiliate, a Third Party, other than a Distributor, to whom AGTC or its Affiliate has, directly or through multiple tiers, granted a right under the Biogen Technology or the Joint Technology to exercise its rights or perform its obligations under this Agreement or any Development Plan.

**1.239.** "**Substitute Discovery Program**" has the meaning set forth in Section 4.4.1.

**1.240.** "**Substitution Notice**" has the meaning set forth in Section 4.4.2.

**1.241.** "**Sued Party**" has the meaning set forth in Section 13.6.3.

**1.242.** "**Tax Authority**" has the meaning set forth in Section 6.8.1.

**1.243.** "**Technology**" means Know-How and Patent Rights.

**1.244.** "**Term**" has the meaning set forth in Section 16.4.

**1.245.** "**Terminated Discovery Products**" has the meaning set forth in Section 4.4.2.

**1.246.** "**Terminated Discovery Program**" has the meaning set forth in Section 4.4.2.

**1.247.** "**Territory**" means all countries of the world.

**1.248.** "**Third Party**" means any Person other than Biogen, AGTC or their respective Affiliates.

**1.249.** "**Third Party IP Rights**" has the meaning set forth in Section 13.6.2(a).

**1.250.** "**Third Party License**" has the meaning set forth in Section 6.6.1(a).

**1.251.** "**Third Party Payments**" has the meaning set forth in Exhibit C.

**1.252.** "**UAB**" means The UAB Research Foundation.

**1.253.** "**UAB Agreement**" means the Non-Exclusive License Agreement with Sublicensing Terms, dated January 19, 2006, as amended March 28, 2014 and June 29, 2015, as may be further amended from time to time, by and between AGTC and UAB.

**1.254.** "**UF/JHU Agreement**" means the Standard Exclusive License Agreement With Sublicensing Terms (also known as Agreement A3288), dated October 7, 2003, as amended November 2004, February 25, 2009, March 30, 2010, December 17, 2013 and July 1, 2015, as may be further amended from time to time, by and among AGTC, UFRF and JHU.

**1.255.** "**UFRF**" means University of Florida Research Foundation, Inc.

**1.256.** "**Valid Claim**" means a claim of (a) an issued and unexpired patent, which claim has not been revoked or held unenforceable, unpatentable or invalid by a decision of a court or other governmental agency of competent jurisdiction, which is not appealable or has not been appealed within the time allowed for appeal, and which has not been abandoned, disclaimed, denied or admitted to be invalid or unenforceable through reissue, re-examination or disclaimer or otherwise, or (b) a patent application for a patent that has been pending less than [***] from the earliest date on which such patent application claims priority and which claim has not been cancelled, withdrawn or abandoned or finally rejected by an administrative agency action from which no appeal can be taken. If a claim of a patent application that ceased to be a Valid Claim due to the passage of time later issues, then it will again be a Valid Claim effective as of the issuance of such patent.

**1.257.** "**XLRP**" means X-linked retinitis pigmentosa.

**1.258.** "**XLRP Development Plan**" means the written plan for Development activities for the XLRP Product to be conducted pursuant to this Agreement, as such written plan may be amended, modified or updated by the Joint Development Committee in accordance with the terms of this Agreement. The initial XLRP Development Plan is attached hereto as Exhibit A-2.

**1.259.** "**XLRP Product**" means any Gene Therapy Product (a) that delivers [***] and (b) with respect to which, absent the license granted to Biogen in Section 5.1, the, Development, Manufacture, Commercialization or use by Biogen as contemplated under this Agreement would infringe a Valid Claim of the AGTC Patent Rights or the Joint Patent Rights or misappropriate AGTC Know-How or Joint Know-How. For clarity, "XLRP Product" includes any Gene Therapy Product developed under the XLRP Program as a back-up product or other additional pre-clinical product that otherwise meets the definition of a "XLRP Product".

**1.260.** "**XLRP Program**" means the Development activities with respect to the XLRP Product under the XLRP Development Plan, in accordance with the terms of this Agreement.

**1.261.** "**XLRS**" means X-linked juvenile retinoschisis.

**1.262.** "**XLRS Development Plan**" means the written plan for Development activities for the XLRS Product to be conducted pursuant to this Agreement, as such written plan may be amended, modified or updated by the Joint Development Committee in accordance with the terms of this Agreement. The initial XLRS Development Plan is attached hereto as Exhibit A-1.

**1.263.** "**XLRS Product**" means any Gene Therapy Product (a) that delivers a retinoschisin-1 ("**RS-1**") transgene (or any functional allelic, codon optimized or other variant, fragment, derivative or modification thereof, in any form) and (b) with respect to which, absent the license granted to Biogen in Section 5.1, the, Development, Manufacture, Commercialization or use by Biogen as contemplated under this Agreement would infringe a Valid Claim of the AGTC Patent Rights or the Joint Patent Rights or misappropriate AGTC Know-How or Joint Know-How. For clarity, "XLRS Product" includes any Gene Therapy Product developed under the XLRS Program as a back-up product or other additional pre-clinical product that otherwise meets the definition of a "XLRS Product".

**1.264.** "**XLRS Program**" means the Development activities with respect to the XLRS Product under the XLRS Development Plan, in accordance with the terms of this Agreement.

**2. GOVERNANCE**.

**2.1. Joint Development Committee**.

2.1.1. Composition. As soon as practicable, but no later than thirty (30) days following the Effective Date, the Parties shall form a joint development committee (the "**Joint Development Committee**" or the "**JDC**"). The JDC shall be comprised of an equal number of representatives from each Party. If mutually agreed by the Parties on a case-by-case basis, the JDC may invite other non-members to participate in the discussions and meetings of the JDC, provided that the presence of such participants shall not be considered in determining whether there is a quorum at the JDC. Each Party shall notify the other Party in writing of its initial representatives to the JDC, and may substitute one or more representatives from time to time upon written notice to the other Party. A designated representative of Biogen will be the chairman of the JDC until the end of the first full Calendar Year following the Effective Date, and thereafter the chairman will be selected alternately, on an annual basis, by AGTC or by Biogen. The chairman shall be responsible for setting the agenda for meetings of the JDC, with input from the other members, and for conducting the meetings of the JDC.

2.1.2. Responsibilities.

(a) *General Responsibilities*. The JDC shall be responsible for oversight of (i) Pre-Funded Activities and Post-Funding Development Activities with respect to each Initial Licensed Program, including the Development Plans and corresponding Development Budgets and timelines thereunder, each Party's Development activities, including pre-clinical work and IND-enabling studies for each Initial Licensed Product and (ii) Pre-Funded Discovery Activities with respect to each Discovery Program, including the Discovery Program Development Plans and corresponding Development Budgets and timelines thereunder.

(b) *Decision-Making Responsibilities*. In addition to the foregoing general responsibilities and any other matters specified in this Agreement for resolution by the JDC, the JDC shall in particular have the following decision-making responsibilities:

(i) discuss, prepare and approve any Development Plan or Development Budget or any amendment or modification to a Development Plan or Development Budget or timelines or activities thereunder, [***], which amendments or modifications the JDC shall be required to formally document on an annual basis as part of the minutes of the meetings of the JDC,

(ii) discuss, prepare and approve the initial Development Plan and Development Budget for the [***], the Non-Ophthalmology Discovery Program, if applicable, and any Substitute Discovery Program,

(iii) designate the [***] Discovery Program within six (6) months of the Effective Date,

(iv) if, under Section 3.2, the Parties share Development Costs for any Initial Licensed Product that is not a Cost Share Product, develop and approve a procedure for sharing of such Development Costs consistent with the procedures set forth in Exhibit C,

(v) oversee and resolve the financial, budgetary and accounting issues which may arise in connection with any Development Plan and the corresponding Development Budget,

(vi) determine if (a) any AGTC Technology conceived, discovered, invented, created, made or reduced to practice or tangible medium outside of a Collaboration Program or (b) any Technology Controlled by Biogen that Biogen desires to use in a Collaboration Program, in each case ((a) and (b)), that comes into the Control of the applicable Party after the Effective Date, is to be used in a Collaboration Program, if such use would require additional Development activities or change the anticipated timing of any Pre-Funded Activities, Pre-Funded Discovery Activities or Post-Funding Development Activities under any Development Plan, subject to the provisions of Section 13.6.2(a) with respect to the use of any Technology that comes into the Control of AGTC or its Affiliates during the Term through a license of Third Party IP Rights, and

(vii) approve the matters contemplated by Exhibit C.

(c) *Oversight*. In addition to the foregoing decision-making responsibilities, the JDC shall have the following oversight responsibilities:

(i) oversee and review the activities under each Development Plan or Development Budgets, including but not limited to timelines, thereunder,

(ii) manage the overall strategy for Development activities with respect to each Collaboration Program under the Development Plans,

(iii) monitor the spending of the Parties under each Development Plan and corresponding Development Budget,

(iv) oversee Manufacturing activities with respect to clinical supplies of Licensed Product, including quality assurance and the selection of, and technology transfer to, any Third Party contract organizations assisting with such Manufacturing activities in accordance with this Agreement,

(v) manage the technology transfer of AGTC Know-How and data from AGTC to Biogen as described in Section 11.1, Section 11.2 and Section 11.3 or in any Development Plan or that is otherwise reasonably requested by Biogen and otherwise necessary or useful for Biogen to perform its obligations or exercise its rights under this Agreement with respect to a Licensed Program, and

(vi) perform such other functions as may be appropriate to further the purposes of this Agreement, in each case with respect to Development activities for the Collaboration Programs, as mutually agreed in writing by the Parties.

The JDC, in its discretion, may establish subcommittees or working groups to assist the JDC in carrying out the responsibilities of the JDC. For clarity, the JDC shall have no oversight authority over activities related to the Commercialization of any Licensed Product or, except as expressly set forth above, the Manufacture and supply of any Licensed Product required for Commercialization.

(d) *General*. The JDC shall conduct its responsibilities hereunder in good faith and with reasonable care and diligence.

2.1.3. Meetings.

(a) Subject to this Section 2.1.3(a), the JDC shall meet in person or by teleconference once per Calendar Quarter (or more often, as mutually agreed by the Parties) on such dates and at such times and places as agreed to by the members of the JDC, provided that the JDC shall meet promptly following FIH Trial Completion with respect to any Initial Licensed Product. Meetings of the JDC shall be alternately hosted by the Parties, with the host determining whether the meeting will be in person or by teleconference, provided that at least one meeting hosted by each Party in each Calendar Year shall be in person and the first in-person meeting shall be hosted by Biogen no later than sixty (60) days from the Effective Date. Each Party shall be responsible for its own expenses relating to attendance at or participation in JDC meetings.

(b) Within ten (10) Business Days following each JDC meeting, the Party hosting the meeting shall cause to be prepared and shall provide to the other Party a draft of reasonably detailed written minutes describing all matters reviewed or considered by the JDC and all determinations made and actions taken by the JDC and a summary of the reasons therefor stated by the members at the meeting. The minutes of any meeting of the JDC must be finalized by approval of the members of the JDC within fifteen (15) Business Days of the meeting. The final minutes shall include the relevant executed amendments to the Development Plans reflecting the discussed and approved changes. The minutes and the drafts of any minutes shall be the Confidential Information of both Parties.

(c) Each Party shall submit to the JDC at least five (5) Business Days prior to any meeting of the JDC all reports required to be submitted by such Party to the JDC at such meeting under this Agreement.

2.1.4. Decision Making. Each Party shall be entitled to cast one vote on matters before the JDC. For the transaction of business, a quorum consisting of not less than one representative of each Party must be present at a meeting, and each Party shall cause at least one representative of such Party to be present at each such meeting. Decisions of the JDC shall be made by unanimous approval, provided that a quorum must be present for any decision to be made by the JDC. If the JDC is unable to reach agreement with respect to any decision within the scope of its authority, such dispute shall be escalated to the Alliance Managers for resolution. If the Alliance Managers are unable to reach agreement with respect to such decision within thirty (30) days of such escalation, such dispute shall be escalated to the Chief Executive Officer of each Party (or his/her nominee), and such Chief Executive Officers (or their nominees, as applicable) will meet promptly to attempt to resolve the dispute by good faith negotiations. If these individuals are unable to resolve the dispute within thirty (30) days of the request for such meeting, the matter shall be decided [***]:

(a) *Pre-Funded Activities for the Initial Licensed Programs*. [***] shall have the final decision-making authority for all matters related to the conduct of Pre-Funded Activities under any Development Plan for an Initial Licensed Program, except that:

(i) if the matter relates to the use of any [***] Technology conceived, discovered, invented, created, made or reduced to practice or tangible medium outside of a Collaboration Program or any Technology Controlled by [***], in each case, in an Initial Licensed Program where such use would require additional Development activities or change the anticipated timing of any Pre-Funded Activities under any Development Plan as described in Section 2.1.2(b)(vi), then Biogen shall have the final decision-making authority if [***] determines, in its sole discretion, that (A) with respect to any Initial Licensed Product, such [***] Technology or such Technology Controlled by [***], as applicable, is necessary in order to Develop, Manufacture, Commercialize or use such Initial Licensed Product or (B) with respect to the XLRP Product, such [***] Technology or such Technology Controlled by [***], as applicable, is useful in order to Develop, Manufacture, Commercialize or use the XLRP Product;

(ii) if the matter is not described in clause (i) above and relates to the addition of new Pre-Funded Activities to, or a change in the scope of, existing Pre-Funded Activities under any Development Plan that would cause a change in the applicable Development Budget, then the matter may only be decided by [***]; provided, however, that, if any Regulatory Authority recommends or suggests a change to the FIH Trial for the XLRS Product (other than the matters described below in Section 2.1.4(a)(iii)) in order to complete such FIH Trial or continue the Development of the XLRS Product, and the Parties disagree on whether to implement such change, [***] shall have the final decision-making authority, subject to Section 3.2.2(a)(i);

(iii) with respect to a decision to conduct (A) a toxicology study with respect to the XLRS Product at a higher dosage than prior studies or (B) an additional arm in the FIH Trial for the XLRS Product, [***] shall have the final decision-making authority, subject to Section 3.2.2(a)(i);

(iv) if Biogen exercises its step-in rights under Section 3.1.3 with respect to an Initial Licensed Program, then [***] shall have the final decision-making authority for all matters related to the conduct of Pre-Funded Activities for such Initial Licensed Program in accordance with the Development Plan; and

(v) notwithstanding anything to the contrary in this Agreement, if the matter relates to the Manufacture of an Initial Licensed Product, then [***] shall have the final decision-making authority, provided that, if such matter would set a regulatory precedent for Specifications for the Manufacture of AAV Products during the period of time that Regulatory Approval for [***] has not been obtained by either Party or their respective Affiliates or sublicensees or [***] from the first Regulatory Approval achieved for such AAV Products, if earlier (the " **Manufacturing Precedent Period**"), then such matter may only be decided by [***]. For clarity, in the event of any requirement by a Regulatory Authority with respect to the Manufacture of an Initial Licensed Product, the Parties shall comply with such requirement and neither Party shall have final decision-making authority.

(b) *Post-Funding Development Activities for the Initial Licensed Programs*. Biogen shall have the final decision-making authority for all matters related to the conduct of the Post-Funding Development Activities under any Development Plan for an Initial Licensed Program, except that:

(i) if the matter relates to Post-Funding Development Activities for an Initial Licensed Program for which AGTC has exercised the Cost Share Option, then the matter (along with the Budget for such matter) may only be decided by [***]; and

(ii) notwithstanding anything to the contrary in this Agreement, if the matter relates to the Manufacture of an Initial Licensed Product, then [***] shall have the final decision-making authority, provided, however, that, if such matter would set a regulatory precedent for Specifications for the Manufacture of AAV Products during the Manufacturing Precedent Period, then such matter may only be decided by [***]. For clarity, in the event of any requirement by a Regulatory Authority with respect to the Manufacture of an Initial Licensed Product, the Parties shall comply with such requirement and [***] shall have final decision-making authority.

(c) *Pre-Funded Discovery Activities*. [***] shall have the final decision-making authority for any matter that relates to a Discovery Program.

Any decision made in exercising a Party's final decision-making authority must be consistent with the terms of this Agreement and within the scope of authority delegated to the JDC under this Agreement, and any Development Costs associated with the decisions set forth in this Section 2.1.4 shall be treated in accordance with Section 3.2 or Section 4.6, as applicable.

2.1.5. Discontinuation of the JDC. The JDC's authority as set forth in this Section 2.1 with respect to a Collaboration Program shall continue to exist until the first to occur of (a) the Parties mutually agreeing to terminate the JDC's authority with respect to such Collaboration Program and (b) (i) with respect to a Discovery Program, the earlier of (w) the Option Exercise Date, (x) the end of the Option Exercise Period for such Discovery Program, (y) the date a Discovery Program becomes a Terminated Discovery Program and (z) the date a Discovery Program becomes an Abandoned Program, (ii) with respect to an Initial Licensed Program for which AGTC has elected the Milestone/Royalty Option pursuant to Section 6.2.2, the date that AGTC is no longer conducting any substantial level of Development activities with respect to such Initial Licensed Program or (iii) with respect to an Initial Licensed Program for which AGTC has elected the Cost Share Option pursuant to Section 6.2.2, the later of (x) completion of all Post-Funding Development Activities for such Initial Licensed Program and (y) formation of the JCC; provided that, in all events, the JDC shall cease to have oversight over activities with respect to a given Initial Licensed Product when the First Commercial Sale of such Initial Licensed Product has occurred. The JDC shall disband when it ceases to have authority over any Collaboration Program pursuant to the

preceding sentence. Notwithstanding anything herein to the contrary, once the JDC ceases to exist, the JDC shall have no further responsibilities under this Agreement and Biogen shall have the right to solely decide, without consultation, any matters previously within the authority of the JDC; provided, however, that any decision requiring AGTC to perform any additional development activities will be decided by mutual agreement of the Parties and any associated Development Costs shall be treated in accordance with Section 3.2 or Section 4.6, as applicable.

**2.2. Joint Commercialization Committee**.

2.2.1. Composition. No later than [***] prior to the anticipated First Commercial Sale of any Cost Share Product and in any event no later than the first filing of a Marketing Application for a BLA for such Cost Share Product, the Parties shall form a joint commercialization committee (the "**Joint Commercialization Committee**" or the "**JCC**"). The JCC shall be comprised of an equal number of representatives from each Party. If mutually agreed by the Parties on a case-by-case basis, the JCC may invite other non-members to participate in the discussions and meetings of the JCC, provided that the presence of such participants shall not be considered in determining whether there is a quorum at the JCC. Each Party shall notify the other Party in writing of its initial representatives to the JCC, and may substitute one or more representatives from time to time upon written notice to the other Party. A designated representative of Biogen will be the chairman of the JCC, and Biogen may change this representative from time to time upon written notice to AGTC. The chairman shall be responsible for setting the agenda for meetings of the JCC, with input from the other members, and for conducting the meetings of the JCC.

2.2.2. Responsibilities.

(a) *General Responsibilities*. The JCC shall be responsible for oversight of Commercialization activities with respect to the Cost Share Products.

(b) *Decision-Making Responsibilities*. In addition to the foregoing general responsibilities and any other matters specified in this Agreement for resolution by the JCC, the JCC shall in particular have the following decision-making responsibilities with respect to the Cost Share Products:

(i) discuss and approve any Commercialization Plan or Commercialization Budget or any amendment or modification to a Commercialization Plan or Commercialization Budget, in each case, for any Cost Share Product, which amendments or modifications the JCC shall be required to formally document on an annual basis as part of the minutes of the meetings of the JCC,

(ii) determine the Commercial FTE Rate, which shall be consistent with Biogen's internal FTE rates for similar activities, and

(iii) approve the matters contemplated by Exhibit C.

(c) *Oversight*. In addition to the foregoing decision-making responsibilities, the JCC shall have the following oversight responsibilities with respect to the Cost Share Products:

(i) oversee and review the activities under the Commercialization Plan for each Cost Share Product,

(ii) address the financial, budgetary and accounting issues which may arise in connection with any Commercialization Plans and the corresponding Budget, and

(iii) perform such other functions as appropriate to further the purposes of this Agreement with respect to Commercialization of the Cost Share Products, as mutually agreed in writing by the Parties.

The JCC, in its discretion, may establish subcommittees to assist the JCC in carrying out the responsibilities of the JCC.

(d) *General*. The JCC shall conduct its responsibilities hereunder in good faith and with reasonable care and diligence.

2.2.3. Meetings.

(a) Subject to this Section 2.2.3(a), after its formation in accordance with Section 2.2.1, the JCC shall meet in person or by teleconference once per Calendar Quarter (or more often, as mutually agreed by the Parties) on such dates and at such times and places as agreed to by the members of the JCC. Meetings of the JCC shall be alternately hosted by the Parties, with the host determining whether the meeting will be in person or by teleconference, provided that at least one meeting hosted by each Party in each Calendar Year shall be in person and the first in-person meeting of the JCC shall be hosted by Biogen no later than sixty (60) days after the date that the JCC is formed in accordance with Section 2.2.1. Each Party shall be responsible for its own expenses relating to attendance at or participation in JCC meetings.

(b) Within ten (10) Business Days following each JCC meeting, the Party hosting the meeting shall cause to be prepared and shall provide to the other Party a draft of reasonably detailed written minutes describing all matters reviewed or considered by the JCC and all determinations made and actions taken by the JCC and a summary of the reasons therefor stated by the members at the meeting. The minutes of any meeting of the JCC must be finalized by approval of the members of the JCC within fifteen (15) Business Days of the meeting. The minutes and the drafts of any minutes shall be the Confidential Information of both Parties.

(c) Each Party shall submit to the JCC at least five (5) Business Days prior to any meeting of the JCC all reports required to be submitted by such Party to the JCC at such meeting under this Agreement.

2.2.4. Decision Making. Each Party shall be entitled to cast one vote on matters before the JCC. For the transaction of business, a quorum consisting of not less than one representative of each Party must be present at a meeting, and each Party shall cause at least one representative of such Party to be present at each such meeting. Decisions of the JCC shall be made by unanimous approval, provided that a quorum must be present for any decision to be made by the JCC. If the JCC is unable to reach agreement with respect to any decision within the scope of its authority, such dispute shall be escalated to the Alliance Managers for resolution. If the Alliance Managers are unable to reach agreement with respect to such decision within thirty (30) days of such escalation, such dispute shall be escalated to the Chief Executive Officer of each Party (or his/her nominee), and such Chief Executive Officers (or their nominees, as applicable) will meet promptly to attempt to resolve the dispute by good faith negotiations. If these individuals are unable to resolve the dispute within thirty (30) days of the request for such meeting, Biogen shall have the final decision-making authority; provided, however, that, if the matter relates to the Manufacture of a Cost Share Product and would set a regulatory precedent for Specifications for the Manufacture of AAV Products during the Manufacturing Precedent Period, then such matter may only be decided by [***]. For clarity, in the event of any requirement by a Regulatory Authority with respect to the Manufacture of a Cost Share Product, the Parties shall comply with such requirement and [***] shall have final decision-making authority. Any decision made by either Party in exercising its final decision-making authority must be consistent with the terms of this Agreement.

2.2.5. Discontinuation of the JCC. The JCC shall continue to exist until the first to occur of (a) the Parties mutually agreeing to disband the JCC and (b) the date that Biogen (or its Affiliate or Sublicensee) is no longer Commercializing any Cost Share Product.

**2.3. Alliance Managers**.

2.3.1. Appointment. Within thirty (30) days following the Effective Date each Party will appoint (and notify the other Party of the identity of) a senior representative of such Party having a general understanding of pharmaceutical Development and Commercialization issues to act as its alliance manager under this Agreement (each an "**Alliance Manager**"). Each Party may replace its Alliance Manager at any time by written notice to the other Party.

2.3.2. Specific Responsibilities. The Alliance Managers may be, but shall not be required to be, members of the JDC or the JCC. The Alliance Managers will serve as the primary contact point between the Parties for the Collaboration Programs for the purpose of providing each Party with information on the progress of Development and Commercialization of each Discovery Program and each Licensed Product and shall have the following responsibilities:

(a) facilitating the flow of information and otherwise promoting communication, coordination and collaboration between the Parties;

(b) coordinating the various functional representatives of each Party, as appropriate, in developing and executing strategies and plans for the applicable Discovery Program or the applicable Licensed Product;

(c) providing a single point of communication for seeking consensus both internally within the respective Party's organization and between the Parties regarding key strategy and planning issues;

(d) assisting the integration of teams across functional areas;

(e) assisting the JDC and, if applicable, the JCC in identifying and raising cross-Party and/or cross-functional disputes in a timely manner; and

(f) performing such other functions as requested by the JDC or, if applicable, the JCC.

**2.4. Other Committees**. The Parties may, by mutual agreement, form such other committees or working groups as may be necessary or desirable to facilitate the activities under each Collaboration Program, including the Development and Commercialization of the Licensed Products.

**2.5. General Authority**. Each of the JDC, the JCC and the Alliance Managers shall have solely the powers expressly assigned to them in this Article 2 and elsewhere in this Agreement. None of the JDC, the JCC, any other committee or working group or any Alliance Manager shall have any power to amend, modify or waive compliance with or determine the other Party's compliance with or breach of this Agreement. In conducting themselves on the JDC, the JCC or any other committees or working groups and as Alliance Managers, and in exercising their rights under this Article 2, all representatives of both Parties shall consider diligently, reasonably and in good faith all input received from the other Party, and shall use reasonable efforts to reach unanimity, where required, on all matters before them.

**3. INITIAL LICENSED PROGRAMS.**

**3.1. Control of Development**. The Parties will conduct all Development activities for each Initial Licensed Program in accordance with the applicable Development Plan for such Initial Licensed Program and this Section 3.1. The initial Development Plans for each Initial Licensed Program are attached hereto as Exhibit A-1 and Exhibit A-2, respectively. There shall be no Development Plan (or corresponding Development Budget) for any Initial Licensed Program for which (a) AGTC is no longer conducting any Development activities and (b) AGTC has not exercised the Cost Share Option. At such time as there is no Development Plan for an Initial Licensed Program, (i) Biogen may conduct Development activities for such Initial Licensed Program in its sole discretion, subject to Section 3.3.1 and (ii) Biogen shall comply with the ongoing reporting obligations set forth in Section 10.1.2.

3.1.1. Development of the XLRS Product. Subject to Section 3.1.3, AGTC will have primary responsibility for Development of the XLRS Product until Regulatory Approval in the United States in accordance with the XLRS Development Plan. AGTC shall promptly notify Biogen when the XLRS Product receives Regulatory Approval in the United States. Thereafter, Biogen will have sole responsibility for the Development of the XLRS Product.

3.1.2. Development of the XLRP Product. Subject to Section 3.1.3, AGTC will have primary responsibility for Development of the XLRP Product until FIH Trial Completion for the XLRP Product in accordance with the XLRP Development Plan. AGTC shall promptly notify Biogen when the XLRP Product achieves FIH Trial Completion. Within thirty (30) days of FIH Trial Completion, AGTC shall provide to Biogen all Program Data from the XLRP Program that is not already in Biogen's possession, and, within thirty (30) days of Biogen's receipt of such Program Data, Biogen shall

notify AGTC as to whether it elects to have primary responsibility for the Development of the XLRP Product. In the event that (a) Biogen elects not to have primary responsibility for Development of the XLRP Product, or (b) Biogen does not notify AGTC of its election within such thirty (30) day period, then AGTC shall continue to use Commercially Reasonable Efforts to Develop the XLRP Product in accordance with the XLRP Development Plan until the XLRP Product receives Regulatory Approval in the United States, after which Biogen will have primary responsibility for the Development of the XLRP Product, provided that, if Biogen notifies AGTC prior to the receipt of a first Regulatory Approval in the United States for the XLRP Product that Biogen desires to take over Development activities for the XLRP Product, AGTC shall transfer such Development activities to Biogen in a manner and on a timeline reasonably determined by Biogen as sufficient to allow for an orderly transition of such activities.

3.1.3. Biogen Step-In Rights. Biogen shall have the right, but not the obligation, to take over (a) all of AGTC's unfinished Development activities under any Development Plan for an Initial Licensed Product with [***] written notice to AGTC upon occurrence of any of the events listed on Schedule 3.1.3. In the event that Biogen properly exercises its right to take over any of AGTC's Development activities under any Development Plan pursuant to this Section 3.1.3, AGTC shall have no further obligation to conduct such Development activities; provided, however, that AGTC shall, at Biogen's request, be obligated to continue conducting any ongoing Clinical Trial under such Development Plan through the completion of such Clinical Trial. AGTC shall transfer any such Development activities to Biogen in a manner and on a timeline to reasonably allow for an orderly transition of such activities. Within forty-five (45) days of the end of any Calendar Quarter in which Biogen has incurred Development Costs in the course of performing Development activities in accordance with any Development Plan under this Section 3.1.3, solely to the extent such Development activities are Pre-Funded Activities or the Parties are otherwise sharing the Development Costs for such Development activities under Section 3.2.2(a)(iii), Biogen shall provide to AGTC a reasonably detailed invoice of all or such portion of such Development Costs (which shall include a determination of Biogen's internal costs) that is the responsibility of AGTC pursuant to Section 3.2, and AGTC shall make non-creditable, non-refundable quarterly payments in accordance with the applicable Development Budget to reimburse Biogen for any undisputed Development Costs payable by AGTC for such Development activities within forty-five (45) days of receipt of such invoice from Biogen. In the event that Biogen exercises its step-in rights with respect to an Initial Licensed Program under this Section 3.1.3 and the JDC determines that it is necessary to conduct any activities not set forth in the Development Plan for such Initial Licensed Program in order to complete the Pre-Funded Activities set forth in such Development Plan (*e.g.*, repeating a study or performing back-up work on the applicable Initial Licensed Product), but in any event, excluding an Additional Clinical Trial (such activities, the "**Additional Biogen Activities**"), then the Parties shall share the Development Costs associated with the Additional Biogen Activities equally in accordance with Section 3.2.2(a)(iii) and thereafter the division of decision-making authority set forth in Section 2.1.4(a)(iv) shall apply, as applicable, with respect to any decisions regarding Development activities for such Initial Licensed Program.

**3.2. Development Costs**. The initial Development Budget for each Initial Licensed Program is set forth in the initial Development Plans attached hereto as Exhibit A-1 and Exhibit A-2, respectively. Any Development Budget may be amended or modified only by the JDC in accordance with the terms of this Agreement.

3.2.1. General. Subject to the provisions of Section 3.2.2(a), AGTC shall be solely responsible for the payment of all Development Costs associated with the Pre-Funded Activities conducted by either Party for each Initial Licensed Program in accordance with the applicable Development Plan. It is the intention of the Parties that the applicable portion of the R&D Pre-Funding paid by Biogen to AGTC in accordance with Section 6.1 will cover all Development Costs associated with the Pre-Funded Activities as set forth in the applicable initial Development Budget. Subject to the provisions of Section 3.2.2(b), Section 3.2.2(c) and Section 3.2.2(d), if AGTC has exercised the Milestone/Royalty Option with respect to an Initial Licensed Program, Biogen shall thereafter be solely responsible for all Development Costs for such Initial Licensed Program. Within three (3) Business Days after the end of any Calendar Quarter in which AGTC has incurred Development Costs in the course of performing Pre-Funded Activities or Post-Funding Development Activities in accordance with any Development Plan, AGTC shall provide to Biogen (a) a statement of actual Development Costs incurred for the first two (2) months of such Calendar Quarter (which shall include a determination of AGTC's internal costs based on the R&D FTE Rate) and (b) a reasonable estimate of Development Costs incurred in the third month of such Calendar Quarter (which shall include an estimate of AGTC's internal costs based on the R&D FTE Rate). Within thirty (30) days of the end of any such Calendar Quarter, AGTC shall provide to Biogen a final report of such Development Costs and, with respect to Development Costs incurred in the course of performing Post-Funding Development Activities, a reasonably detailed invoice of such Development Costs (which shall include a determination of AGTC's internal costs based on the R&D FTE Rate), and Biogen

shall make non-creditable, non-refundable quarterly payments in accordance with the applicable Development Budget to reimburse AGTC for any undisputed Development Costs incurred in the course of performing Post-Funding Development Activities within forty-five (45) days of receipt such invoice from AGTC, provided that, if AGTC has exercised the Cost Share Option with respect to any Initial Licensed Product, AGTC and Biogen shall share responsibility for such Development Costs with respect to the Licensed Program for such Initial Licensed Product in accordance with Section 6.3.

3.2.2. Budget Overages.

(a) *Pre-Funded Activities*. For either of the Initial Licensed Programs, if the Development Costs incurred in conducting any Pre-Funded Activity (including, for the avoidance of doubt, reasonable costs incurred by Biogen in conducting Pre-Funded Activities after exercising its step-in rights as set forth in Section 3.1.3) exceed the budgeted amount for such Pre-Funded Activity as set forth in the applicable Development Budget, then AGTC shall be solely responsible for all such Development Costs in excess of such budgeted amount, provided that, to the extent such excess Development Costs are caused by the recklessness, negligence or intentional misconduct of Biogen or its Affiliates or their respective Sublicensees or Subcontractors, then Biogen shall be solely responsible for such Development Costs. Notwithstanding the foregoing, except to the extent such excess Development Costs are caused by the recklessness, negligence or intentional misconduct of Biogen or its Affiliates or their respective Sublicensees or Subcontractors, in which case Biogen shall be solely responsible for such Development Costs, the following rules shall apply:

(i) If, prior to the completion of the Pre-Funded Activities for an Initial Licensed Program, the JDC amends the Development Plan for such Initial Licensed Program to include additional Development activities or change the scope of any existing Pre-Funded Activities or if a Regulatory Authority requires such additional Development activities or change in scope, and such amendment requires an increase in Development Costs as set forth in the corresponding amended Development Budget, then AGTC and Biogen shall share such increased Development Costs equally, provided that AGTC shall bear [***] and Biogen shall bear [***] of any Development Costs associated with conducting (A) a toxicology study with respect to the XLRS Product at a higher dosage than prior studies or (B) an additional arm in the FIH Trial for the XLRS Product, in each case if determined by Biogen to be conducted pursuant to Section 2.1.4(a)(iii); and provided, further, that with respect to any other change to the FIH Trial for the XLRS Product, if a Party exercises its final decision-making authority under Section 2.1.4(a)(ii) with respect to such change, such Party shall bear [***] and the other Party shall bear [***] of the Development Costs associated with such change.

(ii) Biogen shall bear [***] of such increased Development Costs to the extent arising directly from the use in the XLRP Program of any AGTC Technology conceived, discovered, invented, created, made or reduced to practice or tangible medium outside of a Collaboration Program or any Technology Controlled by Biogen under Section 2.1.2(b)(vi), solely to the extent that Biogen has exercised its final decision-making authority under clause (B) of Section 2.1.4(a)(i) with respect to such use (unless and to the extent that such increased Development Costs arise directly from the use in an Initial Licensed Program of any Third Party IP Rights within the AGTC Technology or the Technology Controlled by Biogen under Section 2.1.2(b)(vi), where AGTC has violated any of the representations and warranties set forth in Section 15.2.13 or Section 15.2.14 with respect to such Third Party IP Rights, in which case AGTC shall bear [***] of such increased Development Costs).

(iii) If, prior to the completion of the Pre-Funded Activities for an Initial Licensed Program, Biogen exercises its step-in rights under Section 3.1.3 with respect to such Initial Licensed Program and the JDC or a Regulatory Authority determines that it is necessary to conduct any Additional Biogen Activities in order to obtain Regulatory Approval for the applicable Initial Licensed Product, then, solely to the extent such Additional Biogen Activities are not Pre-Funded Activities set forth in the initial Development Plan for such Initial Licensed Program, AGTC and Biogen shall share equally the Development Costs associated with such Additional Biogen Activities. For the avoidance of doubt, the provisions of this Section 3.2.2(a)(iii) shall apply in the case of either (a) AGTC's election of the Milestone/Royalty Option under Section 6.2.2 for such Initial Licensed Program or (b) AGTC's election of the Cost Share Option under Section 6.2.2 for such Initial Licensed Program.

(b) *Post-Funding Development Activities*.

(i) If the Development Costs for Post-Funding Development Activities for the XLRS Program exceed the Development Costs budgeted for Post-Funding Development Activities for the XLRS Program as set forth in the Development Budget for the Initial Licensed Program attached hereto as part of Exhibit A-1 as of the Effective Date, then (i) if AGTC has exercised the Milestone/Royalty Option for the XLRS Program, then, (A) with respect to the Development Costs up to [***] or less of the budgeted amount, Biogen shall bear [***] of such excess Development Costs and (B) with respect to the Development Costs greater than [***] of the budgeted amount, AGTC shall bear [***] and Biogen shall bear [***] of such excess Development Costs, in accordance with the processes developed by the JDC in accordance with Section 2.1.2(b)(iv), and (ii) if AGTC has exercised the Cost Share Option for the XLRS Program, then the Parties shall equally share any Development Costs that exceed the budgeted amount in accordance with Section 6.3.

(ii) If the Development Costs for Post-Funding Development Activities for the XLRP Program prior to the Pivotal Trial exceed the Development Costs budgeted for Post-Funding Development Activities for the XLRP Program as set forth in the Development Budget for the XLRP Program attached hereto as part of Exhibit A-2 as of the Effective Date, then (i) if AGTC has exercised the Milestone/Royalty Option for XLRP Program, then, (A) with respect to the Development Costs up to [***] or less of the budgeted amount, Biogen shall bear [***] of such excess Development Costs and (B) with respect to the Development Costs greater than [***] of the budgeted amount, AGTC shall bear[***] and Biogen shall bear [***] of such excess Development Costs, in accordance with the processes developed by the JDC in accordance with Section 2.1.2(b)(iv), and (ii) if AGTC has exercised the Cost Share Option for XLRP Program, then the Parties shall equally share any Development Costs that exceed the budgeted amount in accordance with Section 6.3. Notwithstanding the foregoing, if the Development Costs for the Pivotal Trial and related or subsequent Post-Funding Development Activities (including any extension studies) for the XLRP Product exceed the Development Costs for such Pivotal Trial and related Post-Funding Development Activities as set forth in the Development Budget for the XLRP Program attached hereto as part of Exhibit A-2 as of the Effective Date, then (x) if AGTC has exercised the Milestone/Royalty Option for the XLRP Program, Biogen shall bear [***] of such excess Development Costs and (y) if AGTC has exercised the Cost Share Option for the XLRP Program, the Parties shall equally share such excess Development Costs in accordance with Section 6.3.

(c) *Additional Clinical Trials*. If, following the completion of the Pre-Funded Activities for an Initial Licensed Program, the JDC or any Regulatory Authority determines that it is necessary, in order to obtain Regulatory Approval of an Initial Licensed Product under such Initial Licensed Program, to re-perform or conduct any additional Clinical Trial prior to a Pivotal Trial with respect to such Initial Licensed Product (each, an "**Additional Clinical Trial**"), then the Development Budget for such Initial Licensed Product shall be modified to include the Development Costs of the Additional Clinical Trial and, if AGTC has exercised the Milestone/Royalty Option with respect to such Initial Licensed Program, AGTC shall share such Development Costs equally with Biogen unless, in such event, AGTC notifies Biogen prior to any such Development Costs being incurred that it elects to not share such Development Costs equally, in which case, AGTC shall not be required to share any such Development Costs with Biogen, and such election shall have the effect described in the final paragraph of Section 6.4.1. For clarity, if AGTC has exercised the Cost Share Option with respect to such Initial Licensed Program, the Parties shall share any Development Costs under this Section 3.2.2(c) in accordance with the provisions of Section 6.3.

(d) *Other Activities*. Notwithstanding anything to the contrary, in the event that AGTC conducts any activities with respect to any Initial Licensed Program that are not in accordance with a Development Plan for such Initial Licensed Program and incurs excess Development Costs in connection therewith, Biogen shall have no obligation to reimburse AGTC for any such excess Development Costs.

3.2.3. Excess R&D Pre-Funding. If any Initial Licensed Program is terminated prior to completion of all Pre-Funded Activities for such Initial Licensed Program, then any R&D Pre-Funding for Pre-Funded Activities under such Initial Licensed Program that have not yet been undertaken shall be allocated toward Development Costs payable by Biogen for other Collaboration Programs. [***]

**3.3. Development Diligence**.

3.3.1. Diligence Obligations.

(a) AGTC will use Commercially Reasonable Efforts to carry out the Development activities allocated to it under Section 3.1 and the Development Plan for each Initial Licensed Program. Without limiting the foregoing, AGTC shall, in particular, use Commercially Reasonable Efforts to (i) conduct the Pre-Funded Activities and the Post-Funding Development Activities assigned to AGTC under the XLRS Development Plan and to Develop the XLRS Product through Regulatory Approval in the United States in accordance with the XLRS Development Plan and (ii) conduct the Pre-Funded Activities and the Post-Funding Development Activities assigned to AGTC under the XLRP Development Plan and to Develop the XLRP Product through FIH Trial Completion in accordance with the XLRP Development Plan.

(b) To the extent that such activities are the responsibility of Biogen in accordance with this Agreement, Biogen will use Commercially Reasonable Efforts to develop and seek Regulatory Approval for at least (1) one XLRS Product and (2) one XLRP Product, in each case, in the Major Market Countries.

3.3.2. Exceptions to Diligence Obligations. Notwithstanding any provision of this Agreement to the contrary, the Party obligated to meet diligence requirements (the "**Obligated Party**") will be relieved from and will have no obligation to undertake any efforts described in Section 3.3.1 with respect to the Development of any Initial Licensed Product in the event that:

(a) either Party receives or generates any safety, tolerability or other data reasonably indicating, as measured by the Obligated Party's safety and efficacy evaluation criteria and methodology, that an Initial Licensed Product is not reasonably suitable, for safety reasons, for initiation or continuation of Clinical Trials in humans; or

(b) the other Party materially breaches any of its Development or other obligations under the Development Plans or this Agreement and such breach impairs or limits the Obligated Party's ability to perform its Development activities under this Agreement; provided that, in such event, the Obligated Party shall only be relieved of such obligations to the extent and for so long as the other Party's breach so impairs or limits the Obligated Party's ability to perform its Development activities under this Agreement.

**3.4. Subcontractors**. Each Party may engage consultants, subcontractors, or other vendors (each, a "**Subcontractor**") to perform any work under the Initial Licensed Programs; provided that, with respect to any Subcontractor of AGTC that has not previously been engaged by AGTC within the two (2) years prior to such proposed engagement, all such engagements by AGTC and any contracts related to such engagements shall be subject to the prior written approval of Biogen, such approval not to be unreasonably withheld; and provided, further, that in the event AGTC elects to engage a Subcontractor to provide any service for which Biogen possesses internal capabilities to perform such service, at a cost equal to or less than such Subcontractor and on substantially similar terms (including capability and timing), AGTC shall notify Biogen and Biogen shall determine whether Biogen, at AGTC's sole expense subject to cost sharing provisions included in Section 3.2.2 and Section 6.3, to the extent set forth in the applicable Development Budget, will provide such service to AGTC in accordance with the applicable Development Plan. Biogen shall reasonably consult with AGTC with respect to the engagement of Subcontractors but shall be permitted to engage Subcontractors without prior approval of AGTC. Each contract between a Party and a Subcontractor shall be consistent with the provisions of this Agreement and shall include provisions, including intellectual property provisions, adequate for the other Party to avail itself of the licenses granted hereunder as though such Party had performed the contracted work, and AGTC shall, unless otherwise agreed to by Biogen, ensure that all contracts entered into between AGTC and Subcontractors to perform Manufacturing activities include a provision allowing AGTC to freely assign such contract to Biogen at no cost to either Party. Each Party shall be responsible for the effective and timely management of and payment of its Subcontractors. The engagement of any Subcontractor in compliance with this Section 3.4 shall not relieve the applicable Party of its obligations under this Agreement or any Development Plan. Each Party shall

be solely responsible for any taxes, including income, withholding, payroll, VAT, sales tax or the like, that arise from the use of a Subcontractor. To the extent AGTC engages any Subcontractor to perform any Manufacturing activities, AGTC shall notify Biogen of proposed substantive interactions with any such Subcontractors material to a Initial Licensed Program and shall use reasonable efforts to allow Biogen to participate in any such interactions primarily related to an Initial Licensed Program if Biogen requests such involvement, at Biogen's cost and expense, provided that such reasonable efforts and such participation do not cause undue delay to any Initial Licensed Program.

**3.5. Conduct**. Each Party shall, and shall require its Affiliates and Subcontractors to, comply with all applicable Laws in their conduct of the Development activities with respect to the Initial Licensed Programs, including where appropriate cGMP, GCP and GLP (or similar standards) for the performance of laboratory activities.

**4. DISCOVERY PROGRAMS**.

**4.1. General**. During the Research Period, each Party will conduct research and Development activities with respect to each Discovery Program in accordance with the applicable Discovery Program Development Plan and the terms and conditions of this Agreement. There shall be no Development Plan (or corresponding Development Budget) for any Discovery Program after the Research Period for such Discovery Program. At such time as there is no Development Plan for a Discovery Program, if Biogen has exercised the Option for such Discovery Program pursuant to the terms of Section 4.7, (i) Biogen may conduct Development activities for such Discovery Program in its sole discretion, subject to Section 4.3.1 and (ii) Biogen shall comply with the ongoing reporting obligations set forth in Section 10.1.2.

**4.2. Discovery Program License Grants**.

4.2.1. Subject to the terms and conditions of this Agreement, during the Research Period for a Discovery Program, AGTC, on behalf of itself and is Affiliates, hereby grants to Biogen an exclusive, royalty-free, fully paid-up license (exclusive even as to AGTC and its Affiliates except to the extent necessary for AGTC to perform its obligations under this Agreement) under the (a) Know-How that (i) AGTC or its Affiliates Control as of the Effective Date or that comes into the Control of AGTC or its Affiliates during such Research Period, (ii) relates to one or more product candidates under such Discovery Program or the Development of any of the foregoing and (iii) is necessary or useful for Biogen to perform Biogen's obligations under this Agreement in accordance with the applicable Discovery Program Development Plan for such Discovery Program, (b) any Patent Right that (i) AGTC Controls as of the Effective Date or that comes into the Control of AGTC during such Research Period and (ii) claims or discloses any Know-How described in clause (a) of this Section 4.2.1, (c) AGTC's interest in the Joint Technology and (d) the Materials transferred hereunder, in each case ((a) through (d)), solely to Develop, have Developed, Manufacture and have Manufactured product candidates under such Discovery Program in the Field in the Territory pursuant to the applicable Discovery Program Development Plan for such Discovery Program. Schedule 4.2.1 sets forth the Patent Rights licensed to Biogen under this Section 4.2.1 with respect to each Discovery Program as of the Execution Date, and shall be updated by the Patent Representatives in accordance with Section 13.3.1(a) to include any additional Patent Rights licensed to Biogen under this Section 4.2.1 with respect to each Discovery Program during the Research Period for such Discovery Program.

4.2.2. Subject to the terms and conditions of this Agreement, during the Research Period for a Discovery Program, Biogen, on behalf of itself and its Affiliates, hereby grants to AGTC a non-exclusive, royalty-free, fully paid-up license, under (a) any Know-How that (i) (x) is conceived, discovered, invented, created, made or reduced to practice or tangible medium by Biogen, any of its Affiliates or any of their respective employees, agents or independent contractors in the performance of Biogen's activities under this Agreement, (y) relates to one or more product candidates under such Discovery Program or the Development of any of the foregoing and (z) is necessary or useful for AGTC to perform AGTC's obligations under this Agreement in accordance with any Discovery Program Development Plan or (ii) is not Know-How defined in the foregoing subsection (i) and is Controlled by Biogen as of the Execution Date or otherwise comes into the Control of Biogen during the Term and that Biogen designates, in its sole discretion, under a Discovery Program Development Plan for use in Pre-Funded Discovery Activities thereunder, (b) any Patent Right, other than a Joint Patent Right, that (i) Biogen Controls as of the Effective Date or that comes into the Control of Biogen during such Research Period and (ii) claims or discloses any Know-How described in clause (a) of this Section 4.2.2 and (c) Biogen's interest in the Joint Technology, in each case solely to Develop, have Developed, Manufacture and have Manufactured product candidates under such Discovery Program in the Field in the Territory pursuant to the applicable Discovery Program Development Plan for such Discovery Program.

4.2.3. Subject to the restrictions set forth on Schedule 5.2, each Party shall have the right to grant sublicenses of any and all rights granted to such Party by the other Party under Section 4.2.1 or 4.2.2, as applicable, to one or more of its Affiliates and to one or more Third Parties solely to the extent such Affiliate or Subcontractor requires such a sublicense to carry out activities consistent with the Discovery Program Development Plan. Each such sublicense shall be subject and subordinate to, and consistent with, the terms and conditions of this Agreement and shall require such Sublicensee(s) to comply with all applicable terms of this Agreement.

**4.3. Diligence**.

4.3.1. Diligence Obligations.

(a) During the Research Period for each Discovery Program that is not a Terminated Discovery Program, each Party shall use Commercially Reasonable Efforts to conduct the Pre-Funded Discovery Activities for such Discovery Program as set forth in the applicable Discovery Program Development Plan through Clinical Candidate Designation.

(b) With respect to any Discovery Program for which Biogen has exercised the Option in accordance with Section 4.7, Biogen will use Commercially Reasonable Efforts to develop and seek Regulatory Approval for at least one Discovery Product for such Discovery Program in the Major Market Countries.

4.3.2. Exceptions to Diligence Obligations. Notwithstanding any provision of this Agreement to the contrary, the Obligated Party will be relieved from and will have no obligation to undertake any efforts under Section 4.3.1 with respect to the Development of any Discovery Product in the event that:

(a) either Party receives or generates any safety, tolerability or other data reasonably indicating, as measured by the Obligated Party's safety and efficacy evaluation criteria and methodology, that a Discovery Product is not reasonably suitable, for safety reasons, for initiation or continuation of Clinical Trials in humans; or

(b) the other Party materially breaches any of its Development or other obligations under the Development Plans or this Agreement and such breach impairs or limits the Obligated Party's ability to perform its Development activities under this Agreement; provided that, in such event, the Obligated Party shall only be relieved of such obligations to the extent and for so long as the other Party's breach so impairs or limits the Obligated Party's ability to perform its Development activities under this Agreement.

**4.4. Substitution of Discovery Programs**.

4.4.1. As of the Effective Date, the Discovery Programs are comprised of the [***] Discovery Program, the ALD/[***] Discovery Program and the [***]. With respect to any Discovery Program (or in the case the ALD/[***] Discovery Program is substituted under Section 4.4.4, the Non-Ophthalmology Discovery Program), at any time until the earliest of (i) the Option Exercise Date for such Discovery Program, (ii) the end of the Option Exercise Period for such Discovery Program or (iii) [***] from the Effective Date, Biogen may decide to terminate such Discovery Program and to designate an alternative program as a Discovery Program under this Agreement (such alternative program, a "**Substitute Discovery Program**"), under the following conditions: (a) neither Biogen, nor its Affiliates nor their respective sublicensees is engaged in researching, Developing or Commercializing an AAV Product directed to the gene target that is the subject of such Substitute Discovery Program, (b) Biogen shall have the right to deliver a Substitution Notice and invoke the process set forth in Section 4.4.2 for any reason during the period starting on the Effective Date and ending [***] thereafter, provided that Biogen may only make such request once in any [***], (c) Biogen shall have the right to deliver a Substitution Notice and invoke the process set forth in Section 4.4.2 at any time upon failure of a Discovery Program, as mutually determined by the Parties, even if Biogen has previously delivered a Substitution Notice during the same twelve month period and (d) Biogen may designate a Substitute Discovery Program no more than twice in accordance with this Section 4.4.

4.4.2. In the event Biogen wishes to designate a Substitute Discovery Program, Biogen shall notify AGTC of such desire, identifying the Discovery Program to be terminated (the "**Terminated Discovery Program**") and the corresponding products that are generated by or is the subject of such Discovery Program to be terminated (the "**Terminated Discovery**

**Products**"), and listing indications or gene targets which Biogen is interested in pursuing (the "**Substitution Notice**"). Within thirty (30) days of receiving such Substitution Notice from Biogen, AGTC shall deliver to Biogen a notice (the "**Available Program Notice**") including (a) a list of (i) all then-current [***] Programs in research or development other than an Abandoned Program, and (ii) in AGTC's sole discretion, any other program that AGTC is researching or developing which AGTC desires to research or develop with Biogen as a Discovery Program, and (b) an annotated list identifying those indications or gene targets included in the Substitution Notice [***] (such programs included in clauses (a) and (b), the "**Available Programs**") and shall provide Biogen, at Biogen's request, any information in AGTC's possession regarding any such Available Program sufficient in AGTC's reasonable determination to allow Biogen to determine whether it wishes to designate any Available Program as a Substitute Discovery Program. Biogen shall, within thirty (30) days following receipt of the Available Program Notice, designate any one Available Program as a Substitute Discovery Program (the "**Designation Notice**"). Effective as of the date of the Designation Notice, the Terminated Discovery Program shall immediately cease to be a Discovery Program hereunder and the program identified in the Designation Notice shall be a Substitute Discovery Program (the "**Discovery Program Substitution Date**"). On the Discovery Program Substitution Date, subject to Section 4.5, the terms and conditions of this Agreement applicable to Discovery Programs shall become effective with respect to such Substitute Discovery Program. The JDC shall within fifteen (15) days after the Discovery Program Substitution Date discuss in good faith and determine whether the Substitute Discovery Program shall be subject to the milestones set forth in Category A, Category B or Category C of Section 6.5.2, which may or may not be the same category of milestones as the Terminated Discovery Program.

4.4.3. Provided that Biogen has not designated a Substitute Discovery Program more than twice in accordance with this Section 4.4, with respect to a Terminated Discovery Program, for a period of [***] months following the date of the Substitution Notice, AGTC shall notify Biogen if it decides to continue, either itself or through an Affiliate or Third Party, to Develop products under a Terminated Discovery Program due to a discovery regarding such Terminated Discovery Program. If Biogen wishes to reinstate such Terminated Discovery Program as a Discovery Program, subject to the terms and conditions of this Agreement that applied to such Terminated Discovery Program before the applicable substitution designation was made for such Terminated Discovery Product, then Biogen shall notify AGTC of such desire within thirty (30) days after receipt of such notice of continued development from AGTC. If Biogen notifies AGTC that it does not wish to reinstate such Terminated Discovery Program or if Biogen does not respond to such notice of continued development from AGTC within such thirty (30) day period, such Terminated Discovery Program shall be deemed to be an Abandoned Program and AGTC shall be free to develop products under a Terminated Discovery Program, whether or not through an Affiliate or Third Party. Until such time that such Terminated Discovery Program becomes an Abandoned Program, AGTC shall not extend to any Third Party a right or license that would preclude AGTC from granting to Biogen the license set forth in Section 4.2.1 for such Terminated Discovery Program. For clarity, Biogen may have more than three (3) total Discovery Programs as a result of the provisions of this Section 4.4.3.

4.4.4. Notwithstanding the foregoing, the Parties acknowledge and agree that the viability of the ALD/[***] Discovery Program may be dependent upon certain factors, including the access to certain Third Party Technology. The Parties intend to make inquiries regarding such Third Party Technology to determine whether a license to such Third Party Technology would be necessary or useful in order to conduct the ALD/[***] Discovery Program. If, following such inquiries, Biogen determines that it does not wish to continue the ALD/[***] Discovery Program, the Parties may agree to substitute an alternate non-ophthalmology program for the ALD/[***] Discovery Program, without triggering the formal substitution process set forth in this Section 4.4, but subject to criteria of availability of such program as set forth in this Section 4.4. Biogen shall use reasonable efforts to make such determination during the three (3) month period following the Execution Date, such determination period not to exceed four (4) months or as mutually agreed between the Parties (such alternate non-ophthalmology program, the "**Non-Ophthalmology Discovery Program**"). In the event of any disagreement between the Parties regarding the substitution of an alternate program, Biogen shall have the final decision-making authority with respect to whether or not the ALD/[***] Discovery Program will be substituted, but not, for purposes of clarity, whether a substituted program is an Available Program. For clarity, designation of the Non-Ophthalmology Discovery Program shall not count toward Biogen's two (2) opportunities to designate a Substitute Discovery Program under clause (d) of Section 4.4.1.

**4.5. Discovery Program Development Plans**. For each Discovery Program other than a Substitute Discovery Program, the initial research and Development activities to be conducted by AGTC with respect to such Discovery Program shall be set forth in the initial Discovery Program Development Plan for such Discovery Program. The initial [***] Discovery Program Development Plan is

attached hereto as Exhibit A-3, and the initial ALD/[***] Discovery Program Development Plan is attached hereto as Exhibit A-4. The JDC shall discuss, prepare and approve the initial [***] in accordance with Section 2.1.2(b)(ii) within three (3) months of the Parties' mutual agreement on the gene targets for the [***] in accordance with Section 1.104. Upon such approval by the JDC, the initial [***] Development Plan shall be attached hereto as Exhibit A-5 and the initial [***] Development Plan shall be attached hereto as Exhibit A-6. With respect to the Non-Ophthalmology Discovery Program, if applicable, promptly following the date such Non-Ophthalmology Discovery Program is designated under Section 4.4.4, the JDC shall discuss in good faith and mutually agree upon a Discovery Program Development Plan for such Non-Ophthalmology Discovery Program covering Development activities through Clinical Candidate Designation for such Non-Ophthalmology Discovery Program, which Discovery Program Development Plan shall be attached as Exhibit A-7 hereto. With respect to any Substitute Discovery Program, promptly following the Discovery Program Substitution Date, the JDC shall discuss in good faith and mutually agree upon a Discovery Program Development Plan for such Substitute Discovery Program covering Development activities through Clinical Candidate Designation for such Substitute Discovery Program, which Discovery Program Development Plan shall be attached as Exhibit A-8 (or Exhibit A-9, if applicable) hereto. If the Parties are unable to agree on a Discovery Program Development Plan for any Substitute Discovery Program within thirty (30) days of Biogen's selection of such Substitute Discovery Program, then Biogen shall have final decision-making authority to determine the remaining activities for which agreement has not been reached under such Discovery Program Development Plan, subject to the provisions of Section 4.6. The JDC shall review and update the Discovery Program Development Plans and related Development Budgets on a quarterly basis, or more often as necessary.

**4.6. Development Costs**.

4.6.1. R&D Pre-Funding. Subject to the provisions of Section 4.6.3, AGTC shall be solely responsible for the payment of all Development Costs associated with AGTC's Pre-Funded Discovery Activities through Clinical Candidate Designation for each of the [***] Discovery Program, the ALD/[***] Discovery Program (or the Non-Ophthalmology Discovery Program, if applicable), and the [***] in accordance with the applicable Discovery Program Development Plan. It is the intention of the Parties that the applicable portion of the [***] will cover all Development Costs associated with the Pre-Funded Discovery Activities through Clinical Candidate Designation under the Discovery Program Development Plan for each of the [***] Discovery Program, the ALD/[***] Discovery Program (or the Non-Ophthalmology Discovery Program, if applicable) or the [***] as set forth in the applicable Development Budget, but, for clarity, if such R&D Pre-Funding does not fully cover such Development Costs, [***] will, subject to Section 4.6.2 with respect to Substitute Discovery Programs and Section 4.6.3, be responsible for all such Development Costs required under each Discovery Program. Within three (3) Business Days after the end of any Calendar Quarter in which [***] has incurred Development Costs in the course of performing Pre-Funded Activities in accordance with any Discovery Program Development Plan, [***] shall provide to [***] (a) a statement of actual Development Costs incurred [***] (which shall include a determination of [***] internal costs based on the R&D FTE Rate) and (b) a reasonable estimate of Development Costs incurred [***] (which shall include an estimate of AGTC's internal costs based on the R&D FTE Rate) and, within thirty (30) days of the end of any such Calendar Quarter, [***] shall provide to Biogen a final report of such Development Costs.

4.6.2. Development Costs for Substitute Discovery Programs. Within sixty (60) days of the Discovery Program Substitution Date, AGTC shall, if applicable, notify Biogen in writing of the aggregate Development Costs incurred by AGTC in conducting the Pre-Funded Discovery Activities to date under the Discovery Program Development Plan for the Terminated Discovery Program, and shall provide Biogen with copies of records necessary to verify the foregoing. In the event that the aggregate Development Costs incurred by AGTC in conducting such activities, if any, were less than the total amount of R&D Pre-Funding budgeted for such Discovery Program, then the remainder of the R&D Pre-Funding budgeted for such Discovery Program shall be used to fund the Development Costs associated with the Pre-Funded Discovery Activities of AGTC under the Discovery Program Development Plan for the applicable Substitute Discovery Program. In addition, any excess R&D Pre-Funding under Section 3.2.3 or Section 4.6.4 for a terminated Collaboration Program shall also be used to fund such Development Costs, to the extent necessary. Biogen shall be responsible for any Development Costs associated with the Pre-Funded Discovery Activities of AGTC under the Discovery Program Development Plan for the applicable Substitute Discovery Program as set forth in the applicable Development Budget, to the extent not covered by such remainder of the R&D Pre-Funding from the Terminated Discovery Program or any such other terminated Collaboration Program. Any amounts owed to AGTC by Biogen under this Section 4.6.2 shall be paid by Biogen to AGTC within forty-five (45) days of receipt of an invoice from AGTC delivered within forty-five (45) days after the end of each Calendar Quarter that provides reasonable detail regarding such excess Development Costs.

4.6.3. Budget Overages.

(a) If, during the Research Period for any Discovery Program, any Development Costs incurred by AGTC in conducting any activity set forth in the applicable Discovery Program Development Plan for the [***] Discovery Program, the ALD/[***] Discovery Program (or the Non-Ophthalmology Discovery Program, if applicable), the [***], the [***] or a Substitute Discovery Program exceed the budgeted amount therefor as set forth in the applicable Development Plan, then [***] shall be solely responsible for all Development Costs in excess of the budgeted amount for such activity, provided that, to the extent such excess Development Costs are caused by the recklessness, negligence or intentional misconduct of [***] or its Affiliates or their respective Sublicensees or Subcontractors, [***] shall be solely responsible for such Development Costs.

(b) Notwithstanding the foregoing, if the JDC amends a Discovery Program Development Plan to include additional activities or change the scope of any existing activities or a Regulatory Authority requires that additional activities be performed or a change in scope, with respect to a Discovery Program, and such amendment requires an increase in Development Costs under the corresponding Development Budget, then [***] shall pay such increased Development Costs.

4.6.4. Excess R&D Pre-Funding. If any Discovery Program is terminated prior to completion of all Pre-Funded Discovery Activities for such Discovery Program, then any R&D Pre-Funding for Pre-Funded Discovery Activities under such Discovery Program that have not yet been undertaken shall be allocated toward Development Costs payable by Biogen for other Collaboration Programs (including, as applicable, for any Substitute Discovery Program as set forth in Section 4.6.2). [***]

**4.7. Option Grant; Option Exercise**. With respect to each Discovery Program, AGTC hereby grants to Biogen an exclusive option during the Option Exercise Period for such Discovery Program to obtain an exclusive license under the AGTC Technology and AGTC's interest in the Joint Technology to use, have used, Develop, have Developed, Manufacture, have Manufactured, Commercialize, have Commercialized, import, have imported, export and have exported Discovery Products on the terms set forth in Section 5.1 (the "**Option**"). Within fifteen (15) days of Clinical Candidate Designation for any Discovery Program, AGTC shall notify Biogen of such Clinical Candidate Designation and shall provide Biogen with a Data Package for such Discovery Program. If Biogen reasonably believes that the Data Package is incomplete, it may notify AGTC of any such deficiency, and AGTC will promptly provide to Biogen any additional information reasonably requested by Biogen, provided that no additional Development activities are required to provide such information. Biogen shall have the right to exercise the Option at any time within ninety (90) days of Biogen's receipt of the Data Package, including any additional information reasonably requested by Biogen pursuant to the preceding sentence, for a Discovery Program (the "**Option Exercise Period**"), upon written notice to AGTC (the date that AGTC receives such notice, the "**Option Exercise Date**"). Effective immediately upon payment of the Option Fee within sixty (60) days of the Option Exercise Date as set forth in Section 6.5.1, with respect to any Discovery Program for which Biogen has exercised an Option as set forth in this Section 4.7, (a) AGTC shall and hereby does grant to Biogen the license set forth in Section 5.1 with respect to the Discovery Products for such Discovery Program and (b) the Parties shall update Schedule 1.22-2 hereto to include those Patent Rights set forth on Schedule 4.2.1 with respect to the applicable Discovery Products and to reflect any additional AGTC Patent Rights existing as of the Option Exercise Date. For clarity, upon exercise of the Option by Biogen, all terms and conditions of this Agreement applicable to Licensed Products shall become effective with respect to the applicable Discovery Products, provided that those terms and conditions of this Agreement specifically applicable to Initial Licensed Products (*e.g.*, the Cost Share Option and the Co-Promotion Option) shall not apply to the applicable Discovery Products. Notwithstanding anything to the contrary, in the event that the Parties mutually agree to incorporate [***] under the [***] into a single clinical candidate that achieves Clinical Candidate Designation, then, if Biogen desires to exercise the Option with respect to such clinical candidate, the Option Fee shall be payable only once for such clinical candidate, and such clinical candidate shall thereafter be deemed a "[***]".

**4.8. Subcontractors**. Each Party may engage Subcontractors to perform any work under the Discovery Programs; provided that, with respect to any Subcontractor of AGTC that has not previously been engaged by AGTC within the [***] prior to such proposed engagement, all such engagements by AGTC and any contracts related to such engagements shall be subject to the prior written approval of Biogen, such approval not to be unreasonably withheld; and provided, further, that in the event AGTC elects to engage a Subcontractor to provide any service for which Biogen possesses internal capabilities to perform such service at a cost equal to or less than such Subcontractor and on substantially similar terms (including capability and timing), AGTC shall notify Biogen and Biogen shall determine whether Biogen, at AGTC's sole expense subject to cost sharing provisions included in Section 4.6.3, to the

extent set forth in the applicable Development Budget, will provide such service to AGTC in accordance with the applicable Development Plan. Biogen shall reasonably consult with AGTC with respect to the engagement of Subcontractors but shall be permitted to engage Subcontractors without prior approval of AGTC, at Biogen's sole expense. Each contract between a Party and a Subcontractor shall be consistent with the provisions of this Agreement and shall include provisions, including intellectual property provisions, adequate for the other Party to avail itself of the licenses granted hereunder as though such Party had performed the contracted work. Each Party shall be responsible for the effective and timely management of and payment of its Subcontractors. The engagement of any Subcontractor in compliance with this Section 4.8 shall not relieve the applicable Party of its obligations under this Agreement or any Development Plan. Each Party shall be solely responsible for any taxes, including income, withholding, payroll, VAT, sales tax or the like, that arise from the use of a Subcontractor. To the extent AGTC engages any Subcontractor to perform any Manufacturing activities, AGTC shall notify Biogen of proposed substantive interactions with any such Subcontractors material to a Discovery Program and shall use reasonable efforts to allow Biogen to participate in any such interactions primarily related to a Discovery Program if Biogen requests such involvement, at Biogen's sole cost and expense, provided that such reasonable efforts and such participation do not cause undue delay to any Discovery Program.

**4.9. Conduct**. Each Party shall, and shall require its Affiliates and Subcontractors to, comply with all applicable Laws in their conduct of the Pre-Funded Discovery Activities with respect to the Discovery Programs, including where appropriate cGMP, GCP and GLP (or similar standards) for the performance of laboratory activities.

**5. LICENSE GRANTS**.

**5.1. Exclusive License from AGTC to Biogen**. Subject to the terms and conditions of this Agreement and effective as of the Effective Date for the Initial Licensed Products and as of the Option Exercise Date for the applicable Discovery Product, AGTC, on behalf of itself and its Affiliates, hereby grants to Biogen an exclusive license (exclusive even as to AGTC and its Affiliates except to the extent necessary for AGTC to perform its obligations under this Agreement), with the right to grant sublicenses through multiple tiers pursuant to Section 5.2, under the AGTC Technology, AGTC's interest in the Joint Technology and the Materials transferred hereunder, to use, have used, Develop, have Developed, Manufacture, have Manufactured, Commercialize, have Commercialized, import, have imported, export and have exported Licensed Products (which for purposes of clarity, consist of the Initial Licensed Products and Discovery Products for which Biogen has exercised an Option in accordance with Section 4.7) in the Field in the Territory.

**5.2. Biogen Sublicensees**. Subject to the restrictions set forth on Schedule 5.2, Biogen shall have the right to grant sublicenses through multiple tiers to one or more of its Affiliates and to one or more Sublicensees of any and all rights granted to Biogen under this Agreement by AGTC, provided that in no event may Biogen grant a sublicense, and Biogen shall use reasonable efforts to ensure that none of its Affiliates or their respective Sublicensees grant a sublicense, of any of the rights licensed under Section 5.1 with respect to an Initial Licensed Product to any Person that is (i) Developing or Commercializing a product targeting the same gene as such Initial Licensed Product if, at the time that Biogen grants such sublicense, Biogen is Developing such Initial Licensed Product or (ii) Commercializing a product targeting the same gene as such Initial Licensed Product if, at the time that Biogen grants such sublicense, Biogen is Commercializing such Initial Licensed Product in each case of (i) or (ii), without AGTC's prior written consent, which AGTC may give in its sole discretion. Each such sublicense shall be subject and subordinate to, and consistent with, the terms and conditions of this Agreement. The engagement of any Sublicensee in compliance with this Section 5.2 shall not relieve Biogen of its obligations under this Agreement or any Development Plan. Biogen shall remain responsible for actions or omissions of its Sublicensees and Biogen's breaches under this Agreement that are caused by its Sublicensee's breach of any sublicense agreement (or delay caused by such breach). Biogen shall provide a redacted copy of each sublicense to AGTC promptly following execution of such sublicense.

**5.3. Non-Exclusive License from Biogen to AGTC**. Subject to the terms and conditions of this Agreement, during the Term, Biogen, on behalf of itself and its Affiliates, hereby grants to AGTC a non-exclusive, royalty-free, fully paid-up license in the Territory, with no right to grant sublicenses except as permitted to Subcontractors under Section 3.4 or Section 4.8, under the Biogen Technology and Biogen's interest in the Joint Technology, solely to perform Development activities as set forth in the Development Plans.

**5.4. Enabling Licenses.**

5.4.1. Joint Technology. Subject to the terms and conditions of this Agreement, each Party, on behalf of itself and its Affiliates, hereby grants to the other Party a non-exclusive, worldwide, royalty-free, fully paid-up, irrevocable license, with the right to grant sublicenses through multiple tiers, under its interest in the Joint Technology (other than the Joint Improved Technology), to use, have used, Develop, have Developed, Manufacture, have Manufactured, Commercialize, have Commercialized, import, have imported, export and have exported products or processes, [***].

5.4.2. Enabling License from Biogen to AGTC.

(a) Subject to the terms and conditions of this Agreement and effective as of the Effective Date, Biogen, on behalf of itself and its Affiliates, hereby grants to AGTC a non-exclusive, worldwide, royalty-bearing license, with the right to grant sublicenses through multiple tiers, under the Biogen Platform Improvement Technology to use, have used, Develop, have Developed, Manufacture, have Manufactured, Commercialize, have Commercialized, import, have imported, export and have exported Gene Therapy Products.

(b) If any Gene Therapy Product sold by AGTC, its Affiliates or Sublicensees is Covered by a Valid Claim of a Biogen Platform Improvement Patent Right licensed to AGTC under this Section 5.4.2 in the country in which such Gene Therapy Product is made, used or sold, then on a country-by-country basis AGTC will pay to Biogen a royalty at a rate to be agreed upon by the Parties of up to [***] of net sales (as determined in accordance with Section 5.4.2(d) and calculated in accordance with Section 1.184, which definition of Net Sales shall apply *mutatis mutandis* to such calculation) of such Gene Therapy Product on a country-by-country and Gene Therapy Product-by-Gene Therapy Product basis, until the latest of (a) the expiration of the last to expire of any Valid Claim included in any Patent Right licensed to AGTC under this Section 5.4.2 in such country which Valid Claim Covers the manufacture, use, sale, offer for sale or importation of such Gene Therapy Product in such country, (b) [***].

(c) Such royalties shall be paid in accordance with the provisions of Section 6.7, which shall apply *mutatis mutandis* to payments made by AGTC pursuant to this Section 5.4.2, provided, however, that if AGTC licenses or has prior to the Effective Date licensed, intellectual property rights from one or more Third Parties, in either case, which intellectual property rights are necessary or useful to, and are actually used at any time to, exercise the license under Section 5.4.2(a), whether directly or through any AGTC Affiliate or Sublicensee, then any royalties otherwise payable to Biogen under Section 5.4.2(b) shall be reduced by [***] of the royalties paid to Third Parties pursuant to any such Third Party licenses arising out of and directly attributable and proportionately allocated to the exercise of the license under 5.4.2(a), provided that in no event shall any royalty payable to Biogen under this Section 5.4.2 be reduced to less than [***] (unless the royalty rate determined under Section 5.4.2(b) or Section 5.4.2(d) is less than [***], in which case no royalty reduction will apply); provided, however, that any amounts paid under such Third Party license that are not used to reduce a payment due hereunder as a result of the foregoing limitations may be carried over to reduce subsequent payments due under this Section 5.4.2.

(d) If the Parties are unable to agree upon the applicable royalty rate within thirty (30) days of the commencement of discussions regarding such royalty rate, then the Parties shall select a mutually agreed external neutral expert with significant and relevant experience to decide upon a commercially reasonable royalty rate of up to [***], which external neutral expert shall not have previously served as an employee of either Party or, within the two (2) years prior to the external neutral expert's engagement by the Parties pursuant to this Section 5.4.2, as a consultant or third party expert for either Party. The Parties shall cooperate with such external neutral expert to enable such external neutral expert to reach a decision as quickly as possible. The decision of the external neutral expert shall be final, non-appealable and binding on the Parties. Biogen and AGTC shall share equally the costs and fees of such external neutral expert regardless of the decision by the external neutral expert.

5.4.3. Enabling License from AGTC to Biogen. Subject to the terms and conditions of this Agreement and effective as of the Effective Date, during the Term, AGTC, on behalf of itself and its Affiliates, hereby grants to Biogen a worldwide, royalty-free, perpetual, irrevocable license, with the right to grant sublicenses through multiple tiers, under the AGTC Improved Technology and AGTC's interest in the Joint Improved Technology, to use, have used, Develop, have Developed, Manufacture, have Manufactured, Commercialize, have Commercialized, import, have imported, export and have exported

any products. The license granted under this Section 5.4.3 shall be exclusive (even as to AGTC and its Affiliates except to the extent necessary for AGTC to perform its obligations under this Agreement) with respect to the Joint Improved Technology and non-exclusive with respect to the AGTC Improved Technology, provided that such license shall be exclusive with respect to the AGTC Improved Technology to the extent required pursuant to any Third Party agreement of Biogen.

**5.5. Existing License Agreements**.

5.5.1. The rights granted to Biogen, its Affiliates or Sublicensees under this Agreement are subordinate to the terms and conditions of the Existing License Agreements, including the coordination of prosecution or enforcement of Patent Rights or other intellectual property rights under the applicable agreement.

5.5.2. Biogen shall be entitled to grant a sublicense under its sublicense rights in the [***] Agreements in conjunction with a license to technology owned or controlled by Biogen that (a) is included in or useful for the making of [***] Products and (b) is intended to be included in or used in the manufacture of [***] Products by the Sublicensee. Biogen shall only be entitled to sublicense its rights under each [***] Agreement on the terms set forth in in Section 2.3 of such [***] Agreement.

5.5.3. It is understood that the United States Government (through any of its agencies or otherwise) has funded research, [***] during the course of or under which certain of the inventions of the AGTC Patent Rights licensed to AGTC under Existing License Agreements were conceived or made. The United States Government is entitled, as a right, under the provisions of 35 U.S.C. §202-212 and applicable regulations of Title 37 of the Code of Federal Regulations, to a non-exclusive, nontransferable, irrevocable, paid-up license to practice or have practiced the inventions of such AGTC Patent Rights for governmental purposes. Any license granted to Biogen in this Agreement shall be subject to such right.

5.5.4. Biogen shall include the following provisions in any sublicense to a Sublicensee, revised as appropriate to apply to such Sublicensee as it applies to Biogen, to the extent such AGTC Technology is sublicensed and to the extent such provision applies to AGTC's licensors of such AGTC Technology: 5.5, 8.1.6(b), 11.4, 14.2.4, 14.6.3, 15.1.8, 15.3, 15.4, 15.5, 15.6, 15.7, 17.2, 17.5, 17.6.2, 17.6.3, 18.1, 18.9 and 18.15. The Parties acknowledge and agree that in the event that any Technology is included in the licenses granted to Biogen under this Agreement pursuant to Section 13.6.2(a), additional obligations and restrictions may need to be included in this Agreement prior to such Technology being included in such licenses. Without limiting the foregoing, upon Biogen's election to take a sublicense under Section 13.6.2(a) to any Technology, the Parties shall update Schedule 5.2 to include any restrictions on Biogen's right to sublicense such Technology.

**5.6. Right of Reference**. AGTC hereby grants to Biogen a "Right of Reference", as that term is defined in 21 C.F.R. § 314.3(b) and any analogous regulation outside of the United States, to any data Controlled by AGTC or its Affiliates that is necessary or useful to Develop, Manufacture, Commercialize or use any Licensed Product, and AGTC shall provide a signed statement to this effect, if requested by Biogen, in accordance with 21 C.F.R. § 314.50(g)(3) (or any analogous applicable Law recognized outside of the United States).

**5.7. No Implied Rights**. Except as expressly provided in this Agreement, neither Party shall be deemed to have granted the other Party any license or other right with respect to any intellectual property of such Party.

**5.8. Exclusivity**.

5.8.1. Initial Licensed Programs. On an Initial Licensed Program-by-Initial Licensed Program basis, for so long as AGTC, Biogen or any of their respective Affiliates or Sublicensees are (a) Developing any Initial Licensed Product from such Initial Licensed Program under this Agreement, (i) neither AGTC nor any of its Affiliates shall work independently of this Agreement for itself or any Affiliate or Third Party (including the grant of any license, option or other right to any Third Party) with respect to the Development, Manufacture or Commercialization of any Gene Therapy Product, and (ii) neither Biogen nor any of its Affiliates shall work independently of this Agreement for itself or any Affiliate or Third Party (including the grant of any license, option or other right to any Third Party) with respect to the Development, Manufacture or Commercialization of any Gene Therapy Product, or (b) Commercializing any Initial Licensed Product from such Initial

Licensed Program under this Agreement, (i) neither AGTC nor any of its Affiliates shall work independently of this Agreement for itself or any Affiliate or Third Party (including the grant of any license, option or other right to any Third Party) with respect to the Manufacture or Commercialization of any Gene Therapy Product, and (ii) neither Biogen nor any of its Affiliates shall work independently of this Agreement for itself or any Affiliate or Third Party (including the grant of any license, option or other right to any Third Party) with respect to Manufacture or Commercialization, of any Gene Therapy Product, in each case of (a) or (b), that delivers (1) an RS-1 transgene [***], if such Initial Licensed Program is the XLRS Program or (2) an [***] transgene [***], if such Initial Licensed Program is the XLRP Program.

5.8.2. Discovery Programs.

(a) On a Discovery Program-by-Discovery Program basis for the [***] Discovery Program, the ALD/[***] Discovery Program (or the Non-Ophthalmology Discovery Program, if applicable), the [***], the [***] or any Substitute Discovery Program, commencing at the start of the Research Period with respect to each such Discovery Program and for so long as AGTC, Biogen or any of their Affiliates or Sublicensees are (i) Developing any Discovery Product from such Discovery Program under this Agreement, neither AGTC nor any of its Affiliates shall work independently of this Agreement for itself or any Affiliate or Third Party (including the grant of any license, option or other right to any Third Party) with respect to the Development, Manufacture or Commercialization of any AAV Product, or (ii) Commercializing any Discovery Product from such Discovery Program under this Agreement, neither AGTC nor any of its Affiliates shall work independently of this Agreement for itself or any Affiliate or Third Party (including the grant of any license, option or other right to any Third Party) with respect to the Manufacture or Commercialization of any AAV Product, in each case of (i) or (ii) that delivers (1) [***], with respect to the [***] Discovery Program, 2) [***], with respect to the [***], (3) [***], with respect to the [***], (4) [***], with respect to the [***], (5) if the Non-Ophthalmology Discovery Program is designated in accordance with Section 4.4.4, [***], or (6) if Biogen selects a Substitute Discovery Program under Section 4.4, [***], provided that, for any Abandoned Program, AGTC's obligations under this Section 5.8.2(a), as applicable, shall terminate immediately.

(b) On a Discovery Program-by-Discovery Program basis for the [***] Discovery Program, the ALD/[***] Discovery Program (or the Non-Ophthalmology Discovery Program, if applicable), the [***] Discovery Program or any Substitute Discovery Program, commencing at the start of the Research Period with respect to each such Discovery Program and ending upon the earliest of (i) the date that such Discovery Program becomes a Terminated Discovery Program, (ii) the date that Biogen exercises the Option with respect to such Discovery Program or (iii) [***] years from the Effective Date or [***] years from the Discovery Program Substitution Date with respect to the applicable Substitute Discovery Program, neither Biogen nor any of its Affiliates shall, while Developing a product candidate under such Discovery Program, work independently of this Agreement for itself or any Affiliate or Third Party (including the grant of any license, option or other right to any Third Party) with respect to the Development, Manufacture or Commercialization of any AAV Product that delivers (a) [***], with respect to the [***] Discovery Program, (b) [***], with respect to the [***] Discovery Program, (c) [***], with respect to the [***], (d) [***], with respect to the [***] (e) if the Non-Ophthalmology Discovery Program is designated in accordance with Section 4.4.4, [***], or (e) if Biogen selects a Substitute Discovery Program under Section 4.4, [***].

5.8.3. Competing Program; Change of Control.

(a) Notwithstanding the provisions of Section 5.8.1 and Section 5.8.2, if during the Term either Party acquires a Third Party or a portion of the business of a Third Party (whether by merger, stock purchase or purchase of assets) that is, prior to such acquisition, engaged in researching, Developing or Commercializing a Gene Therapy Product in XLRS or XLRP or an AAV Product in a Discovery Program that would violate the provisions of Section 5.8.1 or Section 5.8.2 if conducted by such Party (a "**Competing Program**"), such Party shall use Commercially Reasonable Efforts to divest such Competing Program promptly following the closing of such acquisition, and in any event shall complete such divestment within one year after the closing of such acquisition; provided that such time period shall be extended, and such Party shall not be in breach of this Section 5.8.3, if at the expiration thereof (and any extensions thereto) such Party provides competent evidence of reasonable ongoing efforts to divest such Competing Program; and provided, further, that such Party shall cease all research, Development and

Commercialization activities with respect to such Competing Program if such Party has not completed such divestment within [***] after the closing of such acquisition (it being understood that such Party may thereafter continue its efforts to divest such asset). During such divestment period, the acquiring Party shall (i) segregate the Competing Program with the Collaboration Programs, including, to the extent practicable, establishing separate teams to conduct Development activities under the Collaboration Programs and such Competing Program, and (ii) use good faith efforts to prevent any Confidential Information relating to the applicable Collaboration Program from being disclosed to, or used by, individuals performing Development activities under such Competing Program. For the avoidance of doubt, neither Party nor its Affiliates may acquire a Competing Program on a standalone basis.

(b) In the event of a Change of Control of either Party during the Term, the obligations of such Party under Section 5.8.1 and Section 5.8.2 shall not apply to any Gene Therapy Product, with respect to XLRS or XLRP, or AAV Product, with respect to a Discovery Program, that (i) is owned or controlled by a Third Party described in the definition of "Change of Control" or its Affiliates prior to or as of the closing of such Change of Control or (ii) becomes owned or controlled by such Third Party or its Affiliates after the closing of such Change of Control if such Gene Therapy Product or AAV Product, as applicable, is not developed using any Know-How or with access to any Know-How, and is not Covered by any Patent Rights, that were Controlled by such Party or licensed to such Party under this Agreement prior to the closing of the Change of Control.

5.8.4. Other Programs. Each Party understands and acknowledges that the other Party may have present or future initiatives or opportunities, including initiatives or opportunities with Third Parties, involving similar products, programs, technologies or processes that may compete with a product, program, technology or process covered by this Agreement. Each Party acknowledges and agrees that nothing in this Agreement other than the provisions of Section 5.8.1, Section 5.8.2 or Section 5.8.3 will be construed as a representation, warranty, covenant or inference that either Party will not itself Develop, Manufacture or Commercialize or enter into business relationships with one or more Third Parties to Develop, Manufacture or Commercialize products, programs, technologies or processes that are similar to or that may compete with any product, program, technology or process covered by this Agreement, provided that such Party will not use the other Party's Confidential Information in breach of this Agreement.

**5.9. Right of Notification for** [***]. AGTC shall notify Biogen of any upcoming publication, presentation or press release regarding the [***] Program, which information, for the avoidance of doubt, shall be AGTC's Confidential Information. If, at any time during the Term, AGTC (a) seeks to grant any rights to the [***] to any Third Party or (b) receives any written expression of interest from a Third Party for the [***], then AGTC shall each such time promptly (and, in any event, no later than five (5) Business Days following the execution of a confidentiality agreement with any Third Party with respect to such potential transaction) provide written notice to Biogen. Thereafter, AGTC shall consider in its sole discretion any timely proposal by Biogen to add the [***] as a Licensed Program under this Agreement. For the avoidance of doubt, AGTC shall have no obligation to negotiate with or enter into any definitive agreement with Biogen with respect to the [***].

**6. FINANCIAL TERMS**.

**6.1. Upfront Fees**. Within fifteen (15) days after the Effective Date, Biogen shall pay to AGTC a sum of Ninety-Four Million Dollars ($94,000,000), payable by wire transfer of immediately available funds according to instructions that AGTC shall provide, and shall be allocated as follows: (a) an upfront fee of [***] in consideration of the licenses granted to Biogen for the XLRS Program, (b) an upfront fee of [***] in consideration of the licenses granted to Biogen for the XLRP Program, (c) an access fee in the aggregate amount of [***] in consideration of the Options granted to Biogen under the Discovery Programs, (d) pre-paid research and Development funding for the XLRS Program in the amount of [***], (e) pre-paid research and Development funding for the XLRP Program in the amount of [***] and (f) prepaid research and Development funding for the Discovery Programs in the aggregate amount of [***] (the amounts in (d) through (f), collectively, the "**R&D Pre-Funding**").

**6.2. Limited Milestone Payments and Election of Financial Terms for the Initial Licensed Products**.

6.2.1. Limited Milestone Payments. In partial consideration for AGTC's development of the AGTC Technology, prosecution and maintenance of the AGTC Patent Rights and the grant of rights hereunder, and regardless of the election by AGTC under Section 6.2.2 below, Biogen shall pay AGTC the amounts set forth below within forty-five (45) days of receipt of notice from AGTC of the first occurrence of each event described below for each of the first XLRS Product and the first XLRP Product to achieve such event (each, a "**Limited Milestone Payment**").

| | **Event Milestone** | **Limited Milestone Payment for an XLRS Product** | **Limited Milestone Payment for an XLRP Product** |
|---|---|---|---|
| (i) | Initiation of dosing of the first subject in the first FIH Trial of the applicable Initial Licensed Product | N/A | $2,500,000 |
| (ii) | Initiation of dosing of the later of (a) the first subject in the second dose cohort in the first FIH Trial of the applicable Initial Licensed Product or (b) the fourth subject in such FIH Trial | $5,000,000 | $10,000,000 |

Each of the Limited Milestone Payments set forth above shall be payable one time only for each of the XLRS Program and the XLRP Program (regardless of the number of XLRS Products or XLRP Products with respect to which, or the number of times with respect to any XLRS Product or XLRP Product, the specified event milestone occurs). No Limited Milestone Payments shall be payable for any subsequent XLRS Product or XLRP Product regardless of the number of XLRS Products or XLRP Products developed. For clarification, if one XLRS Product or XLRP Product replaces another XLRS Product or XLRP Product in development, such replacement XLRS Product or XLRP Product, as applicable, shall only be subject to Limited Milestone Payments that have not previously been triggered by one or more prior XLRS Products or XLRP Products, as applicable.

6.2.2. AGTC Election. With respect to each Initial Licensed Program, AGTC shall have the right to elect either (a) to share Operating Profits or Losses equally with Biogen and receive Limited Milestone Payments with respect to such Initial Licensed Program (the "**Cost Share Option**"), in which case the provisions of Section 6.3 shall apply and the provisions of Section 6.4 shall not apply with respect to such Initial Licensed Program, or (b) to receive Event Milestone Payments, Sales Milestone Payments and royalty payments with respect to such Initial Licensed Program (the "**Milestone/Royalty Option**"), in which case the provisions of Section 6.4 shall apply and the provisions of Section 6.3 shall not apply with respect to such Initial Licensed Program. AGTC may make such election with respect to an Initial Licensed Program by written notice to Biogen at any time during the period starting upon FIH Trial Completion for the Initial Licensed Product from such Initial Licensed Program, and ending forty-five (45) days after receipt from Biogen of the calculation of all Development Costs reasonably incurred by Biogen which Biogen would expect to be reimbursed under Section 6.3 if AGTC exercised the Cost Share Option. In the event that AGTC fails to make an election with respect to such Initial Licensed Program during the applicable forty-five (45) day period, AGTC shall be deemed to have elected the Milestone/Royalty Option for such Initial Licensed Program. For clarity, (i) after AGTC elects the Cost Share Option or the Milestone/Royalty Option for the XLRS Program, then such election shall apply to all XLRS Products and (ii) after AGTC elects the Cost Share Option or the Milestone/Royalty Option for the XLRP Program, then such election shall apply to all XLRP Products.

**6.3. Cost Share Option**. If AGTC exercises the Cost Share Option as set forth in Section 6.2.2 for an Initial Licensed Program, then effective immediately on the date that AGTC exercises the Cost Share Option in accordance with Section 6.2.2, Biogen and AGTC shall equally share in Operating Profits or Losses for Initial Licensed Products arising under such Initial Licensed Program (each, a "**Cost Share Product**") in the Territory as provided in Exhibit C. In addition, no later than sixty (60) days after AGTC exercises the Cost Share Option for an Initial Licensed Program, AGTC shall reimburse Biogen for [***] of all Development Costs

reasonably incurred by Biogen prior to the exercise of the Cost Share Option in the performance of activities in anticipation of the continued Development of the applicable Cost Share Product(s). Biogen shall prepare and provide to AGTC a first draft of the tax partnership agreement within twenty (20) days after AGTC's exercise of the Cost Share Option and AGTC shall provide any comments to Biogen within twenty (20) days of receipt of such first draft. The Parties shall work together, in good faith and consistent with the terms of this Section 6.3 and Exhibit E, to finalize and execute the tax partnership agreement within sixty (60) days after AGTC's exercise of the Cost Share Option.

**6.4. Milestone/Royalty Option**. If AGTC exercises the Milestone/Royalty Option as set forth in Section 6.2.2 for an Initial Licensed Program, then the provisions of this Section 6.4 shall apply with respect to such Initial Licensed Program.

6.4.1. Event Milestone Payments. In partial consideration for AGTC's development of the AGTC Technology and the grant of rights hereunder, Biogen shall pay AGTC the amounts set forth below within forty-five (45) days of the first occurrence of each event described below for each of the first XLRS Product and the first XLRP Product to achieve such event (each, an "**Event Milestone Payment**").

**Table 6.4.1**

[***]

Each of the Event Milestone Payments set forth above shall be payable one time only for each of the XLRS Program and the XLRP Program (regardless of the number of XLRS Products or XLRP Products with respect to which, or the number of times with respect to any XLRS Product or XLRP Product, the specified event milestone occurs). No Event Milestone Payments shall be payable for any subsequent XLRS Product or XLRP Product regardless of the number of XLRS Products or XLRP Products developed. For clarification, if one XLRS Product or XLRP Product replaces another XLRS Product or XLRP Product in development, such replacement XLRS Product or XLRP Product, as applicable, shall only be subject to Event Milestone Payments that have not previously been triggered by one or more prior XLRS Products or XLRP Products, as applicable.

In the event that an XLRS Product or an XLRP Product bypasses an event milestone and achieves a later event milestone, then upon achievement of the later event milestone, Event Milestone Payments shall be payable both for the event milestone achieved and any earlier event milestone that was bypassed, provided that the provisions of this sentence shall not apply if the respective event milestones are territory-based event milestones (*i.e.*, event milestones (ii) through (x) of Table 6.4.1) and relate to events occurring in different territories (*e.g.*, if an XLRS Product or an XLRP Product bypasses event milestones (ii) and (iii) (which relate to events in the United States) and achieves event milestone (iv) (which relates to events in the European Union), the Event Milestone Payments for event milestones (ii) and (iii) shall not become payable). In the event that an XLRS Product or an XLRP Product achieves more than one event milestone concurrently, then the Event Milestone Payments associated with each such event milestone shall be payable concurrently.

Notwithstanding anything to the contrary in this Agreement, if the JDC or any Regulatory Authority determines that it is necessary, in order to obtain Regulatory Approval of an Initial Licensed Product, to conduct any Additional Clinical Trial with respect to such Initial Licensed Product, then all future Event Milestone Payments for such Initial Licensed Product shall be reduced by [***] of the amount otherwise payable; provided, however, that if AGTC has elected to not share the Development Costs associated with such Additional Clinical Trial equally with Biogen as described in Section 3.2.2(c), all future Event Milestone Payments for such Initial Licensed Product shall be reduced by [***] of the amount otherwise payable.

6.4.2. Sales Milestone Payments. In addition to the Event Milestone Payments described in Section 6.4.1, in consideration of the rights granted to Biogen hereunder, and subject to the terms and conditions of this Agreement, Biogen shall pay AGTC the following one-time payments (each, a "**Sales Milestone Payment**") when aggregate Net Sales of any XLRS Product or any XLRP Product, as applicable, in a Calendar Year in the Territory first reach the respective thresholds indicated below:

[***]

Biogen shall make any Sales Milestone Payment payable with respect to a Calendar Year within sixty (60) days after the end of the applicable Calendar Quarter in which such cumulative Net Sales for such Calendar Year were achieved, and such payment shall be accompanied by a report identifying the applicable Initial Licensed Products, the relevant countries, Net Sales of each Initial Licensed Product for each such country, and the amount payable to AGTC under this Section 6.4.2. In the event that more than one of the previously unmet sales milestones are achieved in a Calendar Year with respect to XLRS Products or XLRP Products, then all of the Sales Milestone Payments corresponding to the sales milestones met in such year shall be owed to AGTC.

6.4.3. Royalty Payments.

(a) *Royalties*. In consideration for the license granted to Biogen under Section 5.1, Biogen, on an Initial Licensed Product-by-Initial Licensed Product and country-by-country basis shall, during the Royalty Term for such Initial Licensed Product, pay to AGTC royalties on Net Sales from the sale of such Initial Licensed Product in any Calendar Year as follows:

[***]

(b) *Fully Paid-Up, Royalty Free License*. Following expiration of the Royalty Term for any Initial Licensed Product in a country, no further royalties shall be payable in respect of sales of such Initial Licensed Product in such country and, thereafter the license granted to Biogen under Section 5.1 with respect to such Initial Licensed Product in such country shall be a fully paid-up, perpetual, exclusive, irrevocable, royalty-free license.

**6.5. Financial Terms for Discovery Products**.

6.5.1. Option Fee. Within forty-five (45) days of the Option Exercise Date for a Discovery Program, Biogen shall pay to AGTC an option fee in the aggregate amount of [***] (the "**Option Fee**").

6.5.2. Discovery Event Milestone Payments. In partial consideration for AGTC's development of the AGTC Technology and the grant of rights hereunder, Biogen shall pay AGTC the amounts set forth below within forty-five (45) days of the first occurrence of each event described below for each of the first Discovery Product in each of Category A, Category B and Category C to achieve such event (each, a "**Discovery Event Milestone Payment**").

Table 6.5.2

[***]

For purposes of this Section 6.5.2:

"Category A" means a Discovery Product in [***], such as [***].

"Category B" means a Discovery Product in [***], such as [***].

"Category C" means a Discovery Product in [***], such as [***].

Each of the Discovery Event Milestone Payments set forth above shall be payable one time only for each Discovery Program (regardless of the number of Discovery Products under such Discovery Program with respect to which, or the number of times with respect to any Discovery Product under such Discovery Program, the specified event milestone occurs). No Discovery Event Milestone Payments shall be payable for any subsequent Discovery Product under the same Discovery Program, regardless of the number of Discovery Products under such Discovery Program developed. If, as a result of Biogen's exercise of the Option for [***] or the designation of a Substitute Discovery Program under Section 4.4, there are Discovery Products from more than one Discovery Program in any of Category A, Category B or Category C, then the Discovery Event Milestone Payments set forth under the applicable category shall be payable for the applicable Discovery Product from each such Discovery Program to achieve the specified event milestone. Notwithstanding the foregoing, if Biogen exercises an Option for a clinical candidate that is a [***] Discovery Product, then each Discovery Event Milestone

Payment shall be payable only once for such [***] Discovery Product. For clarity, if Biogen reinstates a Terminated Discovery Program pursuant to Section 4.4.3, Biogen shall pay the applicable Discovery Event Milestone Payments for both the reinstated Terminated Discovery Program and the applicable Substitute Discovery Program on a going forward basis.

In the event that a Discovery Product bypasses an event milestone and achieves a later event milestone, then upon achievement of the later event milestone, Discovery Event Milestone Payments shall be payable both for the event milestone achieved and any earlier event milestone that was bypassed, provided that the provisions of this sentence shall not apply if the respective event milestones are territory-based event milestones (*i.e.*, event milestones (iii) through (xi) of Table 6.5.2) and relate to events occurring in different territories (*e.g.*, if a Discovery Product bypasses event milestones (iii) and (iv) (which relate to events in the United States) and achieves event milestone (v) (which relates to events in the European Union), the Discovery Event Milestone Payments for event milestones (iii) and (iv) shall not become payable). In the event that a Discovery Product achieves more than one event milestone concurrently, then the Discovery Event Milestone Payments associated with each such event milestone shall be payable concurrently.

6.5.3. Royalty Payments.

(a) *Royalties*. In consideration for the license granted to Biogen under Section 5.1, Biogen, on a Discovery Product-by-Discovery Product and country-by-country basis shall, during the Royalty Term for such Discovery Product, pay to AGTC royalties on Net Sales from the sale of such Discovery Product in any Calendar Year as follows:

[***]

(b) *Fully Paid-Up, Royalty Free License*. Following expiration of the Royalty Term for any Discovery Product in a country, no further royalties shall be payable in respect of sales of such Discovery Product in such country and, thereafter the license granted to Biogen under Section 5.1 with respect to such Discovery Product in such country shall be a fully paid-up, perpetual, exclusive, irrevocable, royalty-free license.

**6.6. Payment Adjustments**. With respect to any payments under Section 6.2.1, Section 6.4 or Section 6.5, the following adjustments shall apply in all cases subject to Section 6.6.4.

6.6.1. Third Party Agreements.

(a) *Biogen Third Party Agreements*. If Biogen licenses during the Term or has prior to the Effective Date licensed, intellectual property rights from one or more Third Parties, in either case, which intellectual property rights are necessary or useful to, and are actually used at any time to, Develop, Manufacture, Commercialize or use any Licensed Product, whether directly or through any Biogen Affiliate or Sublicensee (each, a "**Third Party License**"), then any payments otherwise payable to AGTC under Section 6.2.1, Section 6.4 or Section 6.5 with respect to such Licensed Product shall be reduced by [***] of the payments paid to Third Parties pursuant to any such Third Party Licenses (which, in the case of upfront payments, shall be allocated equitably by Biogen in good faith and proportionately among the applicable Collaboration Programs and other relevant programs of Biogen and its Affiliates) arising out of and directly attributable to the Development, Manufacture, Commercialization or use of any Licensed Product, provided that in no event shall any payment payable to AGTC for any Licensed Product be reduced to less than [***] of the amount that would otherwise be owed to AGTC for such Licensed Product as a result of the application of this Section 6.6.1(a); provided, however, that any amounts paid under a Third Party License that are not used to reduce a payment due hereunder as a result of the foregoing limitations may be carried over to reduce subsequent payments due under Section 6.2.1, Section 6.4 or Section 6.5. In the event that AGTC elects the Cost Share Option under Section 6.2.2 for an Initial Licensed Program for which Biogen has carried-over amounts pursuant to the preceding sentence, such carried-over amounts shall be shared by the Parties in accordance with Section 6.3. Notwithstanding anything to the contrary, in the event that Biogen obtains a direct license from any licensor under an AGTC Third Party Agreement upon termination of such AGTC Third Party Agreement pursuant to Section 16.10, then, if AGTC had been paying all amounts due under such AGTC Third Party Agreement prior to such termination, any payments otherwise payable to AGTC under Section 6.2.1, Section 6.4 or Section 6.5 with respect to a Licensed Product shall be reduced by [***] of the payments paid to Third Parties

pursuant to any such Third Party Licenses arising out of and directly attributable to the Development, Manufacture, Commercialization or use of such Licensed Product without any limitation described in this Section 6.6.1(a).

(b) *AGTC Third Party Agreements*.

(i) AGTC shall be solely responsible for all obligations (including any royalty or other obligations that relate to the AGTC Technology) under the Existing License Agreements and any other agreements with Third Parties that are in effect as of the Effective Date. [***]

(ii) Solely to the extent that Biogen elects to take a sublicense under Section 13.6.2(a) under any license to Third Party IP Rights that AGTC or any of its Affiliates enters into during the Term, Biogen shall be responsible for any payment obligations under the applicable AGTC Third Party Agreements arising out of the Development, Manufacture, Commercialization or use of any Licensed Product, provided that any upfront payments under such AGTC Third Party Agreements shall be allocated [***]. AGTC shall be solely responsible for all other obligations under any such AGTC Third Party Agreements. With respect to any amounts paid by Biogen pursuant to any AGTC Third Party Agreements under this Section 6.6.1(b)(ii), Biogen may offset such amounts against payments due to AGTC in accordance with Section 6.6.1(a), as if such AGTC Third Party Agreements were Third Party Licenses thereunder.

(iii) In the event that the Royalty Term for any Licensed Product extends beyond the tenth (10th) anniversary of the First Commercial Sale of such Licensed Product solely because the manufacture, use, sale, offer for sale or importation of such Licensed Product is Covered by a Valid Claim of an AGTC Patent Right Controlled by AGTC under an AGTC Third Party Agreement that AGTC enters into during the Term, then, for the remainder of the Royalty Term, any royalty payments otherwise payable to AGTC under this Agreement with respect to such Licensed Product shall be reduced to the amount of royalty payments, if any, payable by AGTC to such Third Party pursuant to such AGTC Third Party Agreement with respect to such Licensed Product. Notwithstanding anything to the contrary, this Section 6.6.1(b)(iii) shall not apply in the event that the manufacture, use, sale, offer for sale or importation of such Licensed Product is Covered by a Valid Claim of an AGTC Patent Right Controlled by AGTC under an AGTC Third Party Agreement that AGTC enters into during the Term, but for which AGTC provided all or substantially all of the funding that contributed to the invention Covered by such Valid Claim.

6.6.2. Competitive Products. On a country-by-country and Licensed Product-by-Licensed Product basis, in the event Competitive Products to such Licensed Product are sold in a country in the Territory, any royalty otherwise payable to AGTC under this Agreement with respect to Net Sales of such Licensed Product in such country pursuant to Section 6.4.3 or Section 6.5.3 in all subsequent Calendar Quarters shall be reduced by [***] if Biogen's market share during any two (2) consecutive Calendar Quarters, as measured in either Net Sales or units sold of such Licensed Product, decreases by a total of [***] or more of the average Net Sales or units sold of such Licensed Product in such country averaged over the four (4) Calendar Quarters immediately prior to the first sale of such Competitive Product in such country.

6.6.3. No Valid Claims; Orphan Drug Exclusivity. On a country-by-country and Licensed Product-by-Licensed Product basis, any royalty otherwise payable to AGTC under this Agreement with respect to Net Sales of such Licensed Product in such country shall be reduced by [***] at any time when (a) there is no Valid Claim included in the AGTC Patent Rights or Joint Patent Rights in such country that Covers the Manufacture, use or sale of such Licensed Product and (b) [***].

6.6.4. Reductions Cumulative; Royalty Floor; Event Milestone Floor. The payment reductions set forth in this Section 6.6 shall be applied on a cumulative basis; provided, however, that, except as provided in Section 6.6.1(b)(iii), in no event shall any royalty payment payable to AGTC under this Agreement for any Licensed Product in a given Calendar Quarter be reduced to less than the royalty payments payable by AGTC to Third Parties with respect to such Licensed Product in such Calendar Quarter plus [***]; and provided, further, that in no event shall any Limited Milestone Payment, Event Milestone Payment or Discovery Event Milestone Payment payable to AGTC under this Agreement for any Licensed Product be reduced pursuant to this Section 6.6 (or, with respect to Event Milestone Payments, pursuant to the final paragraph of Section 6.4.1) to less than (a) [***] of the amount otherwise payable to AGTC, if the applicable event milestone

relates to the FIH Trial or any earlier event for such Licensed Product; (b) [***] of the amount otherwise payable to AGTC, if the applicable event milestone relates to (i) the Pivotal Trial for such Licensed Product or (ii) the receipt of Regulatory Approval in the United States for such Licensed Product, in the case of clause (ii), solely to the extent that such Licensed Product is the first Initial Licensed Product to receive Regulatory Approval in the United States; provided, however, in each case ((i) and (ii)), that if AGTC has elected to not share the Development Costs associated with such Additional Clinical Trial equally with Biogen under Section 3.2.2(c), then the floor set forth in this clause (b) shall be further reduced to [***] of the amount otherwise payable to AGTC; or (c) [***] of the amount otherwise payable to AGTC, with respect to any other event milestone for any Licensed Product (including, for clarity, a Regulatory Approval event milestone for a Licensed Product that is not the first Initial Licensed Product to receive Regulatory Approval in the United States).

**6.7. Reports and Payments**.

6.7.1. Cumulative Royalties. Any obligation to pay royalties under this Agreement shall be imposed only once with respect to any sale of any Licensed Product.

6.7.2. Royalty Statements and Payments. Within sixty (60) days of the end of each Calendar Quarter, at any time during the Term in which Biogen is making royalty payments to AGTC for any Licensed Products under Section 6.4.3 or Section 6.5.3, Biogen shall deliver to AGTC a report setting forth for the most recently completed Calendar Quarter, the following information, on a Licensed Product-by-Licensed Product, country-by-country and Territory-wide basis: (a) Net Sales of each such Licensed Product, (b) the basis for any adjustments to the royalty payable for the sale of any such Licensed Product and (c) the royalty due hereunder for the sale of each such Licensed Product. No such reports shall be due for any such Licensed Product before the First Commercial Sale of such Licensed Product. The total royalty due for the sale of all such Licensed Products during such Calendar Quarter shall be remitted at the time such report is made.

**6.8. Taxes and Withholding**.

6.8.1. AGTC shall provide such information and documentation to Biogen as are reasonably requested by Biogen that are necessary for Biogen to determine if any withholding taxes apply to any payments to be made by Biogen to AGTC. Biogen shall only make such withholding payments to the extent required by applicable Law and shall subtract such required withholding payments that are actually paid by Biogen to the appropriate Governmental Authority responsible for the collection of such withholding tax (such a Governmental Authority, a "**Tax Authority**") from the payments due to AGTC. For avoidance of doubt, AGTC shall not be responsible for any interest, penalties or additions to tax attributable to Biogen's failure to timely make any such required withholding payments. Biogen shall promptly submit to AGTC appropriate proof of payment by Biogen to the appropriate Tax Authority of the required withholding taxes. At the request of AGTC, Biogen shall give AGTC such reasonable assistance, which shall include the provision of appropriate certificates of such deductions and withholding payments made, together with other supporting documentation as may be required by the relevant Tax Authority, to enable AGTC to claim exemption from such withholding tax or to obtain a repayment thereof or a reduction thereof, and shall provide such additional documentation from time to time as is reasonably requested by AGTC in connection with any of the foregoing. Biogen shall use commercially reasonable efforts to minimize any such withholdings.

6.8.2. Additional Taxes. The amount of any payment to be made by Biogen to AGTC pursuant to this Agreement shall be increased for any sales, value added or similar taxes (any such taxes, "**Additional Taxes**") required to be collected by AGTC from Biogen. Biogen shall provide such information and documentation to AGTC as are reasonably requested by AGTC for AGTC to determine the amount of any Additional Taxes that apply to any payments to be made by Biogen to AGTC, and to satisfy any applicable reporting obligations related to such Additional Taxes.

6.8.3. The Parties agree that the provisions of this Section 6.8 shall also apply to payments made by AGTC to Biogen, if any, under this Agreement, in which case this Section 6.8 shall be read by replacing all references to "AGTC" with "Biogen" and all references to "Biogen" with "AGTC."

**6.9.** Currency. All payments to be made by a Party to the other Party hereunder shall be in immediately available funds via either a bank wire transfer, an ACH (automated clearing house) mechanism, or any other means of electronic funds transfer, at the payee Party's election, to a bank account to be designated by the payee Party in a notice at least ten (10) days before the payment is due. All amounts payable and calculations under this Agreement shall be in United States Dollars. As applicable, Net Sales and any

royalty deductions shall be translated into United States Dollars at the exchange rate used by Biogen for public financial accounting purposes in accordance with GAAP. If, due to restrictions or prohibitions imposed by national or international authority, payments cannot be made as provided in this Article 6, the Parties shall consult with a view to finding a prompt and acceptable solution, and Biogen will deal with such monies as AGTC may lawfully direct.

**6.10.** Late Payments. If a Party does not receive payment of any sum due to it on or before the due date therefor set forth in this Agreement, simple interest shall thereafter accrue on the sum due to the Party from the due date until the date of payment at a per-annum rate of one percent (1%) over the then-current prime rate reported in *The Wall Street Journal* or the maximum rate allowable by applicable Laws, whichever is lower.

**7. REGULATORY AFFAIRS; PHARMACOVIGILANCE**.

**7.1. Regulatory Affairs.**

7.1.1. XLRS Product.

(a) AGTC will initially own all INDs and Orphan Drug Designations and related documentation submitted to any Regulatory Authorities anywhere in the Territory with respect to the XLRS Product, and the Marketing Application, related documentation and initial Regulatory Approval in the United States with respect to the XLRS Product, subject to Section 3.1.3. AGTC will be primarily responsible, in consultation with Biogen, for (a) all regulatory matters and interactions with Regulatory Authorities relating to the conduct of Clinical Trials for the XLRS Product worldwide including (i) overseeing, monitoring and coordinating all regulatory actions, communications and filings with, and submissions to, Regulatory Authorities with respect to the conduct of Clinical Trials for the XLRS Product; (ii) interfacing, corresponding and meeting with Regulatory Authorities with respect to the conduct of Clinical Trials for the XLRS Product; (iii) submitting and maintaining all regulatory filings with respect to Clinical Trials for the XLRS Product, other than any Marketing Applications, and (iv) maintaining and submitting all records required to be maintained or required to be submitted to any Regulatory Authority with respect to Clinical Trials for the XLRS Product and (b) all other regulatory matters in the United States through Regulatory Approval of the XLRS Product, including (i) overseeing, monitoring and coordinating all regulatory actions, communications and filings with, and submissions to, the FDA with respect to marketing authorization for the XLRS Product; (ii) interfacing, corresponding and meeting with the FDA with respect to the XLRS Product; (iii) submitting and maintaining all regulatory filings with respect to the XLRS Product in the United States; and (iv) maintaining and submitting all records required to be maintained or required to be submitted to the FDA with respect to the XLRS Product, provided that Biogen will have final decision-making authority with respect to any decisions regarding the BLA for the XLRS Product, the content of the label for the XLRS Product or post-marketing commitments with respect to the XLRS Product, subject to Section 2.1.4(a)(v) or Section 2.1.4(b)(ii), as applicable.

(b) Biogen will own all Marketing Applications and related documentation submitted to any Regulatory Authority in the ROW Territory and the initial Regulatory Approval in any country in the ROW Territory with respect to the XLRS Product. Biogen will be primarily responsible, in consultation with AGTC, for all regulatory matters relating to marketing authorizations for the XLRS Product in the ROW Territory, through Regulatory Approval, including (i) overseeing, monitoring and coordinating all regulatory actions, communications and filings with, and submissions to, Regulatory Authorities with respect to marketing authorizations for the XLRS Product in the ROW Territory; (ii) interfacing, corresponding and meeting with Regulatory Authorities with respect to matters relating to marketing authorization for the XLRS Product in the ROW Territory; (iii) submitting and maintaining all regulatory filings with respect to the XLRS Product in the ROW Territory, other than those required to be maintained and submitted by AGTC under Section 7.1.1(a); and (iv) maintaining and submitting all records required to be maintained or required to be submitted to any Regulatory Authority with respect to the XLRS Product in the ROW Territory, other than those required to be maintained and submitted by AGTC under Section 7.1.1(a). AGTC shall provide Biogen with any information regarding the XLRS Program reasonably requested by Biogen in order for Biogen to conduct the activities set forth in this Section 7.1.1(b), and shall provide reasonable support to Biogen with respect to such activities upon Biogen's request, including attending meetings and assisting with responses to inquiries from Regulatory Authorities, and Biogen shall reimburse AGTC for AGTC's reasonable Out-Of-Pocket

Costs associated with such activities within forty-five (45) days of receipt of an invoice from AGTC for such Out-Of-Pocket Costs. Biogen will have final decision-making authority regarding the Marketing Applications in the ROW Territory for the XLRS Product, the content of the label for the XLRS Product and post-marketing commitments with respect to the XLRS Product, subject to Section 2.1.4(a)(v) or Section 2.1.4(b)(ii), as applicable.

7.1.2. XLRP Product.

(a) AGTC will initially own all INDs, Orphan Drug Designations obtained prior to FIH Trial Completion and related documentation submitted to any Regulatory Authorities anywhere in the Territory with respect to the XLRP Product, subject to Section 3.1.3. AGTC will be primarily responsible, in consultation with Biogen, for all regulatory matters and interactions with Regulatory Authorities relating to the conduct of Clinical Trials for the XLRP Product worldwide through FIH Trial Completion, including (i) overseeing, monitoring and coordinating all regulatory actions, communications and filings with, and submissions to, Regulatory Authorities with respect to the conduct of the FIH Trial for the XLRP Product; (ii) interfacing, corresponding and meeting with Regulatory Authorities with respect to the conduct of the FIH Trial for the XLRP Product; (iii) submitting and maintaining all regulatory filings with respect to the FIH Trial for the XLRP Product and (iv) maintaining and submitting all records required to be maintained or required to be submitted to any Regulatory Authority with respect to the FIH Trial for the XLRP Product.

(b) Biogen will own all INDs, Marketing Applications and related documentation submitted to any Regulatory Authority, any Orphan Drug Designations and all Regulatory Approvals with respect to XLRP Products after the transfer in Section 11.5 upon FIH Trial Completion for the XLRP Product. Biogen will be primarily responsible, in consultation with AGTC (and, after FIH Trial Completion for the XLRP Product, shall be solely responsible without consultation with AGTC), for all regulatory matters relating to the XLRP Product, including (i) overseeing, monitoring and coordinating all other regulatory actions, communications and filings with, and submissions to, Regulatory Authorities with respect to the XLRP Product; (ii) interfacing, corresponding and meeting with Regulatory Authorities with respect to the XLRP Product; (iii) submitting and maintaining all regulatory filings with respect to the XLRP Product, other than those required to be maintained and submitted by AGTC under Section 7.1.2(a); and (iv) maintaining and submitting all records required to be maintained or required to be submitted to any Regulatory Authority with respect to the XLRP Product, other than those required to be maintained and submitted by AGTC under this Section 7.1.2(a). AGTC shall provide Biogen with any information regarding the XLRP Program reasonably requested by Biogen in order for Biogen to conduct the activities set forth in this Section 7.1.2(b), and shall provide reasonable support to Biogen with respect to such activities upon Biogen's request, including attending meetings and assisting with responses to inquiries from Regulatory Authorities, and Biogen shall reimburse AGTC for AGTC's reasonable Out-Of-Pocket Costs associated with such activities within forty-five (45) days of receipt of an invoice from AGTC for such Out-Of-Pocket Costs. Biogen shall have final decision-making authority regarding Marketing Applications in the Territory for the XLRP Product, the content of the label for the XLRP Product and post-marketing commitments with respect to the XLRP Product, subject to Section 2.1.4(a)(v) or Section 2.1.4(b)(ii), as applicable.

7.1.3. Participation. Within five (5) Business Days after receipt by either Party of any communication from a Regulatory Authority with respect to an Initial Licensed Product (or such shorter time as necessary to allow the other Party an opportunity to review if the time to respond to such communication is less than five (5) Business Days), such receiving Party will provide the other Party, through its Alliance Manager, with a brief written description of the issues raised in such communication or, if such communication is a substantive communication, a copy of such communication. With respect to any such communications or any filings and other submissions to a Regulatory Authority with respect to an Initial Licensed Product, the receiving or filing Party will allow the other Party a reasonable opportunity, taking into account the nature and length of such communications, filings or submissions (but no less than ten (10) Business Days in the case of significant filings if possible), to review and comment on such Party's proposed response, filings or submissions in advance of the transmission of such response, filing or submission, and such receiving or filing Party will reasonably consider all comments provided by the other Party in connection therewith, provided that, if such filing or other submission relates to the XLRS Product and AGTC is the Party making such filing or other submission, with respect to any comments by Biogen regarding the BLA for the XLRS Product, the content of the label or post-marketing commitments for the XLRS Product, AGTC shall have the obligation to incorporate such comments into any such filing or submission. Each Party shall promptly provide the

other Party with a complete copy of any filing or submission actually submitted to a Regulatory Authority. Each Party shall provide the other Party with reasonable advance notice of any formal meeting or teleconference with any Regulatory Authority with respect to an Initial Licensed Product. The Party having such formal meeting or teleconference shall permit the other Party to have, at such other Party's expense, a representative of such other Party attend such formal meetings or teleconferences. AGTC shall provide Biogen with prompt written notice of (but in any event within ten (10) Business Days) after the occurrence of the filing of any IND for the XLRP Product; provided, however, that in all circumstances, AGTC shall inform Biogen of such event prior to public disclosure of such event by AGTC. Notwithstanding anything to the contrary, AGTC shall have no further participation rights under this Section 7.1.3 (including any rights to receive copies of regulatory communications, filings or submissions) with respect to an Initial Licensed Product if (a) AGTC is no longer conducting Development activities with respect to such Initial Licensed Product and (b) AGTC has not exercised the Cost Share Option for such Initial Licensed Product, subject to Section 2.1.4(a)(v) or Section 2.1.4(b)(ii), as applicable.

7.1.4. Discovery Products. Biogen will own all INDs, Orphan Drug Designations, Marketing Applications and related documentation submitted to any Regulatory Authority and all Regulatory Approvals with respect to the Discovery Products. Biogen will be solely responsible for all regulatory matters relating to the Discovery Products, including (i) overseeing, monitoring and coordinating all regulatory actions, communications and filings with, and submissions to, Regulatory Authorities with respect to the Discovery Products; (ii) interfacing, corresponding and meeting with Regulatory Authorities with respect to the Discovery Products; (iii) submitting and maintaining all regulatory filings with respect to the Discovery Products; and (iv) maintaining and submitting all records required to be maintained or required to be submitted to any Regulatory Authority with respect to the Discovery Products.

**7.2. Pharmacovigilance**. Within one hundred and twenty (120) days following the Effective Date (or as soon as reasonably practicable following such one hundred and twenty (120) day period), the Parties shall enter into a written pharmacovigilance agreement governing each Party's respective obligations with respect to allocation of responsibilities for reporting to the other Party and appropriate Regulatory Authorities adverse events, complaints, and other safety-related matters.

**8. COMMERCIALIZATION**.

**8.1. Control of Commercialization Activities**.

8.1.1. General. As between the Parties, and subject to AGTC's Co-Promotion Option as set forth in Section 8.1.4 and the oversight of the JCC, as applicable, in accordance with Section 2.2, Biogen shall have sole and exclusive responsibility for and control of all aspects of Commercialization of the Licensed Products, including: (i) developing and executing a commercial launch and pre-launch plan, (ii) negotiating with applicable Governmental Authorities regarding the price and reimbursement status of each Licensed Product; (iii) marketing and promotion; (iv) booking sales and distribution and performance of related services; (v) handling all aspects of order processing, invoicing and collection, inventory and receivables; and (vi) providing customer support, including handling medical queries and performing other related functions.

8.1.2. Commercialization Plan and Commercialization Budget. At least [***] prior to the anticipated First Commercial Sale of any Cost Share Product, Biogen shall prepare an initial Commercialization Plan and Commercialization Budget for the following [***] for such Cost Share Product and shall present such initial Commercialization Plan and Commercialization Budget to the JCC for review and approval. Thereafter, Biogen shall amend and update the Commercialization Plan and Commercialization Budget annually on a rolling basis for the following [***].

8.1.3. Biogen Product. Biogen shall have the sole right, either itself or through its Affiliates or Sublicensees, to Commercialize the first Initial Licensed Product to receive Regulatory Approval in the United States (the "**Biogen Product**"), and such Initial Licensed Product shall not be subject to the Co-Promotion Option. Biogen shall have sole and exclusive responsibility for and control of Commercialization of the Biogen Product.

8.1.4. AGTC Co-Promotion Option.

(a) AGTC shall have an option (the "**Co-Promotion Option**") to co-promote in the United States the second Initial Licensed Product to receive Regulatory Approval in the United States (such product for which AGTC has exercised the Co-Promotion Option, the "**Co-Promotion Product**"), in accordance with this Section 8.1.4 and

Exhibit B. AGTC may exercise its Co-Promotion Option by providing written notice to Biogen no later than [***] days prior to the date anticipated for receipt of Regulatory Approval for the second Initial Licensed Product to receive Regulatory Approval in the United States, which date Biogen shall communicate to AGTC no later than [***] days prior to the date anticipated for receipt of such Regulatory Approval, and, in the event Biogen fails to so notify AGTC in a timely manner in accordance with this Section 8.1.4(a), AGTC may exercise the Co-Promotion Option within sixty (60) days of the date that Biogen actually notifies AGTC of the date anticipated for receipt of such Regulatory Approval. No later than three (3) months after AGTC exercises its Co-Promotion Option, AGTC and Biogen shall enter into a co-promotion agreement for the Co-Promotion Product consistent with this Section 8.1.4 and Exhibit B ("**Co-Promotion Agreement**").

(b) Upon AGTC's exercise of the Co-Promotion Option in accordance with this Section 8.1.4, the Parties shall coordinate all sales efforts and field activities with respect to the Co-Promotion Product in the United States under the direction of Biogen, and such efforts and activities shall be more fully described in the Co-Promotion Agreement. Except for those rights expressly granted to AGTC herein and in the Co-Promotion Agreement, Biogen shall have sole and exclusive responsibility for and control of all aspects of Commercialization of the Co-Promotion Product.

(c) AGTC may exercise the Co-Promotion Option regardless of whether AGTC has exercised the Cost Share Option or the Milestone/Royalty Option with respect to the Co-Promotion Product. Solely in the event that AGTC has exercised the Milestone/Royalty Option with respect to the Co-Promotion Product, AGTC shall be responsible for all costs and expenses associated with AGTC's co-promotion activities, including the AGTC Customer-Facing FTEs, for the Co-Promotion Product. For clarity, if AGTC has exercised the Cost Share Option with respect to the Co-Promotion Product, the costs and expenses associated with the Parties' respective Customer-Facing FTEs for the Co-Promotion Product shall be shared by the Parties in accordance with the provisions of Section 6.3.

(d) Notwithstanding anything to the contrary, upon any Change of Control of AGTC, the Co-Promotion Option (if not yet exercised pursuant to Section 8.1.4(a)) or Co-Promotion Agreement, as applicable, shall terminate on the date of such Change of Control or the closing of such acquisition, as applicable. If, on the date of such Change of Control or the closing of such acquisition, as applicable, AGTC has exercised the Co-Promotion Option but the Parties have not yet entered into the Co-Promotion Agreement, Biogen shall have no further obligation to enter into a Co-Promotion Agreement.

(e) If AGTC does not exercise the Co-Promotion Option as set forth in this Section 8.1.4, then Biogen shall have the sole and exclusive responsibility for and control of Commercialization of both Initial Licensed Products.

(f) For clarity, AGTC shall have no right to exercise the Co-Promotion Option if only one Initial Licensed Product receives Regulatory Approval in the United States.

8.1.5. Discovery Products. Biogen shall have the sole and exclusive responsibility for and control of Commercialization of the Discovery Products.

8.1.6. Branding and Marks.

(a) Biogen shall select and own all trademarks and trade dress used in connection with the Commercialization of any and all Licensed Products, including all goodwill associated therewith. Neither AGTC nor its Affiliates shall use or seek to register, anywhere in the world, any trademarks which are confusingly similar to any trademarks, trade names, trade dress or logos developed by or on behalf of Biogen its Affiliates or Sublicensees in connection with any Licensed Product. For purposes of clarity, AGTC have sole responsibility for and shall be free to use any trademark, trade name, trade dress or logos developed for the AGTC Platform, even if the Parties agree to use such trademark, trade name, trade dress or logo on or with respect to a Licensed Product.

(b) Biogen shall apply patent markings that meet all requirements of U.S. law Title 35 of United States Code, including without limitation, 35 U.S.C. §287, with respect to all Licensed Products subject to this Agreement. Biogen shall mark the Licensed Products sold in the United States with all applicable United States patent numbers. All Licensed Products shipped to or sold in other countries shall be marked in such manner as to conform with the patent laws and practice of the country of manufacture or sale. Any Licensed Products subject to Patent Rights under an Existing License Agreement that are sold or produced in the United States shall be Manufactured substantially in the United States to the extent required by applicable Law. Biogen shall take all reasonable action necessary on its part as a licensee of any Patent Rights under an Existing License Agreement to enable the Existing Licensors to satisfy their respective obligations to the United States government under Title 35 of the United States Code.

**8.2**. **Commercialization Costs**. Subject to the provisions of Section 8.1.4(c), Biogen shall be solely responsible for all costs associated with Commercialization of the Licensed Products; provided, however, that if AGTC exercises the Cost Share Option for an Initial Licensed Product as set forth in Section 6.2.2, AGTC shall share such costs as set forth in Section 6.3.

**8.3**. **Commercial Diligence**.

8.3.1. Diligence Obligations. Biogen shall use Commercially Reasonable Efforts to Commercialize a given Licensed Product in each Major Market Country in the Territory where Regulatory Approval is obtained for such Licensed Product, in accordance with the terms of this Agreement and, if such Licensed Product is a Cost Share Product, the Commercialization Plan for such Licensed Product. Solely in the event that the Parties enter into a Co-Promotion Agreement, AGTC shall use Commercially Reasonable Efforts to co-promote the Co-Promotion Product in the United States in accordance with the provisions of the Co-Promotion Agreement.

8.3.2. Exceptions to Commercial Diligence Obligations. Notwithstanding any provision of this Agreement to the contrary, the Obligated Party will be relieved from and will have no obligation to undertake any efforts with respect to the Commercialization of any Licensed Product in the event that:

(a) either Party receives or generates any safety, tolerability or other data reasonably indicating, as measured by the Obligated Party's safety and efficacy evaluation criteria and methodology, that a Licensed Product is not reasonably suitable, for safety reasons, for initiation or continuation of Clinical Trials in humans; or

(b) the other Party materially breaches any of its obligations under the this Agreement and such breach impairs or limits the Obligated Party's ability to perform its Commercialization activities under this Agreement; provided that, in such event, the Obligated Party shall only be relieved of such obligations to the extent and for so long as the other Party's breach so impairs or limits the Obligated Party's ability to perform its Commercialization activities under this Agreement.

**9. RECORDS AND AUDITS**.

**9.1. Research and Manufacturing Records**. Each Party shall maintain, consistent with its then-current internal policies and practices, and cause its Affiliates, Sublicensees, employees and Subcontractors to maintain, consistent with its internal policies and applicable Law, for [***], records and laboratory notebooks, inventory, purchase and invoice records and Manufacturing records with respect to the Licensed Products in sufficient detail and in a good scientific manner appropriate for (i) inclusion in filings with Regulatory Authorities, and (ii) obtaining and maintaining intellectual property rights and protections, including Patent Rights. Such records and laboratory notebooks shall be complete and accurate in all material respects and shall fully and properly reflect all work done, data and developments made, and results achieved. Each Party shall allow, and cause its Affiliates, Sublicensees, employees and subcontractors to allow, the other Party, to the extent necessary for such regulatory or intellectual property protection purposes, inspect or copy such records, subject to redaction by such Party.

**9.2. Financial Records**. Each Party shall keep and shall cause its Affiliates and Sublicensees to keep complete and accurate books and accounts of record (i) used for determination of Development, Manufacturing or Commercialization costs and expenses incurred in connection with the performance of its activities and obligations under this Agreement, and (ii) in connection with the sale of Licensed Products, including without limitation, sales analysis, general ledgers, financial statements, and tax returns, in each case,

in accordance with GAAP and such Party's then-current accounting procedures and in sufficient detail to permit accurate determination of all figures necessary for verification of amounts to be paid under this Agreement or used in the calculation of any cost sharing arrangement, including without limitation, royalties, Sales Milestone Payments, amounts for Cost of Goods Sold. Each Party shall, and shall cause its Affiliates and Sublicensees to, maintain such records for a period of at least six (6) years after the end of the Calendar Quarter in which they were generated.

**9.3. Audits**.

9.3.1. Upon reasonable advance written notice by a Party (the "**Auditing Party**") and not more than once in each Calendar Year (except for cause), the other Party (the "**Audited Party**") and its Affiliates shall permit, and shall use reasonable efforts to cause their Sublicensees to permit, the Auditing Party or licensors of AGTC Technology (or an attorney or CPA of such licensor), or an independent certified public accounting firm of internationally recognized standing, selected by the Auditing Party and reasonably acceptable to the Audited Party, to have access during normal business hours to such of the records of the Audited Party and its Affiliates and, if applicable, their Sublicensees as may be reasonably necessary to (a) verify the accuracy of the applicable royalty or milestone payments hereunder, (b) verify the accuracy of any Development, Manufacturing or Commercialization costs and expenses submitted to the other Party for reimbursement or required for purposes of effecting or managing a cost sharing arrangement under this Agreement or (c) verify the accuracy of any financial information provided by the Audited Party to the Auditing Party under Exhibit C, as applicable, in each case, for any year ending not more than thirty-six (36) months prior to the date of such request. No year may be audited more than once, except for cause. The accounting firm will enter a confidentiality agreement reasonably acceptable to the Audited Party governing the use and disclosure of the Audited Party's information disclosed to such firm, and such firm shall disclose to the Auditing Party only whether the information provided by the Audited Party to the Auditing Party as described in clauses (a) through (c) above was accurate, and the specific details concerning any discrepancies, which information shall be Confidential Information of the Audited Party.

9.3.2. Unless disputed by either Party in good faith, if such accounting firm concludes that any payments paid by a Party to the other Party during the audited period were more or less than the amount actually due, the underpaying Party shall pay any additional amounts due, or the overpaid Party will refund any amounts overpaid, as applicable, in each case plus interest as set forth in Section 6.10, within forty-five (45) days after the date the written report of the accounting firm so concluding is delivered to AGTC and Biogen. The written report will be binding on the Parties absent clear error. The fees charged by such accounting firm shall be paid by the Auditing Party; provided, however, that if the audit results in a payment adjustment of more than five percent (5%), then the Audited Party shall pay the reasonable fees and expenses charged by such accounting firm. The Auditing Party shall treat all financial information disclosed by its accounting firm pursuant to this Section 9.3 as Confidential Information of the Audited Party for purposes of Article 14 of this Agreement.

9.3.3. In the event of a good faith dispute by either Party regarding the result of an audit made pursuant to this Section 9.3, the Parties shall agree in good faith on an alternative independent certified public accounting firm of internationally recognized standing to perform a second audit. If such audit is requested by the Audited Party because the Audited Party was found by the initial audit to have underpaid and the second audit confirms that the Audited Party underpaid, then the Audited Party shall bear all costs associated with the second audit. If such audit is requested by the Auditing Party because the Audited Party was found by the initial audit to have overpaid and the second audit confirms that the Audited Party overpaid, then the Auditing Party shall bear all costs associated with the second audit. Notwithstanding the above, in the event that the second audit confirms the findings of the first audit, the requesting Party shall pay. No over or under payment indicated by the initial audit shall be payable in the event of a dispute until the second audit is complete and such second audit shall be binding on the Parties, with any under or over payment determined thereby, plus interest as set forth in Section 6.10, being payable within forty-five (45) days after the date the written report of the accounting firm so concluding is delivered to both Parties.

**10. REPORTS**.

**10.1. Development Reports.**

10.1.1. JDC Reports and Exchange of Information. During the period of the JDC's authority under Section 2.1.5 with respect to any Collaboration Program, each Party shall keep the JDC informed of the progress of its activities under

each Development Plan (and during any period in which AGTC is Manufacturing Licensed Products under Article 12, its Manufacturing activities), including delivering quarterly written updates of its progress under each Development Plan (and Manufacturing activities, if applicable) to the JDC at least five (5) Business Days in advance of each JDC meeting. Any such updates delivered by AGTC shall include, with respect to any Pre-Funded Activities or Pre-Funded Discovery Activities, as applicable, under such Collaboration Program, (a) the number of FTEs employed with respect to the applicable Collaboration Program, (b) the applicable FTE rates of such FTEs and (c) detailed information with respect to any Clinical Trials that AGTC is conducting for such Collaboration Program, including patient enrollment information and information regarding Clinical Trial sites. In addition, each Party shall provide to the JDC at least ten (10) days in advance of each JDC meeting, summaries of Program Data from such Collaboration Program and any AGTC Technology (including AGTC Improved Technology) or Biogen Technology (including Biogen Platform Improvement Technology), as applicable, arising under such Collaboration Program.

10.1.2. Continuing Reporting Obligations. After the JDC's authority is discontinued with respect to any Collaboration Program pursuant to Section 2.1.5, the Parties shall meet annually until the First Commercial Sale of a Licensed Product under such Collaboration Program to discuss the ongoing Development activities with respect to such Collaboration Program. To the extent practicable, the Parties will combine the annual meetings for some or all such Collaboration Programs. Such annual meetings may be in person or by teleconference. No later than December 31st of each Calendar Year until the First Commercial Sale of a Licensed Product from each Collaboration Program, Biogen shall provide to AGTC a report containing summaries of the following items with respect to such Collaboration Program: (a) a status update with respect to research, pre-clinical, clinical and CMC matters for such Collaboration Program, (b) budget and timeline for the Development activities for such Collaboration Program, (c) status of Development activities with respect to the applicable development milestones for such Collaboration Program, (d) changes in Development assumptions for such Collaboration Program due to new information (*e.g.*, new clinical data or feedback from a Regulatory Authority) and (e) the plan for Development activities for such Collaboration Program for the following year across all relevant functions. Biogen shall consider any input it receives from AGTC regarding the Development activities performed under a Collaboration Program in its sole discretion.

**10.2. Commercialization Reports**.

10.2.1. JCC Reports. Biogen shall prepare and present to the JCC an initial Commercialization Plan and Commercialization Budget for any Cost Share Product in accordance with Section 8.1.2, and shall provide the JCC with updates to such Commercialization Plan and Commercialization Budget in accordance with Section 8.1.2. During such time as the JCC is in existence under Section 2.2, Biogen shall keep AGTC reasonably informed, through the JCC, about the status of Biogen's Commercialization activities with respect to each Cost Share Product by providing, on a quarterly basis, a summary of such activities conducted during the prior Calendar Quarter.

10.2.2. Exchange of Commercialization Information. With respect to (a) the first Initial Licensed Product for which Biogen expects to receive Regulatory Approval in the United States and (b) the second Initial Licensed Product for which Biogen expects to receive Regulatory Approval in the United States, if such Initial Licensed Product is a Co-Promotion Product, provided, in each case ((a) and (b)), that such Initial Licensed Product is not a Cost Share Product, the Parties shall, subject to the last sentence of this Section 10.2.2, meet in person or by teleconference to discuss Commercialization activities with respect to such Initial Licensed Product once every [***] months during the period starting twelve (12) months prior to anticipated First Commercial Sale of such Initial Licensed Product in the United States and ending [***] months after First Commercial Sale of such Initial Licensed Product in the United States. During each such meeting, the Parties shall discuss (i) developing and executing a commercial launch and pre-launch plan for such Initial Licensed Product, (ii) negotiating with applicable Governmental Authorities regarding the price and reimbursement status of such Initial Licensed Product and (iii) marketing and promotion plans and activities, in each case, with respect to such Initial Licensed Product. Notwithstanding the foregoing, this Section 10.2.2 shall not apply in the event that AGTC is not conducting a Pivotal Trial for or commercializing any AAV Product during the period in which the Parties would otherwise conduct the meetings described in this Section 10.2.2. Biogen shall consider AGTC's comments regarding Commercialization of any Initial Licensed Product in its sole discretion.

**11. TECHNOLOGY AND REGULATORY TRANSFERS**

**11.1. Initial Technology Transfer**. Within the time periods set forth in a technology transfer plan to be agreed by the Parties within sixty (60) days after the Effective Date, AGTC shall transfer to Biogen at AGTC's sole expense [***], to the extent not already transferred to Biogen under the Manufacturing Technology Agreement, a true and complete copy as reasonably practicable of (a) data embodying any AGTC Know-How and (b) other tangible embodiments of AGTC Know-How, in each case ((a) and (b)), with respect to the Licensed Products existing on the date of such transfer, in such format as Biogen may reasonably request (including by download of digital files to a secure website or e-room designated and controlled by Biogen, to which AGTC has equivalent access).

**11.2. Ongoing Technology Transfers**. The Parties shall conduct a transfer [***], or more frequently at such time as (a) new material Program Data, Manufacturing-related Development data, AGTC Technology or Biogen Technology, as applicable, comes into a Party's Control or (b) Biogen takes control of Development activities, Manufacturing activities or regulatory activities with respect to a Collaboration Program under any provision of this Agreement, including under Section 3.1.3, Section 3.4, Section 4.7, Section 4.8 or Section 12.1, in each case ((a) and (b)), in accordance with a technology transfer plan, to transfer to the other Party (i) if the transferee Party is Biogen, any Program Data, Manufacturing-related Development data or any and all tangible Know-How within the AGTC Technology (including AGTC Improved Technology and any AGTC Technology that is necessary or useful to enable the Manufacture of a Licensed Product), and (ii) if the transferee Party is AGTC, any and all tangible Know-How within the Biogen Technology (including Biogen Platform Improvement Technology) that is necessary for AGTC to exercise its rights or perform its obligations under this Agreement, in each case ((i) and (ii)), to the extent not already transferred to the transferee Party under this Agreement or the Manufacturing Technology Agreement, and in such format as the transferee Party may reasonably request (including, if the transferee Party is Biogen, by download of digital files to a secure website or e-room designated and controlled by Biogen, to which AGTC has equivalent access). Further, each Party shall make appropriate personnel available to the other Party at reasonable times and places, including by telephone during normal business hours, and upon reasonable prior notice for the purpose of assisting such other Party to understand and use the applicable AGTC Technology or Biogen Technology for the Development, Manufacture, Commercialization and use of Licensed Products in accordance with this Agreement. Any activities under this Section 11.2 shall be conducted at AGTC's sole expense.

**11.3. Transfer of Materials**. To facilitate the conduct of each Collaboration Program, each Party shall provide any Materials required by the applicable Development Plan to be transferred to the other Party and may provide to the other Party certain other Materials. Prior to the commencement by either Party of any Development activities with respect to a Collaboration Program, the other Party shall transfer to such Party, within a reasonable timeframe, all Materials reasonably required and as set forth in the applicable Development Plan in order to conduct such Development activities. Prior to the commencement of Manufacturing of any Licensed Product by Biogen, AGTC shall transfer to Biogen, at Biogen's request, any Materials specific to a Licensed Product and reasonable quantities of Materials that are not specific to a Licensed Product but that are used by AGTC or its Affiliates or Subcontractors in the Manufacture of such Licensed Product that are necessary or useful to enable Biogen to practice its license and rights under this Agreement. Subject to Section 12.1.1 and Section 12.1.2, all Materials shall remain the sole property of the supplying Party, shall be used only in the fulfillment of obligations or exercise of rights under this Agreement and solely under the control of the receiving Party, shall not be used or delivered by the receiving Party to or for the benefit of any Third Party (other than a permitted Subcontractor) without the prior written consent of the supplying Party, and, except with respect to any Materials provided by one Party to the other Party hereunder for use in a Clinical Trial, shall not be used in research or testing involving human subjects, unless expressly agreed. All Materials supplied under this Section 11.3 are supplied "as is", with no warranties of fitness for a particular purpose and must be used with prudence and appropriate caution in any experimental work, since not all of their characteristics may be known. The transfer of Materials under this Section 11.3 shall be conducted at AGTC's expense.

**11.4. Restrictions on Use and Transfer of Materials**. Schedule 11.4 sets forth the Materials to which each of the following restrictions applies. Upon the transfer under Section 11.3 of any Third Party Materials not listed on Schedule 11.4, AGTC will notify Biogen of any restrictions applicable to such Materials.

11.4.1. [***] Biological Materials.

(a) Biogen acknowledges that all rights, title and interest in and to all materials scheduled in the [***] Agreements, together with all progeny, mutants, replicates and derivatives (modified or unmodified) thereof (collectively, the "[***] **Biological Material(s)**") shall be owned solely and exclusively by [***]. For clarity, the [***] Biological Materials do not include (a) any virus produced by AGTC, Biogen, or their respective Affiliates or

sublicensees through the use of the [***] Biological Materials, provided that such virus does not contain any [***] Biological Materials or any functional portion or functional fragment thereof (a "[***] **Virus**") or (b) any product produced by a [***] Virus (a "[***] **Product**").

(b) Biogen acknowledges that AGTC is required to inform [***]of any [***] Biological Material created by Biogen that is different from, and a modification to, the [***] Biological Material listed in part (a) of Schedule 11.4. Biogen shall not use the [***] Biological Material other than in accordance with the rights expressly granted by the applicable [***] Agreement. Biogen shall not sell or otherwise transfer any [***] Biological Material to any Affiliate or Third Party, except in connection with a sublicense granted in accordance with the provisions of this Agreement. The [***] Biological Material shall not be used in humans. All of the [***] Biological Material is experimental in nature and shall be used with prudence and appropriate caution since not all of their characteristics are known. Biogen acknowledges that as between AGTC and [***], all right, title and interest in and to all [***] Viruses, [***] Products, and any intellectual property applying thereto or to the production thereof, shall be owned solely and exclusively by AGTC. For the avoidance of doubt, nothing herein prohibits or is intended to prohibit the use of the [***] Products in humans.

(c) Except as expressly provided herein, nothing in this Agreement will be construed to confer any ownership interest, license or other rights upon Biogen by implication, estoppel or otherwise as to any technology, intellectual property rights, products or biological materials of [***], or any other entity, regardless of whether such technology, intellectual property rights, products or biological materials are dominant, subordinate or otherwise related to any [***] Biological Material or the other Materials listed in part (a) of Schedule 11.4.

(d) Biogen shall not enter into any agreement under which Biogen grants to or otherwise creates in itself or any Affiliate or Third Party a security interest in any [***] Agreement or its rights under any [***] Agreement and any such security interest shall be null and void and of no legal effect. This limitation shall apply to any [***] Biological Material or the other Materials listed in part (a) of Schedule 11.4.

11.4.2. [***]. The use of any [***] listed on part (b) of Schedule 11.4 licensed under the Agreement, dated [***], between AGTC and [***], as amended on [***], and as may be further amended shall be subject to the following terms: (i) Biogen shall only have the right to distribute and license the [***] and (ii) shall be subject to the terms and conditions included in Schedule 11.4.2, which terms and conditions allow for commercial use, despite references to "research purposes only".

11.4.3. [***]. Biogen acknowledges that any [***] listed on part (c) of Schedule 11.4 under the [***] Agreement, dated March 13, 2014 by and between [***] and AGTC, shall at all times remain the property of [***]. With respect to such [***], Biogen may transfer such [***] to its Affiliates or Third Parties to the extent necessary for said Affiliates or Third Parties to Manufacture for Biogen (i) AAV or (ii) the raw materials and components used in connection with the preparation of AAV. Biogen shall provide to AGTC written notification of the identity of any such Third Party that receives such Materials from Biogen along with a certification that such transfer is in compliance with this Section 11.4.3 within thirty (30) days of such transfer.

11.4.4. [***]. Biogen acknowledges and agrees that all direct derivatives and modifications to the [***] listed on part (d) of Schedule 11.4 created by AGTC or Biogen shall be the property of [***] and shall be considered Know-How under [***] referenced in part (d) of Schedule 11.4, provided, however, that all other materials, substances, modifications, cell lines, derivations, progeny created, developed or produced by AGTC as a result of AGTC's research or use of such Know-How shall, as between AGTC and [***], be the property of AGTC, including the intellectual property rights associated therewith.

**11.5. Regulatory Transfers**.

11.5.1. No later than thirty (30) days prior to the anticipated date of Regulatory Approval for the XLRS Product in the United States, the Parties shall finalize a mutually agreed regulatory transfer plan for the XLRS Product, which shall include a timeline for execution of such transfer. Thereafter, AGTC shall, at AGTC's expense, commence transfer to Biogen of ownership of the INDs and Orphan Drug Designations and related filings and documentation for the XLRS Product

submitted to any Regulatory Authority in the Territory, and the Marketing Application and related filings and documentation for the XLRS Product submitted to the FDA and the initial Regulatory Approval for the XLRS Product in the United States in accordance with such regulatory transfer plan, and shall complete such transfer within ninety (90) days after the date of Regulatory Approval for the XLRS Product in the United States, which time period may be extended due to a delay caused by a Regulatory Authority; provided, however, that with respect to any Orphan Drug Designations for the XLRS Product in the ROW Territory, AGTC shall transfer such Orphan Drug Designations to Biogen no later than sixty (60) days after the dosing of the first subject in the Pivotal Trial for the XLRS Product, which time period may be extended due to a delay caused by a Regulatory Authority. Thereafter, Biogen will be the sole owner of all INDs, Orphan Drug Designations, Marketing Applications, Regulatory Approvals and related filings and documentation submitted to any Regulatory Authority before, on or after the date of such transfer with respect to the XLRS Product in all countries in the Territory, to the extent that Biogen was not already the owner of any such INDs, Orphan Drug Designations, Marketing Applications, Regulatory Approvals or related filings and documentation in any country in the Territory. Notwithstanding the foregoing, Biogen may request that AGTC continue any ongoing Clinical Trials and, with respect to any associated regulatory filings or documentation submitted to any Regulatory Authority with respect to any ongoing Clinical Trial that AGTC is continuing at Biogen's request at the time of such transfer, AGTC shall not be obligated to transfer ownership of such regulatory filings or documentation to Biogen until the completion of such Clinical Trial.

11.5.2. No later than thirty (30) days prior to the anticipated date of completion of the Clinical Study Report for such FIH Trial, which Clinical Study Report shall be prepared together by the Parties during the course of the Clinical Trial, for the XLRP Product, the Parties shall finalize a mutually agreed regulatory transfer plan for the XLRP Product under which AGTC shall, at AGTC's expense, commence transfer to Biogen ownership of all INDs, Orphan Drug Designations and related filings and documentation submitted to any Regulatory Authority, and the study database with respect to such FIH Trial, and shall take all steps necessary to effectuate such transfer in accordance with such regulatory transfer plan. Thereafter, subject to AGTC's rights under Section 16.8.1, Biogen will be the sole owner of all INDs, Orphan Drug Designations, Marketing Applications, Regulatory Approvals and related filings and documentation submitted to any Regulatory Authority before, on or after the date of such transfer with respect to the XLRP Product in all countries in the Territory, to the extent that Biogen was not already the owner of any such INDs, Orphan Drug Designations, Marketing Applications, Regulatory Approvals or related filings and documentation in any country in the Territory.

**12. MANUFACTURE AND SUPPLY**.

**12.1. Responsibilities**.

12.1.1. Initial Licensed Products. AGTC shall be solely responsible for Manufacturing clinical supply of each Initial Licensed Product, either itself or through one or more Affiliates or Third Parties, in accordance with the applicable Development Plan, until completion of the FIH Trial for such Initial Licensed Product and any other Clinical Trial that the JDC or any Regulatory Authority determines is required to be conducted prior to the Pivotal Trial for such Initial Licensed Product. Thereafter, Biogen shall be solely responsible for Manufacturing clinical supply of such Initial Licensed Product, either itself or through one or more Affiliates or Third Parties, including the Manufacture of clinical supply of the XLRS Product for use by AGTC in the Pivotal Trial for the XLRS Product. Effective as of the date that Biogen becomes responsible for Manufacture of an Initial Licensed Product in accordance with this Section 12.1.1, AGTC shall, and hereby does, subject to the terms of the Existing License Agreements as set forth in Section 11.4, transfer to Biogen ownership of those portions of the master cell banks delivered to Biogen under Section 11.3 and applicable viral seed stocks and/or master viral banks, reagents and reference standards for such Initial Licensed Product, or, if AGTC does not have ownership of the foregoing Materials, AGTC shall, and hereby does, transfer to Biogen all of AGTC's right, title and interest in and to such Materials. Biogen shall be solely responsible for Manufacturing commercial supply of the Initial Licensed Products, either itself or through one or more Affiliates or Third Parties. In the event that the Parties mutually agree that either Party (or its Affiliate or Subcontractor) will Manufacture clinical supply of any Initial Licensed Products for use by the other Party in Clinical Trials or other Development activities conducted by such other Party under this Agreement, the Parties will discuss in good faith and agree upon reasonable terms, including quality assurance provisions, for such Manufacture and supply.

12.1.2. Discovery Products. Biogen shall be solely responsible for Manufacturing clinical and commercial supply of the Discovery Products, either itself or through one or more Affiliates or Third Party Subcontractors. In the event that the Parties mutually agree that AGTC (or its Affiliate or Third Party Subcontractor) will Manufacture clinical supply of any

Discovery Products for use by Biogen in Clinical Trials or other Development activities conducted by Biogen under this Agreement, the Parties will discuss in good faith and agree upon reasonable terms, including quality assurance provisions, for such Manufacture and supply. Biogen shall, subject to the terms and retained rights included in the Existing License Agreements as set forth in Section 11.4, have sole ownership of those portions of the master cell banks delivered to Biogen under Section 11.3 and applicable viral seed stocks and/or master viral banks, reagents and reference standards for such Discovery Product, or, if AGTC does not have ownership of the foregoing Materials, AGTC shall, and hereby does, transfer to Biogen all of AGTC's right, title and interest in and to such Materials .

**12.2. Costs of Supply**. If, in accordance with Section 12.1.1, AGTC Manufactures and supplies quantities of any Licensed Product other than a Cost Share Product to Biogen, AGTC shall supply such quantities to Biogen at the Cost of Goods Sold for such Licensed Product. Biogen shall make all payments to AGTC on the schedule and in accordance with the terms agreed upon by the Parties under Section 12.1.1. If, in accordance with Section 12.1.1, AGTC Manufactures and supplies quantities of any Cost Share Product to Biogen, then the costs of such Manufacture and supply shall be accounted for in the calculation of Operating Profit or Loss as "Cost of Goods Sold" in accordance with the provisions of Exhibit C. With respect to quantities of the XLRS Product Manufactured by Biogen and supplied to AGTC for use in the Pivotal Trial for the XLRS Product, such quantities shall be supplied at no cost, if AGTC has exercised the Milestone/Royalty Option for the XLRS Product. If AGTC has exercised the Cost Share Option for the XLRS Product, then the costs of such Manufacture and supply shall be accounted for in the calculation of Operating Profit or Loss as "Cost of Goods Sold" in accordance with the provisions of Exhibit C.

**12.3. Requirements regarding Supply and Manufacture**. Each of the Parties agrees that all supply and Manufacture of Licensed Products pursuant to this Agreement, whether by a Party or a Third Party, shall, and shall require its Affiliates and Subcontractors to, when required, comply with all applicable Laws, including applicable cGMP.

**13. INTELLECTUAL PROPERTY**.

**13.1. Ownership of Intellectual Property**.

13.1.1. Invention Disclosure; Ownership of Inventions. During the Term, each Party shall notify the other Party within sixty (60) days of any inventions, developments or discoveries that are made by its or its Affiliates' employees, agents or independent contractors in connection with their activities under this Agreement. Each Party shall own all right, title and interest in and to: (a) any and all inventions, developments or discoveries made solely by its or its Affiliates' employees, agents or independent contractors in connection with their activities under this Agreement; (b) any and all Patent Rights claiming any invention, development or discovery described in clause (a) of this Section 13.1.1; and (c) any and all Know-How embodied by or in any invention, development or discovery described in clause (a) of this Section 13.1.1. Inventorship shall be determined in accordance with United States patent laws.

13.1.2. Ownership of Joint Know-How and Joint Patent Rights. The Parties shall jointly own any Joint Technology, other than any Program Data. Subject to the grant of licenses under Section 5.1 and Section 5.4, the exclusivity provisions of Section 5.8, the reversionary rights under Section 16.8.1 and the Parties' other rights and obligations under this Agreement, each Party shall be free to exploit Joint Patent Rights and Joint Know-How pursuant to the license grant set forth in Section 5.4.1, including granting a license under such Joint Technology without accounting to the other Party in accordance with Section 5.4.1.

13.1.3. Program Data. As between the Parties, Biogen shall own all Program Data, and such Program Data shall be the Confidential Information of Biogen. AGTC, on behalf of itself and its Affiliates, hereby agrees to and does hereby assign to Biogen all of AGTC's and its Affiliates' right, title and interest in and to the Program Data. AGTC will, and will cause its Affiliates to, execute and deliver all requested assignments and other documents, and take such other actions as Biogen may reasonably request, in order to perfect and enforce Biogen's rights in the Program Data. Except as expressly provided in this Agreement, and except in the case AGTC elects to receive a license under Section 16.8.1 in which case AGTC shall be free to publish, use or access any Program Data for the applicable Collaboration Program, neither AGTC, its Affiliates nor any Third Party may publish, use, access or cross reference any Program Data without prior written consent from Biogen, which shall not be unreasonably withheld, conditioned or delayed for any publication or presentation regarding the AGTC Platform, generally.

**13.2. Personnel Obligations**. Each employee, agent or independent contractor (including all Subcontractors) of a Party or its respective Affiliates performing work under this Agreement shall, prior to commencing such work, be bound by invention assignment obligations, including: (i) promptly reporting any invention, discovery, process or other intellectual property right; (ii) presently assigning to the applicable Party or Affiliate all of his or her right, title and interest in and to any invention, discovery, process or other intellectual property; (iii) cooperating in the preparation, filing, prosecution, maintenance and enforcement of any patent or patent application; and (iv) performing all acts and signing, executing, acknowledging and delivering any and all documents required for effecting the obligations and purposes of this Agreement. It is understood and agreed that such invention assignment agreement need not reference or be specific to this Agreement.

**13.3. Patent Representatives**.

13.3.1. Responsibilities; Decision-Making.

(a) Within thirty (30) days of the Effective Date, each Party will appoint a patent representative as the point person to manage that Party's review and comment on (a) Patent Rights being prepared, filed, prosecuted and maintained subject to the provisions in this Article 13 and (b) materials for publications, subject to the provisions in Section 14.6 (the "**Patent Representative**"). Each Party shall be permitted to appoint a new Patent Representative upon written notice to the other Party. The Patent Representatives will meet on a regular basis at a frequency to be agreed from time to time by the Patent Representatives, but no less than twice per year, and will (i) determine by mutual agreement in accordance with the principles set forth in Section 13.3.2 whether intellectual property arising out of activities performed under the Collaboration Programs are Product-Specific Technology, AGTC Improved Technology, Joint Improved Technology, Joint Platform Improvement Technology or Biogen Platform Improvement Technology, (ii) determine by mutual agreement, as required under Section 13.4.2(a)(ii), whether any applicable AGTC Patent Right, Joint Platform Improvement Patent Right or Biogen Platform Improvement Patent Right contains at least one claim that Covers the Development, Manufacture, Commercialization or use of any Licensed Product, (iii) determine by mutual agreement whether intellectual property that comes into the Control of AGTC or its Affiliates during the Term falls within the definition of the AGTC Platform, (iv) determine by mutual agreement to update Schedule 1.22-1, Schedule 1.22-2, Schedule 1.23, Schedule 1.40, Schedule 1.212 or Schedule 4.2.1, (v) coordinate as reasonably necessary or useful to achieve the greatest degree of patent coverage and to avoid creating potential issues in prosecution of the Product-Specific Patent Rights and the applicable AGTC Patent Rights, Biogen Patent Rights and Joint Patent Rights and (vi) facilitate the exchange of information between the Parties in matters related to intellectual property.

(b) In the event the Patent Representatives cannot reach an agreement on any matter to be determined by the Patent Representatives pursuant to this Section 13.3 within thirty (30) days, such dispute shall be escalated to the Alliance Managers for resolution. Following such thirty (30)-day period, either Alliance Manager may elect to obtain an opinion on such matter from an independent outside patent counsel mutually agreed by the Patent Representatives, the costs of which shall be borne equally by the Parties. If either Alliance Manager elects to obtain such an opinion, the Alliance Managers shall consider such opinion, but such opinion shall not be binding on the Parties. If the Alliance Managers are unable to reach agreement with respect to such decision within fifteen (15) days of (i) the date of escalation of the dispute, if neither Alliance Manager elects to obtain an opinion of outside patent counsel or (ii) receipt of the opinion of outside patent counsel, if an Alliance Manager elects to obtain such an opinion, such dispute shall be escalated to the Chief Executive Officer of each Party (or his/her nominee), and such Chief Executive Officers (or their nominees, as applicable) will meet promptly to attempt to resolve the dispute by good faith negotiations. In the event that such dispute is escalated to the CEOs (or their nominees, as applicable), the Alliance Managers shall (x) obtain a non-binding opinion of independent outside patent counsel as set forth in this Section 13.3.1(b), if they have not already obtained such an opinion in accordance with this Section 13.3.1(b), and (y) provide such opinion to the CEOs (or their nominees, as applicable) for their consideration.

13.3.2. Classification of Technology. The Patent Representatives shall adhere to the following principles in conducting their activities and exercising their decision-making authority as set forth in Section 13.3.1:

(a) For the purposes of this Agreement, all provisional patent applications shall include at least one claim.

(b) Subject to the provisions of Section 1.212, a Patent Right shall be classified by reference to its claim(s). No Patent Right may be classified in more than one of the following categories: Product-Specific Patent Rights, AGTC Improved Patent Rights, Joint Improved Patent Rights, Joint Platform Improvement Patent Rights and Biogen Platform Improvement Patent Rights; provided, however, that an initial filing may be classified in more than one category if it includes claims in more than one category, subject to the Parties' obligations under Section 13.3.2(c) with respect to subsequent amendments to such initial filing. With respect to any filing classified in more than one category under the preceding proviso, the Party that made such filing shall have the right to continue prosecution and maintenance of such filing until such time as the Patent Representatives classify the applicable Patent Right in one category.

(c) The Parties shall use reasonable efforts to file the applicable Patent Rights so that all claims under any such Patent Right are in no more than one of the following categories (including, if necessary, amended any initial filing such that all claims are in no more than one of such categories): Product-Specific Patent Rights, AGTC Improved Patent Rights, Joint Improved Patent Rights, Joint Platform Improvement Patent Rights and Biogen Platform Improvement Patent Rights.

**13.4. Filing, Prosecution and Maintenance of Patent Rights**.

13.4.1. Product-Specific Patent Rights.

(a) Biogen shall, at its own expense (subject to Section 13.4.1(d)), prepare, file, prosecute and maintain any Product-Specific Patent Rights, in all countries determined by Biogen. Biogen shall keep AGTC advised on the status of the prosecution of all patent applications included within such Product-Specific Patent Rights and the maintenance of any issued patents included within such Product-Specific Patent Rights. Further, Biogen shall consult and reasonably cooperate with AGTC with respect to the preparation, filing, prosecution and maintenance of such Product-Specific Patent Rights, including: (i) allowing AGTC a reasonable opportunity and reasonable time to review and comment regarding such drafts before any applicable filings are submitted to any relevant patent office or Governmental Authority; and (ii) reflecting any reasonable comments offered by AGTC in any final filings submitted by Biogen to any relevant patent office or Governmental Authority, to the extent such comments are intended to prevent any detrimental effect on the prosecution and maintenance of any Patent Rights owned or Controlled by AGTC, provided that Biogen does not reasonably determine that such comments are detrimental to the prosecution, maintenance or enforcement of any Patent Rights owned or Controlled by Biogen.

(b) If Biogen elects not to file a patent application included in such Product-Specific Patent Rights in any country or elects to cease the prosecution or maintenance of any such Product-Specific Patent Right in any country, then Biogen shall provide AGTC with written notice immediately, but not less than sixty (60) days before any action is required, upon the decision to not file or continue the prosecution of such patent application or maintenance of such Patent Right. In the event Biogen has provided notice to AGTC as described in the preceding sentence, Biogen shall permit AGTC, in AGTC's sole discretion, to file or continue prosecution or maintenance of any such Product-Specific Patent Right in such country at AGTC's expense, [***], and provided, further, that, if AGTC has the right to file or continue prosecution or maintenance of such Product-Specific Patent Right, AGTC shall consult with Biogen with respect to the preparation, filing, prosecution and maintenance of such Product-Specific Patent Rights, including: (i) allowing Biogen a reasonable opportunity and reasonable time to review and comment regarding such drafts before any applicable filings are submitted to any relevant patent office or Governmental Authority, (ii) reflecting any reasonable comments offered by Biogen in any final filings submitted by AGTC to any relevant patent office or Governmental Authority to the extent such comments are intended to prevent any detrimental effect on the prosecution and maintenance of any Patent Rights owned or Controlled by Biogen, provided that AGTC does not reasonably determine that such comments to be detrimental to the prosecution, maintenance or enforcement of any Patent Rights owned or Controlled by AGTC and (iii) not taking any position with respect to such Product-Specific Patent Right that would be reasonably likely to adversely affect the scope, validity or enforceability of any of the other Patent Rights being prosecuted and maintained by Biogen under this Agreement without the prior written consent of Biogen, which consent shall not be unreasonably withheld, conditioned or delayed.

(c) Notwithstanding anything to the contrary, if AGTC Controls any Product-Specific Patent Rights pursuant to any Third Party agreement, then Biogen shall have the rights set forth in this Section 13.4.1 with respect to the prosecution and maintenance of such Product-Specific Patent Rights solely to the extent that AGTC has such rights under such Third Party agreement and is authorized to allow its sublicensees to take control over such rights. If AGTC has limited rights with respect to the preparation, filing, prosecution and maintenance of such Product-Specific Patent Rights under such Third Party agreement (*e.g.*, review and comment rights or the right to control prosecution and maintenance without the right to allow sublicensees to take control over such rights) then AGTC shall, to the extent allowable, pass such rights through to Biogen and permit Biogen to exercise such rights through AGTC with respect to the preparation, filing, prosecution and maintenance of such Product-Specific Patent Rights.

(d) Notwithstanding anything to the contrary, with respect to any Product-Specific Patent Right that Covers or claims a Cost Share Product, the Out-of-Pocket Costs incurred by either Party in connection with the preparation, filing, prosecution and maintenance of such Product-Specific Patent Right under this Section 13.4.1 shall be shared by the Parties in accordance with Section 6.3.

13.4.2. Other Patent Rights.

(a) *AGTC Patent Rights*.

(i) As between the Parties, AGTC shall, at its own expense, prepare, file, prosecute and maintain all AGTC Patent Rights (other than those which are Product-Specific Patent Rights which, for clarity, shall be governed by Section 13.4.1 and AGTC Improved Patent Rights, which, for clarity, shall be governed by Section 13.4.2(b)), Biogen Platform Improvement Patent Rights and Joint Platform Improvement Patent Rights, in all countries determined by AGTC, after consultation with Biogen. AGTC shall keep Biogen advised on the status of the prosecution of all patent applications included within such Patent Rights and the maintenance of any issued patents included within such Patent Rights. Further, AGTC shall consult and reasonably cooperate with Biogen with respect to the preparation, filing, prosecution and maintenance of such Patent Rights, including: (i) allowing Biogen a reasonable opportunity and reasonable time to review and comment regarding such drafts before any applicable filings are submitted to any relevant patent office or Governmental Authority; and (ii) considering in good faith any reasonable comments offered by Biogen in any final filings submitted by AGTC to any relevant patent office or Governmental Authority, to the extent such comments are intended to prevent any detrimental effect on the prosecution and maintenance of any Patent Rights owned or controlled by Biogen.

(ii) If AGTC elects not to file a patent application included in such AGTC Patent Rights, Biogen Platform Improvement Patent Rights or Joint Platform Improvement Patent Rights in any country or elects to cease the prosecution or maintenance of any such Patent Right in any country, if any such Patent Right contains at least one claim that Covers the Development, Manufacture, Commercialization or use of any Licensed Product, as determined by the Patent Representatives in accordance with Section 13.3.1, then AGTC shall provide Biogen with written notice immediately, but not less than thirty (30) days before any action is required, upon the decision to not file or continue the prosecution of such patent application or maintenance of such patent. In the event AGTC has provided notice to Biogen as described in the preceding sentence, AGTC shall permit Biogen, in Biogen's sole discretion, to file or continue prosecution or maintenance of any such Patent Right in such country at Biogen's expense, [***], and provided, further, that, if Biogen has the right to file or continue prosecution or maintenance of such AGTC Patent Right, Biogen shall consult with AGTC with respect to the preparation, filing, prosecution and maintenance of such Patent Rights, including: (a) allowing AGTC a reasonable opportunity and reasonable time to review and comment regarding such drafts before any applicable filings are submitted to any relevant patent office or Governmental Authority, (b) reflecting any reasonable comments offered by AGTC in any final filings submitted by Biogen to any relevant patent office or Governmental Authority and (c) not taking any position with respect to such Patent Right that would be reasonably likely to adversely affect the scope, validity or enforceability of any of the other Patent Rights owned or Controlled by AGTC without the prior written consent of AGTC, which consent shall not be unreasonably withheld.

(iii) Notwithstanding anything to the contrary, with respect to any AGTC Patent Rights that contain at least one claim that Covers the Development, Manufacture, Commercialization or use of any Licensed Product, if AGTC Controls any such AGTC Patent Rights pursuant to any Third Party agreement, then AGTC shall have the obligations and Biogen shall have the rights set forth in this Section 13.4.2(a) with respect to the prosecution and maintenance of such AGTC Patent Rights solely to the extent that AGTC has such rights under such Third Party agreement and is authorized to allow its sublicensees to take control over such rights. If AGTC has limited rights with respect to the preparation, filing, prosecution and maintenance of such AGTC Patent Rights under such Third Party agreement (*e.g.*, review and comment rights or the right to control prosecution and maintenance without the right to allow sublicensees to take control over such rights) then AGTC shall, to the extent allowable, pass such rights through to Biogen and permit Biogen to exercise such rights through AGTC with respect to the preparation, filing, prosecution and maintenance of such AGTC Patent Rights.

(b) *Biogen Patent Rights*.

(i) Biogen shall have the sole right, in Biogen's sole discretion, at its own expense, to prepare, file, prosecute and maintain all Biogen Patent Rights (other than any Product-Specific Patent Rights within such Biogen Patent Rights which, for clarity, shall be governed by Section 13.4.1 and Biogen Platform Improvement Patent Rights, which, for clarity, shall be governed by Section 13.4.2(a)), AGTC Improved Patent Rights and Joint Improved Patent Rights, in any country determined by Biogen and Biogen shall have no obligation to prosecute or maintain any such Patent Right. Biogen shall keep AGTC advised on the status of the prosecution of all patent applications included within the AGTC Improved Patent Rights and the Joint Improved Patent Rights and the maintenance of any issued patents included within such AGTC Improved Patent Rights and the Joint Improved Patent Rights. Further, Biogen shall consult and reasonably cooperate with AGTC with respect to the preparation, filing, prosecution and maintenance of such Patent Rights, including: (i) allowing AGTC a reasonable opportunity and reasonable time to review and comment regarding such drafts before any applicable filings are submitted to any relevant patent office or Governmental Authority; and (ii) considering in good faith any reasonable comments offered by AGTC in any final filings submitted by Biogen to any relevant patent office or Governmental Authority, to the extent such comments are intended to prevent any detrimental effect on the prosecution and maintenance of any Patent Rights owned or controlled by AGTC.

(ii) If Biogen elects not to file a patent application included in such AGTC Improved Patent Rights or Joint Improved Patent Rights in any country or elects to cease the prosecution or maintenance of any such AGTC Improved Patent Right or Joint Improved Patent Right in any country, then Biogen shall provide AGTC with written notice immediately, but not less than thirty (30) days before any action is required, upon the decision to not file or continue the prosecution of such patent application or maintenance of such patent. In the event Biogen has provided notice to AGTC as described in the preceding sentence, Biogen shall permit AGTC, in AGTC's sole discretion, to file or continue prosecution or maintenance of any such AGTC Improved Patent Right or Joint Improved Patent Right in such country at AGTC's expense, [***], and provided, further, that, if AGTC has the right to file or continue prosecution or maintenance of such AGTC Improved Patent Right or Joint Improved Patent Right, AGTC shall consult with Biogen with respect to the preparation, filing, prosecution and maintenance of such Patent Rights, including: (i) allowing Biogen a reasonable opportunity and reasonable time to review and comment regarding such drafts before any applicable filings are submitted to any relevant patent office or Governmental Authority, (ii) reflecting any reasonable comments offered by Biogen in any final filings submitted by AGTC to any relevant patent office or Governmental Authority and (iii) not taking any position with respect to such Patent Right that would be reasonably likely to adversely affect the scope, validity or enforceability of any of the other Patent Rights being prosecuted and maintained by Biogen under this Agreement without the prior written consent of Biogen, which consent shall not be unreasonably withheld.

(c) *Joint Patent Rights*. In the event the Parties make any Joint Know-How (other than Product-Specific Know-How, Joint Platform Improvement Know-How and Joint Improved Know-How), the Patent Representatives

shall promptly meet to discuss and determine whether to seek patent protection thereon. Biogen shall have the first right, but not the obligation, to prepare, file, prosecute and maintain any Joint Patent Right (other than any Product-Specific Patent Rights within such Joint Patent Rights which, for clarity, shall be governed by Section 13.4.1, Joint Platform Improvement Patent Rights which, for clarity, shall be governed by Section 13.4.2(a) and Joint Improved Patent Rights, which, for clarity, shall be governed by Section 13.4.2(b)) throughout the world using patent counsel selected by Biogen and reasonably acceptable to AGTC. Biogen shall give AGTC an opportunity to review the text of any application with respect to such Joint Patent Right before filing, shall consult with AGTC with respect thereto, and shall supply AGTC with a copy of the application as filed, together with notice of its filing date and serial number. Biogen shall keep AGTC reasonably informed of the status of the actual and prospective patent filings (including, without limitation, the grant of any Joint Patent Rights), and shall provide advance copies of any official correspondence related to the filing, prosecution and maintenance of such patent filings. AGTC shall reimburse Biogen for fifty percent (50%) of the reasonable Out-of-Pocket Costs incurred by Biogen in preparing, filing, prosecuting and maintaining such Joint Patent Rights, which reimbursement will be made pursuant to invoices submitted by Biogen to AGTC no more often than once per Calendar Quarter. If either Party (the "**Declining Party**") at any time declines to share in the costs of filing, prosecuting and maintaining any such Joint Patent Right, on a country by country basis, the Declining Party shall provide the other Party (the "**Continuing Party**") with thirty (30) days' prior written notice to such effect, in which event, the Declining Party shall (i) have no responsibility for any expenses incurred in connection with such Joint Patent Right after the end of such thirty (30) day period and (ii) if the Continuing Party elects to continue prosecution or maintenance, the Declining Party, upon the Continuing Party's request, shall execute such documents and perform such acts, at the Continuing Party's expense, as may be reasonably necessary (A) to assign to the Continuing Party all of the Declining Party's right, title and interest in and to such Joint Patent Right and (B) to permit the Continuing Party to file, prosecute and maintain such Joint Patent Right.

**13.5. Enforcement of Patent Rights**.

13.5.1. Notice. If either Biogen or AGTC becomes aware of any potential infringement, anywhere in the world, of any issued Patent Right within the AGTC Patent Rights, the Joint Patent Rights, or any Biogen Patent Rights that are Product-Specific Patent Rights or Biogen Platform Improvement Patent Rights, such Party will promptly notify the other Party in writing to that effect. Any such notice shall include any available evidence to support an allegation of infringement by such Third Party.

13.5.2. Enforcement of Product-Specific Patent Rights, AGTC Improved Patent Rights and Joint Improved Patent Rights. Except as otherwise provided in this Section 13.5.2, Biogen shall have the first right, but not the obligation, to take action to obtain a discontinuance of infringement or bring suit against a Third Party infringer of any Product-Specific Patent Right, AGTC Improved Patent Right or Joint Improved Patent Right. Biogen shall have the right to cause AGTC to join Biogen as a party plaintiff to any such suit, at Biogen's expense, where such joinder is necessary for the enforcement of any such Patent Right. If, after ninety (90) days after the date of notice given pursuant to Section 13.5.1, Biogen has not obtained a discontinuance of infringement of any such Patent Right, filed suit against any such Third Party infringer of such Patent Right or provided AGTC with information and arguments demonstrating to AGTC's reasonable satisfaction that there is insufficient basis for the allegation of such infringement of such Patent Right, then AGTC shall have the right, but not the obligation, to bring suit against such Third Party infringer of such Patent Right with Biogen's prior written consent, which may be withheld in Biogen's sole discretion. If such discontinuance, infringement or suit relates to a Cost Share Product, then the Out-of-Pocket Costs incurred by the Parties in connection with any action brought under this Section 13.5.2 shall be shared by the Parties in accordance with Section 6.3. In all other events, each Party shall bear its own expenses in connection with any action taken by a Party pursuant to this Section 13.5.2. Any recovery obtained by either Party as a result of any proceeding against a Third Party infringer shall be allocated as follows:

(a) Except where the recovery relates to a Cost Share Product (in which event, such recovery shall be shared by the Parties in accordance with Section 6.3), such recovery shall first be used to reimburse each Party pro rata for all litigation costs in connection with such litigation paid by that Party; and

(b) Except where the recovery relates to a Cost Share Product (in which event, such recovery shall be shared by the Parties in accordance with Section 6.3), Biogen shall retain [***] and AGTC shall retain [***] of the remaining portion of any such recovery.

13.5.3. Enforcement of other Biogen Patent Rights. Biogen shall have the sole right, in its sole discretion, to take action to obtain a discontinuance of infringement or bring suit against a Third Party infringer of any Biogen Patent Right (other than any Product-Specific Patent Rights within the Biogen Patent Rights which, for clarity, shall be governed by Section 13.5.2 and other than any Biogen Platform Improvement Patent Right which, for clarity, shall be governed by Section 13.5.4).

13.5.4. Enforcement of other AGTC Patent Rights, Joint Platform Improvement Patent Rights and Biogen Platform Improvement Patent Rights. Except as otherwise provided in this Section 13.5.4, AGTC shall have the sole right, in its sole discretion to take action to obtain a discontinuance of infringement or bring suit against a Third Party infringer of any AGTC Patent Right (other than any Product-Specific Patent Rights or AGTC Improved Patent Rights within the AGTC Patent Rights which, for clarity, shall be governed by Section 13.5.2), Joint Platform Improvement Patent Right or Biogen Platform Improvement Patent Right. AGTC shall have the right to cause Biogen to join AGTC as a party plaintiff to any such suit, at AGTC's expense, where such joinder is necessary for the enforcement of any such Patent Right. In the case of a Third Party infringer developing, manufacturing or commercializing an AAV Product that is competitive to a Licensed Product in the same indication and targeting the same gene (a "**Competitive Infringement**") of any such Patent Right, unless AGTC has notified Biogen that it does not wish to bring such action or does not bring such action within the period of time set by court decree, the Parties shall jointly take action to obtain a discontinuance of infringement or bring suit in a Competitive Infringement. Alternatively, if AGTC has notified Biogen that it does not wish to join such action or does not join within a period of time set by court decree, Biogen may take such action without AGTC in which case Biogen shall have the right to cause AGTC to join Biogen as a party plaintiff in such suit, at Biogen's expense, where joinder is necessary for enforcement of the Patent Right. If the infringement is a Competitive Infringement relating to a Cost Share Product, then the Out-of-Pocket Costs incurred by the Parties in connection with any action brought under this Section 13.5.4 shall be shared by the Parties in accordance with Section 6.3. In all other events, each Party shall bear its own expenses in connection with any action taken by a Party pursuant to this Section 13.5.4. Any recovery obtained by AGTC as a result of any proceeding that is not a Competitive Infringement proceeding shall be retained by AGTC. Any recovery obtained by either Party as a result of any Competitive Infringement proceeding against a Third Party infringer shall be allocated as follows:

(a) Except where the recovery arose out of a Competitive Infringement relating to a Cost Share Product (in which event, such recovery shall be shared by the Parties in accordance with Section 6.3), such recovery shall first be used to reimburse each Party pro rata for all litigation costs in connection with such litigation paid by that Party; and

(b) Except where the recovery arose out of a Competitive Infringement relating to a Cost Share Product (in which event, such recovery shall be shared by the Parties in accordance with Section 6.3), Biogen shall retain [***] and AGTC shall retain [***] of the remaining portion of any such recovery.

13.5.5. Enforcement of other Joint Patent Rights. Except as otherwise provided in this Section 13.5.5, Biogen shall have the first right, but not the obligation, to take action to obtain a discontinuance of infringement or bring suit against a Third Party infringer in a Competitive Infringement of any Joint Patent Right that is not a Product-Specific Patent Right, Joint Improved Patent Right or Joint Platform Improvement Patent Right. Biogen shall have the right to cause AGTC to join Biogen as a party plaintiff to any such suit, at Biogen's expense, where such joinder is necessary for the enforcement of any such Joint Patent Right. If, ninety (90) days after the date of notice given pursuant to Section 13.5.1, Biogen has not obtained a discontinuance of infringement of such Joint Patent Right, filed suit against any such Third Party infringer of such Joint Patent Right or provided AGTC with information and arguments demonstrating to AGTC's reasonable satisfaction that there is insufficient basis for the allegation of such infringement of such Joint Patent Right, then AGTC shall have the right, but not the obligation, to bring suit against such Third Party infringer of such Joint Patent Right. With respect to any infringement of a Joint Patent Right that is not a Product-Specific Patent Right or Joint Platform Improvement Patent Right, where such infringement is not a Competitive Infringement, the Parties shall determine by mutual agreement (a) whether to take action to obtain a discontinuance of infringement or bring suit against a Third Party infringer and (b) which Party shall take control of such action or suit. If the infringement is a Competitive Infringement relating to a Cost Share Product, then

the Out-of-Pocket Costs incurred by the Parties in connection with any action brought under this Section 13.5.5 shall be shared by the Parties in accordance with Section 6.3. In all other events, each Party shall bear its own expenses in connection with any action taken by a Party pursuant to this Section 13.5.5. Any recovery obtained by either Party as a result of any such proceeding against a Third Party infringer shall be allocated as follows:

(a) Except where the recovery arose out of a Competitive Infringement relating to a Cost Share Product (in which event, such recovery shall be shared by the Parties in accordance with Section 6.3), such recovery shall first be used to reimburse each Party for all litigation costs in connection with such litigation paid by that Party; and

(b) Except where the recovery arose out of a Competitive Infringement relating to a Cost Share Product (in which event, such recovery shall be shared by the Parties in accordance with Section 6.3):

(i) if the recovery arose out of a Competitive Infringement proceeding, then Biogen shall retain [***] and AGTC shall retain [***] of the remaining portion of any such recovery; and

(ii) if the recovery arose out of any proceeding that is not a Competitive Infringement proceeding, then the Parties shall share the remaining portion of such recovery equally.

13.5.6. Settlements. With respect to any action, suit, proceeding or claim involving a Patent Right under Section 13.5.2, Section 13.5.4 (solely in the case of a Competitive Infringement) or Section 13.5.5, the enforcing Party shall not consent to the entry of any judgment or enter into any settlement with respect to such an action or suit without the prior written consent of the other Party (which consent shall not unreasonably be withheld or delayed), provided that, if such action or suit relates to the infringement of any Biogen Patent Right that is a Product-Specific Patent Right, Biogen may consent to the entry of any judgment or enter into a settlement with respect to such Biogen Patent Right in Biogen's sole discretion.

13.5.7. Cooperation. Each Party shall cooperate (including by executing any documents required to enable the other Party to initiate such litigation) with the other Party in any suit for infringement of any such Patent Right brought by the other Party against a Third Party in accordance with Section 13.5.2, Section 13.5.4 or Section 13.5.5, and shall have the right to consult with the other Party and to participate in and be represented by independent counsel in such litigation. Neither Party shall incur any liability to the other Party as a consequence of such litigation or any unfavorable decision resulting therefrom, including any decision holding any such Patent Right invalid or unenforceable.

**13.6. Infringement and Third Party Licenses**.

13.6.1. Infringement of Third Party Patents. If the Development, Manufacture, Commercialization or use of any Licensed Product, the practice of any AGTC Patent Right or Joint Patent Right, or the exercise of any other right granted by AGTC to Biogen hereunder (collectively, the "**Licensed Activities**") by Biogen or any of its Affiliates or Sublicensees is alleged by a Third Party to infringe such Third Party's Patent Rights or other intellectual property rights, the Party becoming aware of such allegation shall promptly notify the other Party. Additionally, if either Party determines that, based upon the review of any Third Party Patent Right or other Third Party intellectual property rights, it may be desirable to obtain a license from such Third Party with respect thereto so as to avoid any potential claim of infringement by such Third Party against either Party or their respective Affiliates or Sublicensees, such Party shall promptly notify the other Party of such determination and initiate discussions to determine whether such a license is desirable.

13.6.2. Negotiating Third Party Licenses.

(a) Either Party shall have the right to obtain a license under one or more Patent Rights or other intellectual property rights owned or controlled by a Third Party that are necessary or useful to conduct the Licensed Activities (collectively, "**Third Party IP Rights**"), provided that, (i) if AGTC is the licensee, AGTC is granted a sublicensable license under such Third Party IP Rights permitting AGTC and Biogen and their respective Affiliates and sublicensees to practice such Third Party IP Rights in connection with the Licensed Activities and the performance of any of its obligations or the exercise of any of their respective rights under this Agreement, under terms and conditions that, to the extent applicable to Biogen as a sublicensee of such Third Party IP Rights, are not

more onerous in any material respect on Biogen than those contained in this Agreement and (ii) if Biogen is the licensee, [***], to the extent applicable to AGTC as a sublicensee of such Third Party IP Rights, are not more onerous in any material respect on AGTC than those contained in this Agreement. Upon entry into any such agreement, the contracting Party shall promptly provide a copy of such agreement to the other Party and, in the case where AGTC is the contracting Party, AGTC shall provide Biogen with a proposed allocation of upfront payments contemplated by Section 6.6.1(b)(ii). In the case of any such agreement entered into by AGTC, Biogen may, but shall not be required to, at any time after Biogen receives such copy, elect to take a sublicense to such Third Party IP Rights by notice to AGTC, and thereafter Biogen's payment obligations under Section 13.6.2(b) shall apply, and the Know-How and Patent Rights included in such sublicense shall thereafter be deemed AGTC Technology.

(b) The royalties payable under (a) any such agreement that Biogen enters into with a Third Party or (b) solely to the extent that Biogen has elected to take a sublicense under this last sentence of Section 13.6.2(a), any such agreement that AGTC enters into with a Third Party, shall (i) reduce Biogen's royalty obligations under this Agreement as and to the extent provided in Section 6.6.1, if the applicable Third Party IP Rights Cover or claim the Development, Manufacture, Commercialization or use of any Initial Licensed Product for which AGTC has exercised the Milestone/Royalty Option in accordance with Section 6.2.2 or any Discovery Product or (ii) be shared by the Parties in accordance with Section 6.3, if the applicable Third Party IP Rights Cover or claim the Development, Manufacture, Commercialization or use of any Initial Licensed Product for which AGTC has exercised the Cost Share Option in accordance with Section 6.2.2.

13.6.3. Third Party Infringement Suit. If a Third Party sues Biogen or any of Biogen's Affiliates or Sublicensees (each Person so sued being referred to herein as a "**Sued Party**"), alleging that the Licensed Activities of Biogen or any of Biogen's Affiliates or Sublicensees during the Term and pursuant to this Agreement infringe or will infringe such Third Party's Patent Rights, then, if such suit is an indemnifiable claim under Section 17.3, such suit shall, at Biogen's election, be subject to the indemnification provisions of Article 17. If Biogen does not seek and waives indemnification under Section 17.3 with respect to such suit, or if such suit is not an indemnifiable claim, then Biogen shall have the right to lead the defense of such suit and, upon Biogen's request and in connection with the Sued Party's defense of any such Third Party infringement suit, AGTC shall provide reasonable assistance to the Sued Party for such defense. Biogen shall keep AGTC reasonably informed with respect to any such suit involving an [***] Manufacturing Patent Right, and shall consider AGTC's comments with respect thereto in good faith. All activities under this Section 13.6.3 shall be conducted at the expense of the Party taking any action pursuant to this Section 13.6.3, except that, if the Licensed Activities at issue relate to a Cost Share Product, the Parties shall share any Out-Of-Pocket Costs incurred by either Party in connection with such activities equally in accordance with Section 6.3. With respect to any action, suit, proceeding or claim involving a Third Party Patent Right under this Section 13.6.3, the Sued Party shall not consent to the entry of any judgment or enter into any settlement that imposes a financial obligation on AGTC, or that admits the infringement by AGTC and Biogen of such Third Party Patent Rights or that limits the scope, validity, or enforceability of any of the AGTC Patent Rights, without AGTC's written consent, which consent shall not be unreasonably withheld, conditioned or delayed.

13.6.4. Administrative Actions by Third Parties. Each Party shall promptly notify the other Party in the event of any administrative action involving any AGTC Patent Right, Joint Patent Right or Biogen Patent Right of which it becomes aware, including any nullity, revocation, reexamination, opposition, interference, inter partes and post-grant review or compulsory license proceeding. AGTC shall have the first right, but no obligation, to defend against any such action involving (a) an AGTC Patent Right that is not a Product-Specific Patent Right, (b) a Joint Platform Improvement Patent Right or (c) a Biogen Platform Improvement Patent Right, in each case, in its own name and at its own expense. Upon AGTC's request, Biogen may, in its sole discretion, consent to join, and will join if necessary under applicable Law, in any such action at AGTC's expense and cooperate with AGTC at AGTC's expense. Biogen shall have the first right, but no obligation, to defend against any such action involving (x) an AGTC Patent Right that is a Product-Specific Patent Right or (y) a Joint Patent Right that is not a Joint Platform Improvement Patent Right, and the sole right, but not the obligation, to defend against any such action involving a Biogen Patent Right that is not a Biogen Platform Improvement Patent Right, in each case, in its own name, and any such defense shall be at Biogen's expense, subject to AGTC's indemnification obligations under Article 17. AGTC, upon Biogen's request, may, in its sole discretion, consent to join, and will join if necessary under applicable Law, in any such action at Biogen's expense and cooperate with Biogen at Biogen's expense. If the Party having the first right to defend any action involving an AGTC Patent Right, Joint Patent Right or Biogen Platform Improvement Patent Right fails to defend against any such action within ten (10) days of notice thereof, then the other Party

shall have the right to defend such action, in its own name, and any such defense shall be at such other Party's expense. In such event, the Party having the first right to defend such action shall reasonably cooperate, upon the other Party's request, in any such action at the other Party's expense. In the event of any administrative action under this Section 13.6.4 with respect to a Product-Specific Patent Right that Covers a Cost Share Product, the Parties shall share any Out-Of-Pocket Costs incurred by either Party in connection with such administrative action equally in accordance with Section 6.3.

13.6.5. Paragraph IV Notices. Each Party shall immediately give written notice to the other of any certification of which it becomes aware filed pursuant to any statutory or regulatory requirement in any country in the Territory similar to 21 U.S.C. § 355(b)(2)(A)(iv) or § 355(j)(2)(A)(vii)(IV) (or any amendment or successor statute thereto) claiming that any AGTC Patent Right, Joint Patent Right or Biogen Platform Improvement Patent Right Covering any Licensed Product is invalid or that infringement will not arise from the Development, Manufacture, use or Commercialization in the Territory of such Licensed Product by a Third Party. Upon the giving or receipt of such notice, the provisions of Section 13.5 with respect to division of enforcement responsibilities shall apply, *mutatis mutandis*, with respect to any infringement action against such Third Party. In each case, the Party with the right to bring an infringement action shall notify the other Party at least ten (10) days prior to the date set forth by statute or regulation of its intent to exercise, or not exercise, this right. Any infringement action against a Third Party arising under this Section 13.6.5 shall be governed by the provisions of Section 13.5. Without limiting any provision of Section 13.5, in order to establish standing in connection with any action under this Section 13.6.5, upon the request of the Party bringing the action, the other Party shall reasonably cooperate in any such action at the expense of the Party bring the action and shall timely commence or join in any such action at the request and expense of the Party bringing the action.

**13.7. Patent Term Restoration**. The Parties shall reasonably cooperate with each other in obtaining patent term restoration in any country in the Territory under any statute or regulation equivalent or similar to 35 U.S.C. § 156, where applicable to the AGTC Patent Rights, Joint Patent Rights or Biogen Platform Improvement Patent Rights. If any election with respect to seeking such patent term restoration is to be made in any country in the Territory, then if such election is with respect to an AGTC Patent Right that is not a Product-Specific Patent Right, Joint Platform Improvement Patent Right or Biogen Platform Improvement Patent Right then AGTC shall make such election (including, without limitation, by filing supplementary protection certificates and any other extensions that are now or in the future become available) and if such election is with respect to (a) a Product-Specific Patent Right or (b) a Joint Patent Right that is not a Joint Platform Improvement Patent Right, then Biogen shall make such election (including, without limitation, by filing supplementary protection certificates and any other extensions that are now or in the future become available). In each case, the Party without the right to make such election shall abide by such election and cooperate, as reasonably requested by the Party making such election, in connection with the foregoing (including, without limitation, by providing appropriate information and executing appropriate documents).

**13.8. Recording**. If either Party deems it necessary or desirable for any reason to register or record this Agreement or evidence of this Agreement with any patent office or other appropriate Governmental Authority(ies) in one or more jurisdictions in the Territory, the other Party shall reasonably cooperate to execute and deliver to such Party any documents accurately reflecting or evidencing this Agreement that are necessary or desirable, in such Party's reasonable judgment, to complete such registration or recordation. The registering or recording Party shall reimburse the other Party for all reasonable Out-of-Pocket Costs, including attorneys' fees, incurred by such other Party in complying with the provisions of this Section 13.8.

**14. CONFIDENTIALITY**.

**14.1. Confidentiality**. Except to the extent expressly authorized by this Agreement or otherwise agreed in writing, the Parties agree that, during the Term and for [***] thereafter (or indefinitely with respect to trade secrets), each Party (the "**Receiving Party**") receiving any Confidential Information of the other Party (the "**Disclosing Party**") hereunder shall: (a) keep the Disclosing Party's Confidential Information confidential; (b) not publish, or allow to be published, and shall not otherwise disclose, or permit the disclosure of, the Disclosing Party's Confidential Information in any manner not expressly authorized pursuant to the terms of this Agreement; and (c) not use, or permit to be used, the Disclosing Party's Confidential Information for any purpose other than as expressly authorized pursuant to the terms of this Agreement. Each Party shall be responsible for unauthorized disclosures by its agents, directors, officers, employees, consultants, Affiliates and advisors, and any other Third Party to whom such Party discloses such Confidential Information, regardless of whether such disclosure to such Third Party was permitted. For the avoidance of doubt, (i) AGTC Technology shall be the Confidential Information of AGTC, (ii) Biogen Technology shall be the Confidential Information of Biogen and (iii) Joint Technology shall be the Confidential Information of both Parties, with both Parties deemed to be the Receiving Party.

**14.2. Authorized Disclosure**. Notwithstanding the foregoing provisions of Section 14.1, each Party may disclose Confidential Information belonging to the other Party to the extent such disclosure is reasonably necessary to:

14.2.1. file or prosecute patent applications or regulatory filings as contemplated by this Agreement;

14.2.2. prosecute or defend litigation;

14.2.3. exercise its rights and perform its obligations hereunder, provided that such disclosure is covered by terms of confidentiality at least as restrictive as those set forth herein;

14.2.4. allow such Party to comply with the terms and conditions of any agreements with Third Party licensors of the AGTC Technology or the Biogen Technology, as applicable, provided that such disclosure is covered by terms of confidentiality at least as restrictive as those set forth herein or, with respect to AGTC Technology licensed under an Existing License Agreement, those set forth in the applicable Existing License Agreement disclosed in Schedule 15.2.7; and

14.2.5. comply with applicable Law.

In the event a Party shall deem it reasonably necessary to disclose Confidential Information belonging to the other Party pursuant to this Section 14.2, the Disclosing Party shall to the extent possible give reasonable advance written notice of such disclosure to the other Party and take all reasonable measures to ensure confidential treatment of such information.

**14.3. SEC Filings and Other Disclosures**. Either Party may disclose the terms of this Agreement (a) to the extent required to comply with applicable Law, including the rules and regulations promulgated by the United States Securities and Exchange Commission or any equivalent governmental agency in any country in the Territory, (b) in connection with a prospective acquisition, merger or financing for such Party, to prospective acquirers or merger candidates or to existing or potential investors or financing sources and (c) to any sublicensee, collaborator or potential sublicensee or permitted collaborator of such Party, provided that, in the case of clause (b) or (c), prior to such disclosure each such candidate, investor or financing source shall agree in writing to be bound by obligations of confidentiality and non-use no less restrictive in scope than those set forth in this Article 14; and provided, further, that in the case of clause (a), such Party shall initially submit the redacted version of the Agreement agreed to by the Parties in writing within ten (10) days after the Execution Date with a request for confidential treatment of all of the redacted portions of such attached Agreement. With respect to any subsequent disclosure regarding this Agreement by a Party as required to comply with applicable Law, including the rules and regulations promulgated by the United States Securities and Exchange Commission or any equivalent governmental agency in any country in the Territory (including in response to comments from the Securities and Exchange Commission regarding a request for confidential treatment), such Party shall provide a copy of the intended disclosure to the other Party prior to filing of such disclosure, and the other Party shall have five (5) Business Days (or in the case of a Current Report on Form 8-K, two (2) Business Days) prior to the filing thereof to review such disclosure and provide comments to such Party. Such Party shall implement all reasonable comments provided by the other Party within such period, it being understood that each Party is solely responsible for the accuracy and completeness of all SEC disclosures made by such Party.

**14.4. Residual Knowledge Exception**. Notwithstanding any provision of this Agreement to the contrary, and subject to the terms and conditions of any pre-existing exclusive license granted by either Party to one or more Third Parties, Confidential Information for the purpose of clause (c) of Section 14.1 will not include Residual Knowledge. Any use made by the Receiving Party of Residual Knowledge is on an "as is, where is" basis, with all faults and all representations and warranties disclaimed and at its sole risk. Notwithstanding the foregoing, nothing in this Section 14.4 shall (a) affect the obligations of either Party with respect to confidentiality obligations of Confidential Information under Article 14; (b) constitute, or be deemed to result in, a license under any Technology or other intellectual property right; or (c) affect any other rights or remedies a Party may have under this Agreement or otherwise.

**14.5. Restrictions on Material Non-Public Information**. Each Party acknowledges that it is aware that the United States securities laws prohibit certain Persons who have received material, non-public information with respect to a public company from purchasing or selling securities of that public company and from communicating such information to any other Person under circumstances in which it is reasonably foreseeable that such Person is likely to purchase or sell such securities. Each Party acknowledges that it is familiar with the United States Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder (collectively, the "**1934 Act**"); and agrees that it will neither use, nor cause or permit any person to use, any Confidential Information in contravention of the 1934 Act, including Rule l0b-5 and Rule 14e-3 thereunder, or other applicable securities laws.

**14.6. Public Announcements; Publications**.

14.6.1. Coordination. AGTC and Biogen will, from time to time and at the request of the other Party, discuss the general information content relating to this Agreement that may be publicly disclosed; provided, however, that Biogen shall have no obligation to consult with AGTC with respect to any scientific publication or public announcement concerning Biogen's Development, Manufacture, Commercialization or use of any Licensed Product (except as otherwise expressly set forth in Section 14.6.4).

14.6.2. Announcements. Except as may be expressly permitted under clause (a) of Section 14.3, neither Party will make any public announcement regarding this Agreement without the prior written approval of the other Party. For the sake of clarity, nothing in this Agreement shall prevent Biogen from making any scientific publication or public announcement concerning Biogen's Development, Manufacture or Commercialization activities with respect to any Licensed Product under this Agreement; provided, however, that, except as permitted under Section 14.2, Biogen shall not disclose any of AGTC's Confidential Information in any such publication or announcement without obtaining AGTC's prior written consent to do so and consult with AGTC if such scientific publication or public announcement involves the AGTC Platform. The Parties agree that the Parties intend to jointly release the press release attached hereto as Exhibit D following the Effective Date.

14.6.3. Use of Names. Biogen shall not and shall ensure that its Affiliates and Sublicensees shall not:

(a) use the name or insignia of [***] or the name of any [***] officers, faculty, other researches or students, or any adaptation of such names, in any advertising, promotional or sales literature, including any press release or any document employed to obtain funds, without the prior written approval of [***]; this restriction shall not apply to any information required by law to be disclosed to any governmental entity;

(b) use the names of UFRF, or the University of Florida, nor of any of either institutions employees, agents or affiliates, nor the name of any inventor of Patent Rights under any UFRF Existing License Agreement, nor any adaptation of such names, in any promotional, advertising or marketing materials or any similar form of publicity, or to suggest any endorsement by such entities or individuals, without the prior written approval of UFRF in each case;

(c) use the name of The Johns Hopkins University or the Johns Hopkins Health System or any of its constituent parts, such as the Johns Hopkins Hospital or any contraction thereof or the name of inventors in any advertising, promotional, sales literature or fundraising documents without prior written consent from an authorized representative of JHU; Biogen, Affiliates and Sublicensees shall allow at least seven (7) business days' notice of any proposed public disclosure for JHU's review and comment or to provide written consent; and

(d) use [***] name, the name of any inventor of Patent Rights governed by the [***] Agreement, or the name of [***] in any sales promotion, advertising or any other form of publicity without the prior approval of [***], except as required by Law; should Biogen be required by regulatory or legal requirements to disclose the existence of this Agreement, any of the terms in the [***] Agreement or the names of [***] or [***], [***] shall have thirty (30) days to review (i) redaction of terms, including but not limited to royalty rates, and milestone or other payments, and (ii) the manner in which the names of [***] or [***] are used.

14.6.4. Publications. During the Term, each Party shall submit to the other Party (the "**Non-Disclosing Party**") for review and approval any proposed academic, scientific and medical publication or public presentation related to any Collaboration Program, any Licensed Product or any activities conducted pursuant to this Agreement (including under any Development Plan). In both instances, such review and approval will be conducted for the purposes of preserving the value of the AGTC Technology and the AGTC Platform, the Biogen Technology, the Joint Technology, the rights granted to each Party hereunder and determining whether any portion of the proposed publication or presentation containing the Non-Disclosing Party's Confidential Information should be modified or deleted. Written copies of such proposed publication or presentation required to be submitted hereunder shall be submitted to the Non-Disclosing Party no later than thirty (30) days before submission for publication or presentation. The Non-Disclosing Party shall provide its comments with respect to such publications and presentations within ten (10) Business Days of its receipt of such written copy. The review period may be extended for an additional sixty (60) days in the event the Non-Disclosing Party can demonstrate reasonable need for such

extension including for the preparation and filing of patent applications. Notwithstanding anything to the contrary, the Non-Disclosing Party may require that the other Party redact the Non-Disclosing Party's Confidential Information from any such proposed publication or presentation. AGTC and Biogen will each comply with standard academic practice regarding authorship of scientific publications and recognition of contribution of other parties in any publication. For the sake of clarity, Biogen's obligation to submit any publication to AGTC for review and approval under this Section 14.6.4 shall not apply to any publication which does not contain AGTC's Confidential Information or involve the AGTC Platform or disclose any non-public information included in the AGTC Technology.

**15. REPRESENTATIONS AND WARRANTIES**.

**15.1. Representations and Warranties of Each Party**. Except as may be disclosed in Schedule 15.1, which may be updated within five (5) days following the HSR Clearance Date, each of AGTC and Biogen hereby represents, warrants and covenants to the other Party as of the Execution Date and the Effective Date as follows:

15.1.1. it is a corporation duly organized, validly existing and in good standing under the laws of the state of its incorporation;

15.1.2. it (i) has the requisite power and authority and the legal right to enter into this Agreement and to perform its obligations hereunder, (ii) has the requisite resources and expertise to perform its obligations hereunder and (iii) has taken all requisite action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder;

15.1.3. this Agreement has been duly executed and delivered on behalf of such Party, and constitutes a legal, valid, binding obligation, enforceable against such Party in accordance with its terms;

15.1.4. it has obtained all necessary consents, approvals and authorizations of all Governmental Authorities and other persons or entities required to be obtained by such Party in connection with the execution and delivery of this Agreement;

15.1.5. the execution, delivery and performance by such Party of this Agreement and its compliance with the terms and provisions hereof does not and will not conflict with or result in a breach of any of the terms and provisions of or constitute a default under (i) a loan agreement, guaranty, financing agreement, agreement relating to one or more Patent Rights or other agreement or instrument binding or affecting it or its property; (ii) the provisions of its charter or operative documents or bylaws; or (iii) any order, writ, injunction or decree of any court or Governmental Authority entered against it or by which any of its property is bound;

15.1.6. it has not, and will not, after the Execution Date and during the Term, grant any right to any Third Party that would conflict with the rights granted to the other Party or would be inconsistent with its obligations hereunder;

15.1.7. neither it nor any of its Affiliates has been debarred by the FDA or is the subject of a conviction described in Section 306 of the FD&C Act or is subject to any similar sanction of other Governmental Authorities in the Territory. Neither AGTC nor any of its Affiliates has used, in any capacity, any Person who either has been debarred by the FDA, is the subject of a conviction described in Section 306 of the FD&C Act or is subject to any such similar sanction in their development programs for the Licensed Products. Neither Party shall engage, in any capacity in connection with this Agreement or any ancillary agreements, any Person who either has been debarred by the FDA, is the subject of a conviction described in Section 306 of the FD&C Act or is subject to any such similar sanction. Each Party shall inform the other Party in writing promptly upon learning that it or any Person engaged by such Party or any of its Affiliates who is performing services under this Agreement or any ancillary agreements is debarred or is the subject of a conviction described in Section 306 of the FD&C Act, or, to such Party's knowledge, if any action, suit, claim, investigation or legal or administrative proceeding is pending or is threatened, relating to the debarment or conviction of such Party, any of its Affiliates or any such Person performing services hereunder or thereunder;

15.1.8. it shall at all times comply with all material Laws applicable to its activities under this Agreement; and

15.1.9. each Party hereby agrees that until the expiration of six (6) months after the expiration or termination of this Agreement, neither it nor any of its controlled Affiliates will solicit to employ any of the officers or employees of the other Party without obtaining the prior written consent of the other Party; provided, however, that the foregoing shall not prohibit such Party from: (i) publishing general job advertisements or similar notices that are not targeted specifically at such Party's employees or (ii) soliciting employees whose employment with such Party has terminated not less than six (6) months prior to such solicitation.

**15.2. Additional Representations and Warranties of AGTC**. In addition to the representations, warranties and covenants made by AGTC elsewhere in this Agreement, except as disclosed in Schedule 15.2 as may be updated in accordance with this Section 15.2, AGTC hereby represents, warrants and covenants to Biogen (i) as of the Execution Date and the Effective Date (provided that AGTC may (1) supplement any schedule referred to in this Section 15.2 or (2) add one or more new schedules to this Section 15.2 with respect to the applicable representation and warranty made as of the Effective Date in each case ((1) and (2)) within five (5) days following the HSR Clearance Date, but any such supplement or new schedule may only contain information arising after the Execution Date and may not correct, modify or delete any information set forth in any such schedule on the Execution Date) and (ii) with respect to any Discovery Program, as of the Option Exercise Date for such Discovery Program, solely with respect to (a) AGTC Technology, other than AGTC Technology or Joint Technology already disclosed to Biogen under an existing Collaboration Program or through the Patent Representatives (including Technology covered by the following representations and warranties as of the Effective Date), that is necessary or useful to such Discovery Program and (b) Existing License Agreements and Control Limitation Agreements relating specifically to such Discovery Program, in each case ((a) and (b)), as such AGTC Technology and agreements may be added to existing schedules or set forth in new schedules to this Section 15.2:

15.2.1. the issued AGTC Patent Rights are, to AGTC's Knowledge, valid and enforceable patents. To AGTC's Knowledge, no Third Party is infringing any such Patent Rights in the Field. AGTC has not received written notice challenging the extent, validity or enforceability of such Patent Rights (including by way of example through the institution or written threat of institution of interference, nullity, opposition, inter partes or post grant review or similar invalidity proceedings before the United States Patent and Trademark Office or any analogous foreign Governmental Authority);

15.2.2. (a) Schedule 1.22-1 contains a complete and correct list of all Patent Rights owned or otherwise Controlled by AGTC or its Affiliates (and indicating which entity owns or Controls each Patent Right and which are owned and which are Controlled) that, to AGTC's Knowledge, are necessary or that AGTC believes to be useful for the Development, Manufacture, Commercialization or use of the Initial Licensed Products and (b) Schedule 1.22-2, as of the Option Exercise Date, contains a complete and correct list of all Patent Rights owned or otherwise Controlled by AGTC or its Affiliates (and indicating with entity owns or Controls each Patent Right and which are owned and which are Controlled) that, to AGTC's Knowledge, are necessary or that AGTC believes to be useful or the Development, Manufacture, Commercialization or use of the Discovery Products;

15.2.3. it has, and to its Knowledge, its licensors have, complied in all material respects with all applicable Laws, including, with respect to any issued patents and pending patent applications (excluding United States provisional patent applications), any disclosure requirements of the USPTO or any other Governmental Authority, in connection with the filing, prosecution and maintenance of the AGTC Patent Rights and it has, and to its Knowledge, its licensors have, timely paid all filing and renewal fees payable with respect to any AGTC Patent Rights for which it controls prosecution and maintenance;

15.2.4. it has obtained, or caused its Affiliates, as applicable, to obtain, assignments from the inventors of all inventorship rights to the AGTC Patent Rights that are owned by AGTC or such Affiliates and, to AGTC's Knowledge, there has been no failure on the part of any licensor of AGTC Patents Rights that are licensed by AGTC to obtain assignments from the inventors of all inventorship rights to such licensed Patent Rights, and to AGTC's Knowledge, all assignments of inventorship rights relating to the AGTC Patent Rights are valid and enforceable, and the inventorship of the AGTC Patent Rights owned by AGTC, and, to AGTC's knowledge, of the AGTC Patent Rights licensed to AGTC, is properly identified on each patent or patent application;

15.2.5. other than as expressly permitted by this Agreement, it shall not, and shall cause its Affiliates not to (i) license, sell, assign or otherwise transfer to any Person any AGTC Technology (or agree to do any of the foregoing) or (ii) incur with respect to any AGTC Technology, any lien, encumbrance, charge, security interest, mortgage, liability, grant of

license to Third Parties or other restriction (including in connection with any indebtedness), in each case ((i) and (ii)), other than license grants to Third Parties that do not breach or conflict with the rights and licenses granted to Biogen hereunder;

15.2.6. it has sufficient right, power and authority to grant all of the right, title and interest in the licenses granted or to be granted to Biogen or Biogen's Affiliates under this Agreement;

15.2.7. there are no AGTC Third Party Agreements, other than the Existing License Agreements expressly disclosed on Schedule 15.2.7, true and complete copies of which have been provided to Biogen and, to AGTC's Knowledge, other than as disclosed on Schedule 1.22-1 or Schedule 1.22-2 or as set forth in such Existing License Agreements, no Third Party has any right, title or interest in or to, or any license under, any of the AGTC Patent Rights or AGTC Know-How;

15.2.8. except as provided in the Existing License Agreements and other than as disclosed on Schedule 1.22-1 or Schedule 1.22-2, AGTC is the sole and exclusive owner of the AGTC Patent Rights listed on Schedule 1.22-1 or Schedule 1.22-2, all of which are free and clear of any liens, charges and encumbrances other than licenses granted to Third Parties that do not breach or conflict with the rights and licenses granted to Biogen hereunder;

15.2.9. to AGTC's Knowledge, it has the right to use, and to permit Biogen, Biogen's Affiliates and Biogen's Sublicensees to use, the AGTC Know-How for all permitted purposes under this Agreement;

15.2.10. the AGTC Know-How is free and clear of liens, charges or encumbrances other than licenses granted to Third Parties that do not breach or conflict with the rights and licenses granted to Biogen hereunder;

15.2.11. it and its Affiliates have taken commercially reasonable measures consistent with industry practices to protect the secrecy, confidentiality and value of all AGTC Know-How that constitutes trade secrets under applicable Law (including requiring all employees, consultants and independent contractors to execute binding and enforceable agreements requiring all such employees, consultants and independent contractors to maintain the confidentiality of such AGTC Know-How) and, to AGTC's Knowledge, such AGTC Know-How has not been used, disclosed to or discovered by any Third Party except pursuant to such confidentiality agreements and there has not been a breach by any party to such confidentiality agreements;

15.2.12. except as provided in the Existing License Agreements, no AGTC Technology existing as of the Execution Date or the Effective Date is subject to any funding agreement with any government or governmental agency;

15.2.13. to AGTC's Knowledge, the manufacture, use, sale, offer for sale, supply or importation by AGTC or Biogen (or their respective Affiliates or Sublicensees) of any Licensed Product does not and will not infringe any issued patent of any Third Party.

15.2.14. it has not received notice of any claims, and there are no judgments or settlements against or owed by AGTC or, to AGTC's Knowledge, any pending or threatened claims or litigation, in each case relating to the AGTC Technology;

15.2.15. it and its Affiliates are, and shall remain, in compliance in all material respects with any AGTC Third Party Agreements;

15.2.16. it will not without Biogen's written consent, amend any AGTC Third Party Agreement in a manner that materially adversely affects the rights granted to Biogen hereunder or AGTC's ability to fully perform its obligations hereunder;

15.2.17. it will furnish Biogen with copies of all notices received by AGTC relating to any alleged breach or default by AGTC that would give rise to a termination right under AGTC Third Party Agreements no later than ten (10) days after AGTC's receipt thereof. In the event AGTC does not resolve any such alleged breach or default, it shall notify Biogen within a sufficient period of time before the expiration of the cure period for such breach or default under such AGTC Third Party Agreement such that Biogen, in its sole discretion, is able to cure or otherwise resolve such alleged breach or default. Biogen shall have the right, but not the obligation, to cure or otherwise resolve any such alleged breach or default, including

making any required payments under such AGTC Third Party Agreement on AGTC's behalf. If Biogen makes any payments to a Third Party in connection with the cure or other resolution of such alleged breach or default of AGTC, then, notwithstanding anything to the contrary in this Agreement, Biogen may credit the full amount of such payments against any royalties or other payments payable to AGTC pursuant to this Agreement;

15.2.18. it will promptly furnish Biogen with copies of all (i) amendments of the AGTC Third Party Agreements and (ii) correspondence with or from licensors under the AGTC Third Party Agreements to the extent material to Biogen or the rights granted to Biogen or Biogen's Affiliates under this Agreement;

15.2.19. all terms and conditions of the Existing License Agreements applicable to Biogen in its role as a sublicensee or otherwise required to be included in sublicense agreements under such Third Party Agreements are expressly set forth in this Agreement;

15.2.20. neither AGTC nor any of its Affiliates are a party to or otherwise subject to any Control Limitation Agreement with respect to any Technology that would, but for such Control Limitation Agreement, be included in the rights licensed or assigned to Biogen or its Affiliates pursuant to this Agreement with respect to (a) the Initial Licensed Products, as of the Execution Date and the Effective Date or (b) a Discovery Product, as of the Option Exercise Date for such Discovery Product; and

15.2.21. there is no action, claim, demand, suit, proceeding, arbitration, grievance, citation, summons, subpoena, inquiry or investigation of any nature, civil, criminal, regulatory or otherwise, in law or in equity, pending or, to AGTC's Knowledge, threatened, with any judicial or arbitrative body against AGTC or any of its Affiliates in connection with the AGTC Technology, the Discovery Programs or the Licensed Products.

**15.3. Additional Covenant and Representation of Biogen**.

15.3.1. In addition to the representations, warranties and covenants made by Biogen elsewhere in this Agreement, Biogen hereby covenants to AGTC that Biogen shall not encumber, other than under sublicenses as expressly permitted under this Agreement, or otherwise grant a security interest in, any of the AGTC Technology to any Third Party.

15.3.2. Biogen represents and warrants that it will comply, and will ensure that its Affiliates comply, with all local, state and international laws and regulations relating to the [***] Biological Material and to the development, manufacture, use, sale and importation of [***] Viruses and [***] Products. Without limiting the foregoing, Biogen represents and warrants that it will comply with all United States export control laws and regulations with respect to [***] Biological Material and any [***] Viruses and [***] Products developed or made through the use thereof.

**15.4. Special Exceptions for Licensors Under Existing License Agreements**. Notwithstanding anything to the contrary in this Agreement, nothing in this Agreement shall be construed as:

15.4.1. a warranty or representation by UFRF as to the validity or scope of any right included in the AGTC Patent Rights licensed under the UF/JHU Agreement;

15.4.2. a warranty or representation that anything made, used, sold or otherwise disposed of under the license granted in the UF/JHU Agreement will or will not infringe patents of Third Parties;

15.4.3. an obligation to bring or prosecute actions or suits against Third Parties for infringement of AGTC Patent Rights granted in the UF/JHU Agreement;

15.4.4. an obligation to furnish any Know-How not provided in AGTC Patent Rights granted in the UF/JHU Agreement or any services other than those specified in the UF/JHU Agreement; or

15.4.5. a warranty or representation by UFRF that it will not grant licenses to others to make, use or sell products not covered by the claims of the AGTC Patent Rights granted in the UF/JHU Agreement which may be similar and/or compete with products made or sold by Biogen.

**15.5. UFRF Disclaimer**. EXCEPT AS OTHERWISE EXPRESSLY SET FORTH IN THE UF/JHU AGREEMENT, UFRF MAKES NO REPRESENTATIONS AND EXTENDS NO WARRANTIES OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING BUT NOT LIMITED TO WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, AND VALIDITY OF PATENT RIGHTS CLAIMS, ISSUED OR PENDING. UFRFASSUMES NO RESPONSIBILITIES WHATSOEVER WITH RESPECT TO USE, SALE, OR OTHER DISPOSITION BY BIOGEN, ITS SUBLICENSEE(S), OR THEIR VENDEES OR OTHER TRANSFEREES OF PRODUCTS INCORPORATING OR MADE BY USE OF INVENTIONS LICENSED UNDER SUCH AGREEMENT.

**15.6. Duties of the Parties**. None of the licensors under the UF/JHU Agreement are commercial organizations. They are institutes of research and education. Therefore, such licensors have no ability to evaluate the commercial potential of any AGTC Patent Rights or processes or other license or rights granted in such Agreement. It is therefore incumbent upon Biogen to evaluate the rights and products in question, to examine the materials and information provided by such licensors, and to determine for itself the validity of any AGTC Patent Rights or processes licensed under such Agreement, its freedom to operate, and the value of any such AGTC Patent Rights or processes or other rights granted.

**15.7. Representations by JHU**. JHU has represented to AGTC that it has good and marketable title to its interest in the inventions claimed under AGTC Patent Rights licensed under the UF/JHU Agreement with the exception of certain retained rights of the United States government, which may apply if any part of the JHU research was funded in whole or in part by the United States Government. JHU does not warrant the validity of any patents or that practice under such patents shall be free of infringement. EXCEPT AS EXPRESSLY SET FORTH IN THIS SECTION 15.7, BIOGEN, AND BIOGEN'S AFFILIATES AND SUBLICENSEE(S) AGREE THAT THE AGTC PATENT RIGHTS LICENSED UNDER THE UF/JHU AGREEMENT ARE PROVIDED "AS IS", AND THAT JHU MAKES NO REPRESENTATION OR WARRANTY WITH RESPECT TO THE PERFORMANCE OF SUCH LICENSED PRODUCT(S) AND LICENSED PROCESSES INCLUDING THEIR SAFETY, EFFECTIVENESS, OR COMMERCIAL VIABILITY. JHU DISCLAIMS ALL WARRANTIES WITH REGARD TO SUCH PRODUCT(S) AND PROCESSES(S) LICENSED UNDER THE UF/JHU AGREEMENT, INCLUDING, BUT NOT LIMITED TO, ALL WARRANTIES, EXPRESSED OR IMPLIED, OF MERCHANTABILITY AND FITNESS FOR ANY PARTICULAR PURPOSE. NOTWITHSTANDING ANY OTHER PROVISION OF THIS AGREEMENT, JHU ADDITIONALLY DISCLAIMS ALL OBLIGATIONS AND LIABILITIES ON THE PART OF JHU AND INVENTORS, FOR DAMAGES, INCLUDING, BUT NOT LIMITED TO, DIRECT, INDIRECT, SPECIAL AND CONSEQUENTIAL DAMAGES, ATTORNEYS' AND EXPERTS' FEES, AND COURT COSTS (EVEN IF JHU HAS BEEN ADVISED OF THE POSSIBILITY OF SUCH DAMAGES, FEES OR COSTS), ARISING OUT OF OR IN CONNECTION WITH THE MANUFACTURE, USE, OR SALE OF THE PRODUCT(S) AND PROCESSES LICENSED UNDER THIS AGREEMENT. BIOGEN, AND BIOGEN'S AFFILIATES AND SUBLICENSEE(S) ASSUME ALL RESPONSIBILITY AND LIABILITY FOR LOSS OR DAMAGE CAUSED BY A PRODUCT AND/OR PROCESS MANUFACTURED, USED, OR SOLD BY LICENSEE, ITS SUBLICENSEE(S) AND AFFILIATED COMPANIES WHICH IS A LICENSED PRODUCT(S) OR LICENSED PROCESSES AS DEFINED IN THE UF/JHU AGREEMENT.

**15.8. Representation by Legal Counsel**. Each Party hereto represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption shall exist or be implied against the Party which drafted such terms and provisions.

**15.9. Disclaimer**. THE FOREGOING WARRANTIES OF EACH PARTY ARE IN LIEU OF ANY OTHER WARRANTIES, EXPRESS OR IMPLIED, INCLUDING ANY IMPLIED WARRANTIES OF MERCHANTABILITY OR ANY IMPLIED WARRANTIES OF FITNESS FOR A PARTICULAR PURPOSE, ALL OF WHICH ARE HEREBY SPECIFICALLY EXCLUDED AND DISCLAIMED.

**15.10. No Guarantee of Success**. Biogen and AGTC acknowledge and agree that nothing in this Agreement will be construed as representing any estimate or projection of (a) the successful Development or Commercialization of any Licensed Product under this Agreement, (b) the number of Licensed Products that will or may be successfully Developed or Commercialized under this Agreement, (c) anticipated sales or the actual value of any Licensed Products that may be successfully Developed or Commercialized under this Agreement or (d) the damages, if any, that may be payable if this Agreement is terminated for any reason. Neither Party makes any representation, warranty or covenant, either express or implied, that (i) it will successfully Develop, Manufacture, Commercialize or, other than is expressly required under Section 3.3.1, Section 4.3.1 or 8.3.1, continue to Commercialize any Licensed Product in any country, (ii) if Commercialized, that any Licensed Product will achieve any particular sales level, whether in

any individual country or cumulatively throughout the Territory or (iii) other than is expressly required under Section 3.3.1, Section 4.3.1 and Section 8.3.1, that either Party will devote, or cause to be devoted, any level of diligence or resources to Developing or Commercializing any Licensed Product in any country, or in the Territory in general.

**16. GOVERNMENT APPROVALS; TERM AND TERMINATION**.

**16.1. HSR Filing and Clearance**. Subject to the terms hereof, AGTC and Biogen agree to cooperate and to use their respective reasonable best efforts to obtain any government clearances or approvals, or expirations or terminations of waiting periods, required for the consummation of the transactions contemplated under this Agreement under the HSR Act, the Sherman Antitrust Act, as amended, the Clayton Act, as amended, the Federal Trade Commission Act, as amended, and any other federal, state or foreign law or, regulation or decree designed to prohibit, restrict or regulate actions for the purpose or effect of monopolization or restraint of trade (collectively "**Antitrust Laws**"), and to respond to any government requests for information under any Antitrust Law. The Parties will consult and cooperate with one another, and consider in good faith the views of one another, in connection with any analyses, appearances, presentations, memoranda, briefs, arguments, opinions and proposals made or submitted by or on behalf of any party hereto in connection with proceedings under or relating to any Antitrust Law. Biogen, in consultation with AGTC, shall be entitled to direct any proceedings or negotiations with any governmental entity relating to any of the foregoing, provided that it shall afford AGTC and its counsel a reasonable opportunity to participate therein. Except as prohibited by applicable Law, each Party shall keep the other party and/or its counsel informed of any substantive communication received by such party from, or given by such party to any governmental entity, in each case regarding any of the transactions contemplated hereby; and permit the other party and/or its counsel to review any substantive communication given by it to, and consult with each other in advance of any meeting or conference with, any such governmental entity. Without limiting the generality of the foregoing, each of AGTC and Biogen shall, within ten (10) days after the Execution Date (or such later time as may be agreed to in writing by the Parties) file with the United States Federal Trade Commission and the Antitrust Division of the United States Department of Justice any HSR Filing required of it under the HSR Act in the reasonable opinion of either Party with respect to the transactions contemplated hereby. The Parties shall cooperate with one another to the extent necessary in the preparation of any such HSR Filing. Each Party shall be responsible for its own costs, expenses, and filing fees associated with any HSR Filing; provided, however, that Biogen shall be solely responsible for any fees (other than penalties that may be incurred as a result of actions or omissions on the part of AGTC) required to be paid to any governmental agency in connection with making any such HSR filing for acquisitions by Biogen hereunder. In the event the United States Federal Trade Commission or the United States Department of Justice seeks a preliminary injunction under the HSR Act against AGTC and Biogen to enjoin the transactions contemplated by this Agreement, Biogen shall have the first right, but not the obligation, to defend against such preliminary injunction, at Biogen's cost and expense, in consultation with AGTC. If Biogen has not obtained a discontinuance of such injunction within sixty (60) days of submitting the HSR Filing or if Biogen does not to pursue such discontinuance, AGTC shall have the right, but not the obligation, to take over such defense, at AGTC's cost and expense, in consultation with Biogen.

**16.2. Termination Upon HSR Denial**. In the event that the Parties make an HSR Filing under Section 16.1, this Agreement shall terminate (a) at AGTC's option, immediately upon notice to Biogen, in the event that the United States Federal Trade Commission or the United States Department of Justice seeks a preliminary injunction under the HSR Act against AGTC and Biogen to enjoin the transactions contemplated by this Agreement, provided that Biogen is not pursuing a discontinuance of such injunction under Section 16.1, (b) at the election of either Party, immediately upon notice to the other Party, in the event that the United States Federal Trade Commission or the United States Department of Justice obtains a preliminary injunction under the HSR Act against AGTC and Biogen to enjoin the transactions contemplated by this Agreement or (c) at the election of either Party, immediately upon notice to the other Party, in the event that the HSR Clearance Date shall not have occurred on or prior to one hundred eighty (180) days after the effective date of the HSR Filing.

**16.3. Other Government Approvals**. Each of AGTC and Biogen shall cooperate with the other Party to make all registrations, filings and applications, to give all notices and to obtain as soon as practicable all governmental or other consents, transfers, approvals, orders, qualifications authorizations, permits and waivers, if any, and to do all other things necessary or desirable for the consummation of the transactions as contemplated hereby.

**16.4. Term**. With respect to any Collaboration Program, the term of this Agreement will commence (a) with respect to any Collaboration Program other than a Substitute Discovery Program, on the Effective Date, or (b) with respect to a Substitute Discovery Program, on the Discovery Program Substitution Date and, in each case ((a) and (b)), shall extend on a Collaboration Program-by-Collaboration Program, country-by-country, or product-by-product basis, as applicable, unless this Agreement with respect to a

Collaboration Program, country or product under such Collaboration Program is terminated earlier (in accordance with this Article 16 or with respect to a Discovery Program, if earlier, on the date such Discovery Program becomes an Abandoned Program), until the later of (i) with respect to each Licensed Product that is not a Cost Share Product, the last to expire of any Royalty Term for such Licensed Product in such country in the Territory or (ii) with respect to each Cost Share Product, the date that Biogen is no longer Developing or Commercializing such Cost Share Product in such country in the Territory (the "**Term**").

**16.5. Termination by AGTC**.

16.5.1. Termination for Breach by Biogen. In the event that Biogen commits a material breach of its obligations under this Agreement and such material breach remains uncured for [***] days (or in the case of non-payment that constitutes a material breach, [***] days), measured from the date written notice of such material breach is given to Biogen, AGTC may, in its sole discretion, terminate this Agreement either for cause in its entirety or on a Collaboration Program-by-Collaboration Program or Licensed Product-by-Licensed Product basis with respect to the Collaboration Programs or Licensed Products to which such material breach directly relates, in each case, in one or more countries in the Territory, at any time during the Term after such [***] day period (or [***] day period in the case of non-payment), by giving written notice to Biogen; provided, however, that if any breach other than non-payment is not reasonably curable within [***] days and if Biogen is making a bona fide effort to cure such breach, such termination shall be delayed for so long as Biogen is continuing to make such bona fide efforts to cure such breach. The cure period shall be tolled pending resolution of any bona fide dispute between the Parties as to whether any such material breach has occurred.

16.5.2. Termination for Biogen Patent Challenge. Except to the extent the following under this Section 16.5.2 is unenforceable under the law of the applicable jurisdiction where the applicable Patent Right is pending or issued, in the event that Biogen or any of its Affiliates, individually or in association with any other person or entity, initiates or assists in initiating or continuing a determination that any Patent Right owned or Controlled by AGTC is invalid or unenforceable or otherwise limit the scope of any such Patent Right (a "**Biogen Patent Challenge**") through any administrative, judicial or other similar proceeding with respect to such Patent Right in a particular jurisdiction, AGTC may either, at its sole discretion (i) [***] days' prior written notice to Biogen, unless such Biogen Patent Challenge is dropped within such [***] day period; or (ii) elect, in lieu of termination, [***]. In any event, Biogen shall reimburse AGTC for all costs incurred by AGTC, its Affiliates or their respective sublicensees in connection with such Biogen Patent Challenge upon written notice to Biogen. Biogen will include the obligations set forth in this Section 16.5.2 in any sublicenses of its rights under this Agreement and shall use reasonable efforts to ensure its Sublicensees' compliance with such obligations, provided that AGTC shall have no termination right under this Section 16.5.2 in the event of any failure by such a Sublicensee to comply with such obligations, unless (a) Biogen has not included such provision in the applicable sublicense and (b) such Sublicensee individually or in association with any other person or entity, initiates or assists in initiating or continuing a determination that any Patent Right owned or Controlled by AGTC is invalid or unenforceable or otherwise limit the scope of any such Patent Right. AGTC will be a third party beneficiary of such provisions in any sublicense agreement solely for the purpose of enforcing its rights under such sublicense provisions directly.

**16.6. Termination by Biogen**.

16.6.1. Termination for Convenience. At any time upon at least [***] days' written notice to AGTC, Biogen may terminate this Agreement with respect to any Initial Licensed Program, any Initial Licensed Product or any Discovery Program during the Research Period for such Discovery Program, without cause, for any or no reason, which termination shall be effective after the expiration of such [***] day period. At any time upon written notice to AGTC, Biogen may terminate this Agreement with respect to any Discovery Product for which Biogen has exercised the Option, without cause, for any or no reason, which termination shall be effective immediately upon receipt of such notice by AGTC. In the event any Discovery Program is terminated under this Section 16.6.1, such Discovery Program shall be deemed to be an Abandoned Program.

16.6.2. Termination for Breach by AGTC. In the event that AGTC commits a material breach of its obligations under any or all Collaboration Programs or with respect to any or all Licensed Products under this Agreement and such material breach remains uncured for [***] days (or in the case of non-payment that constitutes a material breach, [***] days), measured from the date written notice of such material breach is given to AGTC, Biogen may, in its sole discretion, terminate this Agreement either for cause in its entirety or on a Collaboration Program-by-Collaboration Program or

Licensed Product-by-Licensed Product basis with respect to the Collaboration Programs or Licensed Products to which such material breach directly relates, in each case, in one or more countries in the Territory, at any time during the Term after such [***] day period (or the applicable [***] day period), by giving written notice to AGTC; provided, however, that if any breach other than non-payment is not reasonably curable within [***] days and if AGTC is making a bona fide effort to cure such breach, such termination shall be delayed for so long as AGTC is continuing to make such bona fide efforts to cure such breach. The cure period shall be tolled pending resolution of any bona fide dispute between the Parties as to whether any such material breach has occurred.

16.6.3. Termination Due to Material Adverse Event. This Agreement will terminate in its entirety if a Material Adverse Event has occurred and Biogen provides notice of termination to AGTC within three (3) Business Days after the Schedule Revision Date that such Material Adverse Event has occurred.

**16.7. Termination for Insolvency**. To the extent permissible under applicable Law, in the event that either Party makes an assignment for the benefit of creditors, appoints or suffers appointment of an administrator, receiver or trustee over all or substantially all of its property to which this Agreement relates, files a petition under any bankruptcy or insolvency act or has any such petition filed against it which is not dismissed within sixty (60) days of the filing thereof (each, an "**Insolvency Event**" and the Party undergoing such Insolvency Event, the "**Insolvent Party**"), then the other Party may terminate this Agreement effective immediately upon written notice to Insolvent Party. In the event of a rejection of this Agreement by the Insolvent Party or any trustee thereof under Section 365 of the Bankruptcy Code:

16.7.1. All rights and licenses now or hereafter granted by the Insolvent Party to the other Party under or pursuant to this Agreement, including, for the avoidance of doubt, the licenses granted under Sections 5.1, 5.3, 5.4 and 16.8.1, are, for all purposes of Section 365(n) of the Bankruptcy Code, licenses of rights to "intellectual property" as defined in the Bankruptcy Code. Upon the rejection of this Agreement by the Insolvent Party or any trustee thereof, the Insolvent Party, for itself and any successors or assigns, including any trustee, agrees that the other Party, as licensee of such rights under this Agreement, shall retain and may fully exercise all of its rights and elections under the Bankruptcy Code. The Insolvent Party shall, during the term of this Agreement, create and maintain current copies of all intellectual property licensed under this Agreement. Each Party acknowledges and agrees that "embodiments" of such intellectual property within the meaning of Section 365(n) include, without limitation, laboratory notebooks, product samples and inventory, research studies and data, all Marketing Applications and Regulatory Approvals and rights of reference therein, of the AGTC Technology on the one hand or the Biogen Technology and Program Data on the other hand, and in either case, the Joint Technology. If (i) a case under the Bankruptcy Code is commenced by or against the Insolvent Party, (ii) this Agreement is rejected as provided in the Bankruptcy Code, and (iii) the other Party elects to retain its rights hereunder as provided in Section 365(n) of the Bankruptcy Code, the Insolvent Party (in any capacity, including debtor-in-possession) and its successors and assigns (including a trustee) shall:

(a) provide to the other Party all such intellectual property (including all embodiments thereof) in the possession of the Insolvent Party on terms agreed by the Parties, promptly upon the other Party's written request.

(b) not interfere with the non-insolvent Party's rights under this Agreement, or any agreement supplemental hereto, to such intellectual property (including such embodiments), including any right to obtain such intellectual property (or such embodiments) from another entity, to the extent provided in Section 365(n) of the Bankruptcy Code.

16.7.2. All rights, powers and remedies of each Party provided herein are in addition to and not in substitution for any and all other rights, powers and remedies now or hereafter existing at law or in equity (including the Bankruptcy Code) in the event of the commencement of a case under the Bankruptcy Code with respect to the Insolvent Party. The Parties agree that they intend the following rights to extend to the maximum extent permitted by law, and to be enforceable under Bankruptcy Code Section 365(n) upon any rejection of this Agreement: the right of access on terms agreed by the Parties to any intellectual property (including all embodiments thereof) of the Insolvent Party which is necessary for the Manufacture, use, sale, import or export of Licensed Products.

**16.8. Effects of Termination**.

16.8.1. Effects of Termination.

(a) *Termination by AGTC for Cause of an Initial Licensed Program*.

(i) In the event AGTC terminates this Agreement for cause with respect to any Initial Licensed Program or Initial Licensed Product pursuant to Section 16.5, then, with respect to such Initial Licensed Program or Initial Licensed Product in such applicable countries, at AGTC's election, (1) Biogen shall, and hereby does, grant to AGTC an exclusive, royalty-bearing license, with the ability to sublicense, under any Know-How and Patent Rights Controlled by Biogen or its Affiliates that are necessary for, or useful for and were in use by, Biogen or its Affiliates or Sublicensees in, the Initial Licensed Program or the Development, Manufacture or Commercialization of the applicable Initial Licensed Product at the time of such termination, to Develop, Manufacture, Commercialize and use such Initial Licensed Product in the Field in the terminated country(ies), (2) at Biogen's expense, Biogen shall within thirty (30) days of AGTC's request, transfer or begin transferring all Marketing Applications and Regulatory Approvals with respect to such Initial Licensed Product(s) to AGTC, (3) at Biogen's expense, Biogen shall within thirty (30) days of AGTC's request, diligently conduct a Know-How transfer to AGTC, including all relevant Program Data, included in the license set forth in clause (1), (4) Biogen shall within thirty (30) days of AGTC's request, transfer a reasonable amount of such Initial Licensed Product for clinical and commercial use, along with work in process for such clinical and commercial supply to the extent practicable, requested by AGTC, and AGTC shall reimburse Biogen for such Materials at Cost of Goods Sold (provided that, if the terminated Initial Licensed Product is a Cost Share Product, AGTC may subtract from such reimbursement any amounts already reimbursed by AGTC to Biogen for such Materials), and (5) all other rights and obligations of the Parties under this Agreement with respect to such Initial Licensed Program or such Initial Licensed Product shall terminate, except that, with respect to any terminated Initial Licensed Program, (a) Biogen's obligations under Section 5.8.1 shall continue for two (2) years after the effective date of such termination under Section 16.5 and (b) the restrictions on Biogen's use, licensing, assignment and transfer of Joint Technology that constitutes an improvement or enhancement to the [***] Manufacturing Technology as set forth in Section 5.4.1 shall continue until the later of (i) five (5) years from the effective date of termination with respect to such Initial Licensed Program and (ii) the date that this Agreement has expired or terminated with respect to both Initial Licensed Programs.

(ii) Effective upon AGTC's election to obtain the license in Section 16.8.1(a)(i), Biogen hereby grants to AGTC a "Right of Reference", as that term is defined in 21 C.F.R. § 314.3(b) and any analogous regulation outside of the United States, to any data Controlled by Biogen or its Affiliates that relates to the terminated Initial Licensed Program or Initial Licensed Product, as applicable, including without limitation applicable Program Data, any Preclinical Studies or Clinical Trials or any Initial Licensed Product solely to Develop, Manufacture and Commercialize the Initial Licensed Product with respect to which this Agreement has been terminated, and Biogen shall provide a signed statement to this effect, if requested by AGTC, in accordance with 21 C.F.R. § 314.50(g)(3) (or any analogous applicable Law recognized outside of the United States).

(b) *Termination by Biogen for Convenience of an Initial Licensed Program*.

(i) In the event Biogen terminates this Agreement for convenience with respect to any Initial Licensed Program or Initial Licensed Product pursuant to Section 16.6.1, with respect to such Initial Licensed Program or Initial Licensed Product at AGTC's election (1) Biogen shall, and hereby does, grant to AGTC an exclusive, royalty-bearing license under any Know-How and Patent Rights Controlled by Biogen or its Affiliates that are necessary for, or useful for and were in use by, Biogen or its Affiliates or Sublicensees in, the Initial Licensed Program or the Development, Manufacture or Commercialization of the applicable Initial Licensed Product at the time of such termination, to Develop, Manufacture, Commercialize and use such Initial Licensed Product in the Field in the Territory, (2) at Biogen's expense, Biogen shall within thirty (30) days of AGTC's request, transfer or begin transferring all Marketing

Applications and Regulatory Approvals with respect to such Initial Licensed Product to AGTC, (3) at Biogen's expense, Biogen shall within thirty (30) days of AGTC's request conduct a Know-How transfer to AGTC, including all relevant Program Data, included in the license set forth in clause (1), (4) Biogen shall within thirty (30) days of AGTC's request, transfer a reasonable amount of such Initial Licensed Product for clinical and commercial use, along with work in process for such clinical and commercial supply to the extent practicable, requested by AGTC, and AGTC shall reimburse Biogen for such Materials at Cost of Goods Sold (provided that, if the terminated Initial Licensed Product is a Cost Share Product, AGTC may subtract from such reimbursement any amounts already reimbursed by AGTC to Biogen for such Materials), and (5) all other rights and obligations of the Parties under this Agreement with respect to such Initial Licensed Program or such Initial Licensed Product, including the Parties' respective obligations under Section 5.8, shall terminate, except that, with respect to any terminated Initial Licensed Program, (a) [***] (ii) the date that this Agreement has expired or terminated with respect to both Initial Licensed Programs and (b) Biogen's obligations under Section 5.8.1 with respect to such terminated Initial Licensed Program shall continue until the date that is [***] days after the effective date of termination.

(ii) Effective upon AGTC's election to obtain the license in Section 16.8.1(b)(i), Biogen hereby grants to AGTC a "Right of Reference," as that term is defined in 21 C.F.R. § 314.3(b) and any analogous regulation outside of the United States, to any data Controlled by Biogen or its Affiliates that relates to the terminated Initial Licensed Program or Initial Licensed Product, as applicable, including without limitation applicable Program Data, any Preclinical Studies or Clinical Trials or to any Initial Licensed Product solely to Develop, Manufacture and Commercialize the Initial Licensed Product with respect to which this Agreement has been terminated, and Biogen shall provide a signed statement to this effect, if requested by AGTC, in accordance with 21 C.F.R. § 314.50(g)(3) (or any analogous applicable Law recognized outside of the United States).

(c) *Termination of a Discovery Program; Abandoned Programs*. In the event either Party terminates this Agreement with respect to any Discovery Program or any Discovery Product pursuant to any provision of Section 16.5 or Section 16.6, or in the event that any Discovery Program becomes an Abandoned Program hereunder, except as otherwise expressly provided herein, all rights and obligations of the Parties under this Agreement with respect to such Discovery Program or such Discovery Product, including the Parties' respective obligations under Section 5.8, shall terminate.

(d) *Consideration for the Reversionary Licenses*. In consideration for either of the licenses granted to AGTC with respect to an Initial Licensed Product pursuant to Section 16.8.1(a) or Section 16.8.1(b), AGTC shall pay to Biogen a royalty on Net Sales of such Initial Licensed Product (calculated in accordance with Section 1.184, which shall apply *mutatis mutandis* to such calculation) on a Calendar Quarter basis as follows: (i) such Net Sales shall be multiplied by the applicable royalty rate set forth in Section 6.4.3(a); (ii) the reductions set forth in Section 6.6.1 (provided that the reductions set forth in Section 6.6.1 shall apply only with respect to Third Party royalty payments, and no other Third Party payments), Section 6.6.2 and Section 6.6.3 shall be applied to the amount set forth in clause (i), if applicable; and (iii) the amount set forth in clause (ii) shall be multiplied by a percentage, as set forth in Table 16.8.1(d)-1 (in the case of a license granted under Section 16.8.1(a)) or Table 16.8.1(d)-2 (in the case of a license granted under Section 16.8.1(b)) below based on the effective date of termination with respect to such Initial Licensed Product and whether such Initial Licensed Product was a Cost Share Product. Such royalties shall be payable to Biogen on a country-by-country and Initial Licensed Product-by-Initial Licensed Product basis, until the latest of (a) the expiration of the last to expire of any Valid Claim included in any AGTC Patent Right or Joint Patent Right that is issued or pending in such country as of the effective date of termination, which Valid Claim Covers the manufacture, use, sale, offer for sale or importation of such Initial Licensed Product in such country, (b) [***]. The royalty floor set forth in Section 6.6.4 shall not apply with respect to any Initial Licensed Product that AGTC is Commercializing under this Section 16.8.1; provided, however, that in no event shall any royalty payment payable to Biogen under this Section 16.8.1(d) for any Initial Licensed Product in a given Calendar Quarter be reduced to less than the royalty payments payable by Biogen to Third Parties with respect to such Initial Licensed Product in such Calendar Quarter plus [***]. In addition, AGTC shall be responsible for any milestone payments payable by Biogen to Third Parties arising out of the Development, Manufacture, Commercialization or use of such

Initial Licensed Product. The provisions of Section 6.7, Section 9.2 and Section 9.3 shall apply *mutatis mutandis* during such time as AGTC is Commercializing an Initial Licensed Product under this Section 16.8.1. In the event of a termination by AGTC for Biogen's non-payment pursuant to Section 16.5.1, AGTC may credit the amount of the non-payment, together with interest that accrued pursuant to Section 6.10 from the first and any subsequent payments due to Biogen under this Section 16.8.1(d) until such amount is exhausted.

**Table 16.8.1(d)-1**

**Consideration for the License under Section 16.8.1(a) (Termination by AGTC for Cause of an Initial Licensed Program)**

**[***]**

**Table 16.8.1(d)-2**

**Consideration for the License under Section 16.8.1(b) (Termination by Biogen for Convenience of an Initial Licensed Program)**

[***]

(e) *Termination for Cause by Biogen*. In the event Biogen terminates this Agreement pursuant to Section 16.6.2 for cause, with respect to any Collaboration Program or Licensed Product in any country in the Territory, except as otherwise expressly provided herein, all rights and obligations of each Party with respect to such Collaboration Program or Licensed Product in such country shall cease, provided that AGTC's obligations under Section 5.8.1 or Section 5.8.2, as applicable, with respect to the applicable Licensed Program, shall survive for a period of [***] years from the effective date of such termination. In the event Biogen terminates this Agreement in its entirety pursuant to Section 16.6.2 for cause, except as otherwise expressly provided herein, all rights and obligations of each Party under this Agreement shall cease, provided that AGTC's obligations under Section 5.8.1 and Section 5.8.2 shall survive for a period of [***] years from the effective date of such termination.

16.8.2. Sublicense Survival. In the event of any termination of this Agreement, any permitted sublicense of either Party shall, at the applicable Sublicensee's option, survive such termination, provided that the Sublicensee is not in breach of any of its obligations under such sublicense and provided, further, that, in the case of a Sublicensee of Biogen, such Sublicensee has not initiated or assisted in the initiation or continuation of any Biogen Patent Challenge. In order to effect this provision, at the request of the Sublicensee, the licensor Party shall enter into a direct license with the Sublicensee on substantially the same terms as the sublicense, provided that the licensor Party shall not be required to undertake obligations in addition to those required by this Agreement, and that the licensor Party's rights under such direct license shall be consistent with its rights under this Agreement, taking into account the scope of the license granted under such direct license.

16.8.3. Accrued Rights. Expiration or termination of this Agreement for any reason shall be without prejudice to any right which shall have accrued to the benefit of either Party prior to such termination, including damages arising from any breach under this Agreement. Expiration or termination of this Agreement shall not relieve either Party from any obligation which is expressly indicated to survive such expiration or termination.

16.8.4. Survival of Certain Provisions. In addition to any other provisions expressly stated in this Agreement to survive expiration or termination of this Agreement, the following sections (and any other sections referenced therein for the corresponding time periods set forth therein) of this Agreement shall survive expiration or termination of this Agreement for any reason: Article 1, Section 5.4, Section 5.5.3, Section 5.7, Section 5.8.4, Section 6.7, Section 6.8, Section 6.9, Section 6.10, Article 9 (solely with respect to record-keeping and audits of records for activities conducted under this Agreement prior to the effective date of termination), Section 11.4, Section 13.1, Section 14.1 (for the time periods set forth therein), Section 14.2, Section 14.3, Section 14.4, Section 14.5, Section 14.6, Section 15.5, Section 15.7, Section 15.9, Section 15.10, Section 16.8, Section 17.1, Section 17.2 through Section 17.5 (solely with respect to indemnification of claims arising from activities conducted under this Agreement prior to the effective date of termination), Section 18.1 and Section 18.3 through Section 18.16.

**16.9. Effects of Material Breach by AGTC in Lieu of Termination**. Notwithstanding anything to the contrary, in the event of any material breach by AGTC of its obligations under this Agreement that remains uncured following the applicable cure period under Section 16.6.2, Biogen may elect, in lieu of terminating this Agreement in whole or in part as a result of such material breach, (a) to convert the financial terms for any Cost Share Product to which such material breach directly relates to the Milestone/Royalty Option and (b) to reduce all further royalty and milestone payments payable by Biogen to AGTC under this Agreement, for a Licensed Product to which such material breach directly relates, as follows: (i) with respect to royalty payments payable for such Licensed Product, on a country-by-country basis, for each Calendar Quarter during the Royalty Term for such Licensed Product, (x) Net Sales of such Licensed Product shall be multiplied by the applicable royalty rate set forth in Section 6.4.3(a) or Section 6.5.3(a), as applicable; (y) the reductions set forth in Section 6.6.1, Section 6.6.2 and Section 6.6.3 shall be applied to the amount set forth in clause (x), if applicable; and (z) the amount set forth in clause (y) shall be multiplied by a percentage, as set forth in Table 16.9 below based on the date that the applicable cure period under Section 16.6.2 ends with respect to such Licensed Product and whether such Licensed Product was a Cost Share Product, and (ii) with respect to any further milestone payments payable for Licensed Product, such milestone payments shall be equal to the milestone payments otherwise payable to AGTC for such Licensed Product, multiplied by a percentage, as set forth in Table 16.9 below based on the date that the applicable cure period under Section 16.6.2 ends with respect to such Licensed Product and whether such Licensed Product was a Cost Share Product. The royalty floor and event milestone floor set forth in Section 6.6.4 shall not apply with respect to any Licensed Product for which Biogen has elected to reduce further royalty and milestone payments under this Section 16.9, provided that, subject to Section 6.6.1(b)(iii), in no event shall (i) any royalty payment payable to AGTC under this Agreement for any Licensed Product in a given Calendar Quarter be reduced to less than the royalty payments payable by AGTC to Third Parties plus [***] with respect to such Licensed Product in such Calendar Quarter or (ii) any milestone payment payable to AGTC under this Section 16.9 for any Initial Licensed Product be reduced to less than the milestone payments payable by AGTC to Third Parties for the applicable event milestone with respect to such Initial Licensed Product. In addition, Biogen shall be responsible for any milestone payments payable by AGTC to Third Parties arising out of the Development, Manufacture, Commercialization or use of such Licensed Product for event milestones that do not correspond to any milestone payment under this Agreement. In the event of a termination by Biogen for AGTC's non-payment pursuant to Section 16.6.2, Biogen may credit the amount of the non-payment, together with interest that accrued pursuant to Section 6.10 from the first and any subsequent milestone or royalty payments due to AGTC under this Agreement until such amount is exhausted.

**Table 16.9**

[***]

**16.10. Termination of AGTC Third Party Agreements**. In the event that any AGTC Third Party Agreement is terminated, so long as Biogen is not in default of any obligation under this Agreement, Biogen shall have rights to obtain a direct license to any such AGTC Third Party Agreement subject to the terms and conditions as expressly set forth in such AGTC Third Party Agreement.

**17. LIABILITY, INDEMNIFICATION AND INSURANCE**.

**17.1. No Consequential Damages**. Except with respect to liability arising from a breach of Section 5.8 or Article 14, from any willful misconduct or intentionally wrongful act, or to the extent such Party may be required to indemnify the other Party under this Article 17, in no event will either Party or any of its respective Affiliates, agents or representatives be liable under this Agreement for any special, indirect, incidental or consequential damages, whether in contract, warranty, tort, negligence, strict liability or otherwise, including loss of profits or revenue suffered by either Party or any of its respective Affiliates, agents or representatives. Without limiting the generality of the foregoing (a) "consequential damages" will be deemed to include, and neither Party will be liable to the other Party or any of the other Party's Affiliates, agents, representatives or stockholders for, any damages based on or measured by, any Event Milestone Payment due upon any unachieved event milestone under Section 6.2.1, Section 6.4.1 or Section 6.5.2, any Sales Milestone Payment due upon any unachieved annual Net Sales level under Section 6.4.2, any unearned royalties under Section 6.4.3 or Section 6.5.3, or any other unearned, speculative or otherwise contingent payments provided for in this Agreement and (b) "consequential damages" will be deemed to include, and neither Party will be liable to the other Party or any of the other Party's Affiliates or representatives for, any damages based on or measured by the other Party's, its Affiliates' or its Sublicensees' loss of projected or speculative future sales of the Licensed Product(s).

**17.2. Indemnification by Biogen**. Biogen will indemnify, defend and hold harmless AGTC, each of its Affiliates and each licensor of the AGTC Technology, and each of its and its Affiliates' or such licensor's employees, officers, directors, trustees and agents and inventors of AGTC Technology licensed under the UAB Agreement (each, an "**AGTC Indemnified Party**") from and against any and all liability, loss, damage, expense (including reasonable attorneys' fees and expenses) and cost (collectively, a "**Liability**") that the AGTC Indemnified Party may be required to pay to one or more Third Parties resulting from or arising out of:

17.2.1. any claims of any nature arising out of the Development, Manufacture, Commercialization, consumption or use of any Licensed Product by, or on behalf of, Biogen (other than by any AGTC Indemnified Party), or under the authority of Biogen including without limitation death of or injury to any Person or out of damage to property, other than claims for which AGTC is required to indemnify Biogen pursuant to Section 17.3; or

17.2.2. the breach by Biogen of any of its representations, warranties, covenants or obligations set forth in this Agreement;

except, in each case, to the extent such Liabilities are caused by the recklessness, negligence or intentional misconduct of AGTC or any AGTC Indemnified Party. Notwithstanding anything to the contrary, if AGTC has exercised the Cost Share Option with respect to an Initial Licensed Product, in the event of any Third Party claim against AGTC, Biogen or any AGTC Indemnified Party or Biogen Indemnified Party arising out of the Development, Manufacture, Commercialization, consumption or use of the applicable Cost Share Product, Biogen and AGTC will coordinate in defending such claim and will share any Liabilities resulting from or arising out of such claim equally in accordance with Section 6.3, except to the extent such claim is caused by the recklessness, negligence or intentional misconduct of, or a breach of any representation or warranty by, (i) AGTC or any AGTC Indemnified Party, in which case, AGTC shall indemnify Biogen Indemnified Parties under Section 17.3 or (ii) Biogen or any Biogen Indemnified Party, in which case Biogen shall indemnify the AGTC Indemnified Parties under this Section 17.2.

**17.3. Indemnification by AGTC**. AGTC will indemnify, defend and hold harmless Biogen, its Affiliates, Sublicensees, Distributors and each of its and their respective employees, officers, directors and agents (each, a "**Biogen Indemnified Party**") from and against any and all Liabilities that the Biogen Indemnified Party may be required to pay to one or more Third Parties resulting from or arising out of:

17.3.1. the breach by AGTC of any of its representations, warranties, covenants or obligations set forth in this Agreement;

17.3.2. any claim that the practice of the [***] Technology to Develop, Manufacture, Commercialize or use any Initial Licensed Product infringes or misappropriates any issued patent or other proprietary right owned or possessed by any Third Party, other than any such claim to the extent that (i) it is based on the practice of the AGTC Technology in combination with Technology other than AGTC Technology that is utilized in the Development, Manufacture, Commercialization or use of any Initial Licensed Product as a result of Biogen's exercise of its final decision-making authority or (ii) it arises from Biogen's election not to take a license or sublicense to any Technology under Section 13.6.2(a); or

17.3.3. any claims of any nature arising out of the research, Development or Manufacturing activities performed by AGTC with respect to any Collaboration Programs prior to the Execution Date or any research, Development or Manufacturing activities performed by AGTC hereunder during the Term, other than claims for which Biogen is required to indemnify AGTC under Section 17.2;

except, in each case, to the extent such Liabilities are (i) caused by the recklessness, negligence or intentional misconduct of Biogen or any Biogen Indemnified Party or (ii) that Biogen has already recovered such Liabilities under Section 3.2.2(a)(ii). Notwithstanding anything to the contrary, if AGTC has exercised the Cost Share Option with respect to an Initial Licensed Product, in the event of any Third Party claim against AGTC, Biogen or any AGTC Indemnified Party or Biogen Indemnified Party that the practice of the AGTC Technology to Develop, Manufacture, Commercialize or use of the applicable Cost Share Product infringes or misappropriates any issued patent or other proprietary right owned or possessed by such Third Party, Biogen and AGTC will coordinate in defending such claim and will share any Liabilities resulting from or arising out of such claim equally in accordance with Section 6.3, except to the extent such claim is caused by the recklessness, negligence or intentional misconduct of, or a breach of any representation or warranty by, (i) AGTC or any AGTC Indemnified Party, in which case, AGTC shall indemnify Biogen Indemnified Parties under this Section 17.3 or (ii) Biogen or any Biogen Indemnified Party, in which case Biogen shall indemnify the AGTC Indemnified Parties under Section 17.2.

**17.4. Procedure**. Each Party will notify the other Party in writing in the event it becomes aware of a claim for which indemnification may be sought hereunder. In case any proceeding (including any governmental investigation) shall be instituted involving any Party in respect of which indemnity may be sought pursuant to this Article 17, such Party (the "**Indemnified Party**") shall promptly notify the other Party (the "**Indemnifying Party**") in writing and the Indemnifying Party and Indemnified Party shall meet to discuss how to respond to any claims that are the subject matter of such proceeding. The Indemnified Party shall reasonably cooperate with the Indemnifying Party in defense of such matter. The Indemnifying Party, upon request of the Indemnified Party, shall retain counsel reasonably satisfactory to the Indemnified Party to represent the Indemnified Party and shall pay the fees and expenses of such counsel related to such proceeding. In any such proceeding, the Indemnified Party shall have the right to retain its own counsel, but the fees and expenses of such counsel shall be at the expense of the Indemnified Party unless (a) the Indemnifying Party and the Indemnified Party shall have mutually agreed to the retention of such counsel, (b) the named parties to any such proceeding (including any impleaded parties) include both the Indemnifying Party and the Indemnified Party and representation of both Parties by the same counsel would be inappropriate due to actual or potential differing interests between them. All such fees and expenses shall be reimbursed as they are incurred. The Indemnifying Party shall not be liable for any settlement of any proceeding effected without its written consent, but, if settled with such consent or if there be a final judgment for the plaintiff, the Indemnifying Party agrees to indemnify the Indemnified Party from and against any loss or liability by reason of such settlement or judgment. The Indemnifying Party shall not, without the written consent of the Indemnified Party, effect any settlement of any pending or threatened proceeding in respect of which the Indemnified Party is, or could have been, a party and indemnity could have been sought hereunder by the Indemnified Party, unless such settlement includes an unconditional release of the Indemnified Party from all liability on claims that are the subject matter of such proceeding.

**17.5. Special Indemnification by Biogen of the Existing Licensors**.

17.5.1. Biogen shall, at all times during the term of this Agreement and thereafter, indemnify, defend and hold UFRF, the Florida Board of Governors, the University of Florida Board of Trustees, the University of Florida, and each of their directors, officers, employees, and agents, and the inventors of the any Patent Rights licensed to AGTC under the UFRF Existing License Agreements, regardless of whether such inventors are employed by the University of Florida at the time of the claim, harmless against all claims and expenses, including legal expenses and reasonable attorneys' fees, whether arising from a Third Party claim or resulting from UFRF's enforcing this indemnification clause against Biogen arising out of the death of or injury to any person or persons or out of any damage to property and against any other claim, proceeding, demand, expense and liability of any kind whatsoever (other than patent infringement claims) resulting from the production, manufacture, sale, use, lease, consumption, marketing, or advertisement of Licensed Products or use of any processes licensed hereunder or arising from any right or obligation of Biogen hereunder. Notwithstanding the above, UFRF at all times reserves the right to retain counsel of its own to defend UFRF's, the Florida Board of Governors', the University of Florida Board of Trustees', the University of Florida's, and the inventor's interests.

17.5.2. Biogen warrants that it now maintains and will continue to maintain liability insurance coverage appropriate to the risk involved in producing, manufacturing, selling, marketing, using, leasing, consuming, or advertising the products subject to this Agreement. Notwithstanding the foregoing, Biogen may self-insure to the extent that it self-insures for its other products.

17.5.3. JHU and [***] who are employees of JHU (hereinafter "**JHU Inventors**") will have no legal liability exposure to Third Parties if JHU does not license the Licensed Products and processes licensed under the UF/JHU Agreement, and any royalties JHU and the JHU Inventors may receive is not adequate compensation for such legal liability exposure. Furthermore, JHU and JHU Inventors will not, under the provisions of the UF/JHU Agreement or otherwise, have control over the manner in which Biogen or its Affiliates or its Sublicensees or those operating for its account or Third Parties who purchase Licensed Products and processes licensed under the UF/JHU Agreement from any of the foregoing entities, develop, manufacture, market or practice the inventions of such Licensed Products and processes. Therefore, Biogen, and its Affiliates and Sublicensees shall indemnify, defend with counsel reasonably acceptable to JHU, and hold JHU, The Johns Hopkins Health Systems, their present and former trustees, officers, JHU Inventors, agents, faculty, employees and students harmless as against any judgments, fees, expenses, or other costs arising from or incidental to any product liability or other lawsuit, claim, demand or other action brought as a consequence of the practice of said inventions by any of the foregoing entities, whether or not JHU or said JHU Inventors, either jointly or severally, is named as a party defendant in any such lawsuit and whether or not JHU or the JHU Inventors are alleged to be negligent or otherwise responsible for any injuries to persons or property. Practice of the inventions covered by such Licensed Products and

processes, by an Affiliate or an agent or a Sublicensee or a Third Party on behalf of or for the account of Biogen or by a Third Party who purchases such Licensed Products and processes from Biogen, shall be considered Biogen's practice of said inventions for purposes of this Section 17.5.3. The obligation of Biogen to defend and indemnify as set out in this Section 17.5.3 shall survive the termination of this Agreement or the UF/JHU Agreement, shall continue even after assignment of rights and responsibilities to an Affiliate or Sublicensee, and shall not be limited by any other limitation of liability elsewhere in this Agreement or the UF/JHU Agreement.

17.5.4. Biogen shall indemnify, defend and hold harmless [***] and its current and former directors, governing board members, trustees, officers, faculty, medical and professional staff, employees, students, and agents and their respective successors, heirs and assigns (collectively, the "[***] **Indemnitees**") from and against any claim, liability, cost, expense, damage, deficiency, loss or obligation of any kind or nature (including reasonable attorneys' fees and other costs and expenses of litigation) by or owed to a Third Party, based upon, arising out of, or otherwise relating to the activities of Biogen, its Affiliates and Sublicensees under this Agreement, including any cause of action relating to product liability concerning any product, process, or service made, used, sold or performed pursuant to any right or license granted under this Agreement (collectively, the "[***] **Claims**"); provided, however, that Biogen's indemnification obligations hereunder shall not apply to any [***] Claim to the extent that it is attributable to the gross negligence or willful misconduct of any [***] Indemnitee.

17.5.5. Biogen shall, at its own expense, provide attorneys reasonably acceptable to [***] to defend against any actions brought or filed against any [***] Indemnitee hereunder with respect to the subject of indemnity contained herein, whether or not such actions are rightfully brought. Any [***] Indemnitee seeking indemnification hereunder shall promptly notify Biogen of such [***] Claim; provided that any failure of or delay in such notification shall not affect Biogen's indemnification obligation unless and to the extent such failure or delay is materially prejudicial to Biogen. The [***] Indemnitees shall provide Biogen, at Biogen's expense, with reasonable assistance and full information with respect to such [***] Claim and give Biogen sole control of the defense of any [***] Claim. Neither Biogen nor [***] shall settle any [***] Claim without the prior written consent of the other, which consent shall not be unreasonably withheld.

**17.6. Insurance**.

17.6.1. Insurance Obligations of AGTC. AGTC will maintain, at its cost, reasonable insurance against liability and other risks associated with its activities contemplated by this Agreement, provided that, if AGTC is engaged in any Development activities with respect to the Licensed Products hereunder, AGTC will maintain, in force from thirty (30) days prior to enrollment of the first subject in a Clinical Trial, a Clinical Trials/product liability insurance policy providing coverage of at least [***] per claim and [***] annually in the aggregate, and provided, further, that, if AGTC exercises its Co-Promotion Option, that such coverage is increased to at least [***] at least thirty (30) days before Biogen initiates the First Commercial Sale of the applicable Licensed Product. AGTC will furnish to Biogen evidence of such insurance upon request.

17.6.2. Insurance Obligations of Biogen. Biogen, together with its Affiliates, will maintain, at its cost, reasonable insurance against liability and other risks associated with its activities contemplated by this Agreement, provided that, at a minimum, Biogen will maintain, in force from thirty (30) days prior to enrollment of the first subject in a Clinical Trial, a Clinical Trials/product liability insurance policy providing coverage of at least [***] per claim and [***] annually in the aggregate, and provided, further, that such coverage is increased to at least [***] at least thirty (30) days before Biogen initiates the First Commercial Sale of a Licensed Product. Biogen will furnish to AGTC evidence of such insurance upon request. Notwithstanding the foregoing, so long as (i) substantially all of Biogen's equity securities remain publicly traded on a nationally recognized stock exchange and (ii) Biogen or any Affiliate of Biogen is researching, developing and commercializing Licensed Products under this Agreement, Biogen may self-insure against liability and other risks associated with its and its Affiliates' activities under this Agreement to the extent that it self-insures in respect of its other products, but at a minimum will self-insure at levels that are consistent with levels customarily maintained against similar risks by similar companies in Biogen's industry.

17.6.3. Upon request of AGTC or an Existing Licensor, Biogen will furnish to AGTC or such licensor with a certificate of insurance of each product liability insurance policy obtained.

**18. MISCELLANEOUS.**

**18.1. Assignment**. Neither this Agreement nor any interest hereunder shall be assignable by either Party without the prior written consent of the other Party, except as follows: (a) either Party may, subject to the terms of this Agreement, assign its rights and obligations under this Agreement by way of sale of itself or the sale of the portion or substantially all of the portion of such Party's business to which this Agreement relates, through merger, sale of assets and/or sale of stock or ownership interest, provided that such sale is not primarily for the benefit of its creditors and (b) either Party may assign its rights and obligations under this Agreement to any of its Affiliates, provided that the assigning Party shall remain liable for all of its rights and obligations under this Agreement. The assigning Party shall promptly (and in any event within two (2) Business Days) notify the other Party of any assignment or transfer under the provisions of this Section 18.1. This Agreement shall be binding upon the successors and permitted assigns of the Parties and the name of a Party appearing herein shall be deemed to include the names of such Party's successors and permitted assigns to the extent necessary to carry out the intent of this Agreement. Any assignment not in accordance with this Section 18.1 shall be void. Biogen shall not assign this Agreement without the prior written consent of [***], except that Biogen may assign this Agreement to an Affiliate or a successor in connection with the merger, consolidation or sale of all or substantially all of its assets or that portion of its business to which this Agreement relates; provided, however, that any permitted assignee agrees in writing to be bound by the terms of this Agreement.

**18.2. Change of Control**.

18.2.1. Notification. Each Party shall notify the other Party in writing promptly (and in any event within four (4) Business Days) following the entering into of a definitive agreement with respect to a Change of Control of such Party.

18.2.2. Effects of Change of Control of AGTC. In addition to the applicable effects of Section 5.8.3, if any, if during the Term AGTC undergoes a Change of Control with respect to one or both Initial Licensed Programs, then upon the closing of such Change of Control, on an Initial Licensed Program-by-Initial Licensed Program basis:

[***]

Notwithstanding the foregoing, if during the Term AGTC undergoes a Change of Control with respect to all Collaboration Programs, the effects set forth above in paragraphs [***] shall apply with respect to all Collaboration Programs.

18.2.3. Effects of Change of Control of Biogen. In addition to the applicable effects of Section 5.8.3, if any, if during the Term Biogen undergoes a Change of Control with respect to one or both Initial Licensed Programs, then upon the closing of such Change of Control, on an Initial Licensed Program-by-Initial Licensed Program basis:

[***]

Notwithstanding the foregoing, if during the Term Biogen undergoes a Change of Control with respect to all Collaboration Programs, the effects set forth above in paragraphs [***] shall apply with respect to all Collaboration Programs.

**18.3. Force Majeure**. Each Party shall be excused from the performance of its obligations under this Agreement to the extent that such performance is prevented by force majeure (defined below) and the nonperforming Party as promptly as practicable provides notice of the prevention to the other Party. Such excuse shall be continued so long as the condition constituting force majeure continues and the nonperforming Party takes Commercially Reasonable Efforts to remove the condition. For purposes of this Agreement, "force majeure" shall include conditions beyond the control of the Parties, including an act of God, voluntary or involuntary compliance with, or change in, any regulation, law or order of any government, omissions or delays in acting by any Regulatory Authority or other Governmental Authority or from the other Party, war, terrorism, civil commotion, riot, labor strike or lock-out, unavailability of raw materials, embargo, epidemic, failure or default of public utilities or common carriers, destruction of production facilities or materials by fire, flood, earthquake, storm or like catastrophe.

**18.4. Correspondence and Notices**.

18.4.1. Ordinary Notices. Subject to the provisions of Section 18.4.2, correspondence, reports, documentation and any other communication in writing between the Parties in the course of ordinary implementation of this Agreement shall be delivered by hand, sent by registered or certified mail (return receipt requested) postage prepaid or sent using a nationally recognized express courier service, in each case to the employee or representative of the other Party who is designated by such other Party to receive such written communication.

18.4.2. Extraordinary Notices. Any notice or notification required or permitted to be provided pursuant to the terms and conditions of this Agreement (including, without limitation, any notice of force majeure, breach, termination, change of address, etc.) shall be in writing and shall be deemed given upon receipt if delivered personally or by facsimile transmission (receipt verified), five (5) days after deposited in the mail if mailed by registered or certified mail (return receipt requested) postage prepaid, or on the next Business Day if sent by overnight delivery using a nationally recognized express courier service and specifying next business day delivery (receipt verified), to the Parties at the following addresses or facsimile numbers (or at such other address or facsimile number for a Party as shall be specified by like notice; provided, however, that notices of a change of address shall be effective only upon receipt thereof):

All correspondence to Biogen shall be addressed as follows:

Biogen MA Inc.
225 Binney Street
Cambridge, Massachusetts 02142
Attn: General Counsel
Fax: (866) 546-2758

with a copy to:

Marc Rubenstein
Ropes & Gray LLP
Prudential Tower, 800 Boylston Street
Boston, MA 02199-3600
Telephone: 617-951-7826
Facsimile: 617-235-0706

All correspondence to AGTC shall be addressed as follows:

Applied Genetic Technologies Corporation
11801 Research Drive
Suite D
Alachua, Florida 32615
Attn: Larry Bullock, Chief Financial Officer

with a copy to:

Hemmie Chang
Foley Hoag LLP
Seaport West, 155 Seaport Boulevard
Boston, MA 02210-2600
Telephone: 617-832-1175
Facsimile: 617-832-7000

**18.5. Amendment**. No amendment, modification or supplement of any provision of this Agreement shall be valid or effective unless made in writing and signed by a duly authorized officer of each Party.

**18.6. Waiver**. No provision of this Agreement shall be waived by any act, omission or knowledge of a Party or its agents or employees except by an instrument in writing expressly waiving such provision and signed by a duly authorized officer of the waiving Party. The waiver by either of the Parties of any breach of any provision hereof by the other Party shall not be construed to be a waiver of any succeeding breach of such provision or a waiver of the provision itself.

**18.7. Severability**. If any clause or portion thereof in this Agreement is for any reason held to be invalid, illegal or unenforceable, the same shall not affect any other portion of this Agreement, as it is the intent of the Parties that this Agreement shall be construed in such fashion as to maintain its existence, validity and enforceability to the greatest extent permitted by law. In any such event, this Agreement shall be construed as if such clause of portion thereof had never been contained in this Agreement, and (after negotiation by the parties) there shall be deemed substituted therefor such provision as will most nearly carry out the intent of the Parties as expressed in this Agreement to the fullest extent permitted by applicable law.

**18.8. Descriptive Headings**. The descriptive headings of this Agreement are for convenience only and shall be of no force or effect in construing or interpreting any of the provisions of this Agreement.

**18.9. Export Control**. This Agreement is made subject to any restrictions concerning the export of products or technical information from the United States of America or other countries which may be imposed upon or related to AGTC or Biogen from time to time. Each Party agrees that it will not export, directly or indirectly, any technical information acquired from the other Party under this Agreement or any products using such technical information to a location or in a manner that at the time of export requires an export license or other governmental approval, without first obtaining the written consent to do so from the appropriate agency or other governmental entity. Specifically, each Party understands that the Arms Export Control Act (AECA), including its implementing International Traffic In Arms Regulations (ITAR) and the Export Administration Act (EAA), including its Export Administration Regulations (EAR), are some (but not all) of the laws and regulations that comprise the U.S. export laws and regulations. Each Party further understands that the U.S. export laws and regulations include (but are not limited to): (I) ITAR and EAR product/service/data-specific requirements; (2) ITAR and EAR ultimate destination-specific requirements; (3) ITAR and EAR end user-specific requirements; (4) ITAR and EAR end use-specific requirements; (5) Foreign Corrupt Practices Act; and (6) anti-boycott laws and regulations. Each Party will comply with all then-current applicable export laws and regulations of the U.S. Government (and other applicable U.S. laws and regulations) pertaining to the patents and products licensed under the [***] Agreement (including any associated products, items, articles, computer software, media, services, technical data, and other information). Each Party certifies that it will not, directly or indirectly, export (including any deemed export), nor re-export (including any deemed re-export) such patents or products (including any associated products, items, articles, computer software, media, services, technical data, and other information) in violation of U.S. export laws and regulations or other applicable U.S. laws and regulations.

**18.10. Governing Law**. This Agreement, and all claims arising under or in connection therewith, shall be governed by and interpreted in accordance with the substantive laws of the State of Delaware, without regard to conflict of law principles thereof.

**18.11. Entire Agreement**. This Agreement, together with all related agreements referenced herein, constitutes and contains the complete, final and exclusive understanding and agreement of the Parties and cancels and supersedes any and all prior negotiations, correspondence, understandings and agreements, whether oral or written, between the Parties respecting the subject matter hereof and thereof, including that certain Mutual Confidentiality Agreement between the Parties dated May 27, 2014 which is hereby superseded and replaced in its entirety as of the Effective Date, and any Confidential Information disclosed by the Parties under such Mutual Confidentiality Agreement shall be treated in accordance with the provisions of Article 14.

**18.12. Independent Contractors**. Both Parties are independent contractors under this Agreement. Nothing herein contained shall be deemed to create an employment, agency, joint venture or partnership relationship between the Parties hereto or any of their agents or employees, or any other legal arrangement that would impose liability upon one Party for the act or failure to act of the other Party. Neither Party shall have any express or implied power to enter into any contracts or commitments or to incur any liabilities in the name of, or on behalf of, the other Party, or to bind the other Party in any respect whatsoever.

**18.13. Counterparts**. This Agreement may be executed in two (2) counterparts, each of which shall be an original and both of which shall constitute together the same document. Counterparts may be signed and delivered by facsimile, each of which shall be binding when received by the applicable Party.

**18.14. Interpretation**. Except where the context expressly requires otherwise, (a) the use of any gender herein shall be deemed to encompass references to either or both genders, and the use of the singular shall be deemed to include the plural (and vice versa), (b) the words "include", "includes" and "including" shall be deemed to be followed by the phrase "without limitation," (c) the word "will" shall be construed to have the same meaning and effect as the word "shall," (d) any definition of or reference to any agreement, instrument or other document herein shall be construed as referring to such agreement, instrument or other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein), (e) any reference herein to any Person shall be construed to include the Person's successors and assigns, (f) the words "herein", "hereof" and "hereunder", and words of similar import, shall be construed to refer to this Agreement in its entirety and not to any particular provision hereof, (g) all references herein to Sections or Exhibits shall be construed to refer to Sections or Exhibits of this Agreement, and references to this Agreement include all Exhibits hereto, (h) the word "notice" shall mean notice in writing (whether or not specifically stated) and shall include notices, consents, approvals and other written communications contemplated under this Agreement, (i) provisions that require that a Party, the Parties or any committee hereunder "agree," "consent" or "approve" or the like shall require that such agreement, consent or approval be specific and in writing, whether by written agreement, letter, approved minutes or otherwise (but excluding e-mail and instant messaging), (j) references to any specific law, rule or regulation, or article, section or other division thereof, shall be deemed to include the then-current amendments thereto or any replacement or successor law, rule or regulation thereof, (k) any definition of or reference to any agreement, instrument or other document herein shall be construed as referring to such agreement, instrument or other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein), and (l) the term "or" shall be interpreted in the inclusive sense commonly associated with the term "and/or."

**18.15. No Third Party Rights or Obligations**. No provision of this Agreement shall be deemed or construed in any way to result in the creation of any rights or obligation in any Person not a Party to this Agreement, provided that each Person indemnified by either Party under Article 17 is an intended Third Party beneficiary for the sole purpose of enforcing such indemnification.

**18.16. Remedies Cumulative**. All rights and remedies of each Party under this Agreement will be cumulative and non-exclusive of any other rights or remedies available to such Party at law or in equity or provided for in this Agreement.

**18.17. Further Actions**. Each Party agrees to execute, acknowledge and deliver such further instruments, and to do all such other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

[*Signature page follows.*]

IN WITNESS WHEREOF, duly authorized representatives of the Parties have duly executed this Agreement to be effective as of the Effective Date.

| **BIOGEN MA INC.** | | **APPLIED GENETIC TECHNOLOGIES CORPORATION** | |
|---|---|---|---|
| By | /s/ Douglas Williams | By | /s/ Susan B. Washer |
| Name: | Douglas Williams, Ph.D. | Name: | Susan B. Washer |
| Title: | Executive Vice President, Research and Development | Title: | President and CEO |

CONFIDENTIAL MATERIALS OMITTED AND FILED SEPARATELY WITH THE SECURITIES AND EXCHANGE COMMISSION. TRIPLE ASTERISKS [***] DENOTE OMISSIONS.

**SCHEDULE 1.22-1**

AGTC PATENT RIGHTS FOR THE INITIAL LICENSED PRODUCTS

**(i) AGTC Owned Patents**

**[***]**

**(ii) Co-Owned Patent Rights**

**[***]**

**(iii) In-licensed Patents**

[***]

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**SCHEDULE 1.22-2**

AGTC PATENT RIGHTS FOR THE DISCOVERY PRODUCTS

[*To be included as of the Option Exercise Date.*]

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**SCHEDULE 1.23**

AGTC PLATFORM

The AGTC Platform is further described as follows:

1. "[***] Manufacturing Patent Rights" has the meaning set forth in Section 1.140 and, as of the Execution Date, consists of the following Patent Rights:

(i) AGTC Owned Patents

[***]

(ii) Co-Owned Patent Rights

[***]

(iii) In-licensed Patents

[***]

2. "Capsid Optimization Patent Rights" has the meaning set forth in Section 1.52 and, as of the Execution Date, consists of the following Patent Rights:

[***]

3. "Promoter Patent Rights" has the meaning set forth in Section 1.216 and, as of the Execution Date, consists of the following Patent Rights:

None as of the Execution Date.

4. "AGTC Assays" has the meaning set forth in Section 1.15 and, as of the Execution Date, consists of the following assays:

**[***]**

**SCHEDULE 1.40**

BIOGEN PATENT RIGHTS

*None as of the Execution Date.*

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**SCHEDULE 1.56**

CLINICAL CANDIDATE DESIGNATION CRITERIA

[***]

**SCHEDULE 1.212**

PRODUCT-SPECIFIC PATENT RIGHTS OF AGTC

[***]

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**SCHEDULE 3.1.3**

BIOGEN STEP-IN EVENTS

[***]

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**SCHEDULE 4.2.1**

LICENSED PATENT RIGHTS FOR THE DISCOVERY PROGRAMS

1. ALD/[***] Discovery Program

   [***]

2. [***] Discovery Program I

   [***]

3. [***] Discovery Program II

   [***]

4. [***] Discovery Program

   [***]

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**SCHEDULE 5.2**

SUBLICENSING RESTRICTIONS

1. [***] Agreement

With respect to AGTC Technology sublicensed to Biogen pursuant to the [***] Agreement, Biogen may grant further sublicenses to such AGTC Technology through itself or its Affiliates to Third Parties, provided that each sublicense agreement: (a) shall incorporate by reference the terms and conditions of the [***] Agreement as set forth in this Agreement, (b) shall be consistent with the terms, conditions and limitations of the [***] Agreement, (c) shall name [***] and [***] as intended third party beneficiaries with respect to the indemnification obligations of the Sublicensee, (d) shall include a prohibition from further sublicensing the rights delivered thereunder, and (e) shall comply with the applicable provisions of Section 5.5.4 of this Agreement. Biogen agrees to provide a copy of each executed sublicense agreement to AGTC for delivery to [***] and [***] (which copy may be redacted for Biogen's, its Affiliate's or any Sublicensee's confidential information, for information regarding intellectual property that is unrelated to the AGTC Patent Rights licensed under the [***] Agreement or other confidential information not necessary for [***] and [***] to ensure compliance with the [***] Agreement). Notwithstanding anything to the contrary, Biogen and any Sublicensee shall be free, without notice or consent, to engage distributors or to sublicense to contractors or collaborators for the purpose of manufacturing, research, development or any other purpose other than granting sublicense rights to commercialize or sell Licensed Products to Third Parties, provided that the provisions of this paragraph in this Schedule 5.2 shall be incorporated into each such sublicense agreement.

2. [***] Agreement

With respect to AGTC Technology sublicensed to Biogen pursuant to the [***] Agreement, Biogen may grant further sublicenses to such AGTC Technology through itself or its Affiliates to Third Parties, provided that, in the case of Biogen granting rights to commercialize or sell Licensed Products to a Sublicensee, Biogen shall notify [***] of the identity of such Sublicensee within thirty (30) days after the grant of such further sublicense. Further, in the event Biogen grants such a further sublicense of commercialization rights to a Sublicensee, any such downstream sublicense agreement must require the Sublicensee to comply with the terms of the [***] Agreement as set forth in this Agreement. For clarity, any Sublicensee of Biogen shall be free, without notice or consent, to engage distributors or to sublicense to contractors or collaborators for the purpose of manufacturing.

**SCHEDULE 11.4**

THIRD PARTY MATERIALS

(a)

[***] Biological Materials:

[***]

(b)

[***] Materials:

[***]

(c)

[***]

(d)

[***]

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**SCHEDULE 11.4.2**

[***]RESTRICTIONS

[***]

**SCHEDULE 15.1**

MUTUAL DISCLOSURE SCHEDULE

(a) AGTC Disclosures

[***].

(b) Biogen Disclosures

[***]

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**SCHEDULE 15.2**

AGTC DISCLOSURE SCHEDULE

15.2.4

[***]

15.2.20

[***]

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**SCHEDULE 15.2.7**

EXISTING LICENSE AGREEMENTS

UFRF Agreements

1. Standard Exclusive License Agreement With Know How - [***] Vectors License (A12044), dated November 5, 2012, by and between AGTC and University of Florida Research Foundation, Inc.
    a. Amendment - January 30, 2014
    b. Amendment – June 30, 2015 (Omnibus Amendment)
2. Standard Non-Exclusive License Agreement (A10571), dated September 18, 2012, by and between AGTC and University of Florida Research Foundation, Inc.
    a. Amendment – June 30, 2015 (Omnibus Amendment)

UFRF/JHU Agreements

3. Standard Exclusive License Agreement With Sublicensing Terms (A3288), dated October 7, 2003, by and among AGTC, University of Florida Research Foundation, Inc. and Johns Hopkins University
    a. Amendment - November 2004 (First Amendment)
    b. Amendment - December 3, 2004 (Side Letter)
    c. Amendment - February 25, 2009 (Second Amendment)
    d. Amendment - March 30, 2010 (Third Amendment)
    e. Amendment - December 17, 2013 (Fourth Amendment)
    f. Amendment – July 1, 2015 (Omnibus Amendment)
4. [***] Agreement, dated March 13, 2014, by and among AGTC, University of Florida Board of Trustees and Johns Hopkins University
    a. Amendment – July 1, 2015 (Omnibus Amendment)

[***]Agreements

[***]

UAB Agreements

5. Non-Exclusive License Agreement with Sublicensing Terms [***], dated January 19, 2006, by and between AGTC and UAB Research Foundation
    a. Amendment - March 28, 2014 (First Amendment)
    b. Amendment – June 29, 2015 (Second Amendment)
    c. Side Letter – June 29, 2015 (Request Letter)

[***] Agreements

[***]

[***] Agreements

[***]

**EXHIBIT A-1**

INITIAL XLRS DEVELOPMENT PLAN

[***]

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**EXHIBIT A-2**

INITIAL XLRP DEVELOPMENT PLAN

[***]

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**EXHIBIT A-3**

INITIAL [***] DISCOVERY PROGRAM DEVELOPMENT PLAN

[***]

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**EXHIBIT A-4**

INITIAL ALD/[***] DISCOVERY PROGRAM DEVELOPMENT PLAN

CONFIDENTIAL MATERIALS OMITTED AND FILED SEPARATELY WITH THE SECURITIES AND EXCHANGE COMMISSION. TRIPLE ASTERISKS [***] DENOTE OMISSIONS.

**EXHIBIT A-5**

INITIAL [***]DEVELOPMENT PLAN

[***]

CONFIDENTIAL MATERIALS OMITTED AND FILED SEPARATELY WITH THE SECURITIES AND EXCHANGE COMMISSION. TRIPLE ASTERISKS [***] DENOTE OMISSIONS.

**EXHIBIT A-6**

INITIAL [***]DEVELOPMENT PLAN

[***]

CONFIDENTIAL MATERIALS OMITTED AND FILED SEPARATELY WITH THE SECURITIES AND EXCHANGE COMMISSION. TRIPLE ASTERISKS [***] DENOTE OMISSIONS.

**EXHIBIT B**

CO-PROMOTION TERMS

[***]

CONFIDENTIAL MATERIALS OMITTED AND FILED SEPARATELY WITH THE SECURITIES AND EXCHANGE COMMISSION. TRIPLE ASTERISKS [***] DENOTE OMISSIONS.

**EXHIBIT C**

FINANCIAL PLANNING, ACCOUNTING AND REPORTING FOR THE COST SHARE PRODUCT(S)

1. [***]

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**EXHIBIT D**

PRESS RELEASE

## BIOGEN AND AGTC ENTER COLLABORATION TO DEVELOP GENE THERAPIES IN OPHTHALMOLOGY

*Companies to advance a potentially transformative treatment approach for genetic diseases of the eye*

*AGTC to receive $124M upfront, with potential future milestone payments and royalties*

*AGTC to host conference call today at 8 a.m. EDT*

**CAMBRIDGE, Mass. & GAINESVILLE, Fla. – July 1, 2015** – Biogen (NASDAQ: BIIB) and AGTC (NASDAQ: AGTC) today announced a broad collaboration and license agreement to develop gene-based therapies for multiple ophthalmic diseases. The collaboration will focus on the development of a portfolio of AGTC's therapeutic programs, including both a clinical stage candidate and a pre-clinical candidate for orphan diseases of the retina that can lead to blindness in children and adults. The agreement also includes options for early stage discovery programs in two ophthalmic diseases and one non-ophthalmic condition, as well as an equity investment in AGTC by Biogen and a license agreement for manufacturing rights.

"With this collaboration, we hope to advance gene therapies to open possibilities for patients who suffer from diseases that are well understood, but have no adequate treatment," said Olivier Danos, Ph.D., senior vice president, cell & gene therapy at Biogen. "AGTC is an exceptional partner to help us advance our gene therapy capabilities by targeting diseases of the eye – an organ that provides an ideal setting for the localized, selective delivery of gene-based therapies."

"We expect this collaboration will further validate our novel adeno-associated virus (AAV) gene therapy platform and support the development of new therapies that may allow for transformative treatments for these rare inherited eye diseases and other clinical indications," added Sue Washer, president and CEO of AGTC. "Biogen's significant commitment to advancing gene therapies and demonstrated success in developing innovative therapies to treat complex diseases, combined with our proprietary manufacturing technology and extensive gene therapy experience, makes this an ideal partnership."

The lead development programs in the collaboration include a clinical candidate for X-linked Retinoschisis (XLRS) and a pre-clinical candidate for the treatment of X-Linked Retinitis Pigmentosa (XLRP). XLRS, a disease affecting young males beginning during the teenage years, can lead to serious complications such as vitreous hemorrhage or retinal detachment during adulthood. XLRP usually causes night blindness by the age of ten and progresses to legal blindness by an individual's early forties. Both conditions represent significant unmet needs that may be addressed by replacing the single, faulty gene causing each disease.

**Collaboration Overview**
Biogen will make an upfront payment in the amount of $124 million to AGTC, which includes a $30 million equity investment in AGTC at a price equal to $20.63 per share and certain prepaid research and development expenditures. Biogen will be granted a license to the XLRS and XLRP programs and the option to license discovery programs for three additional indications at the time of clinical candidate selection.

Under the collaboration, AGTC is eligible to receive upfront and milestone payments exceeding $1 billion. This includes up to $472.5 million collectively for the two lead programs, which also will carry royalties in the high single digit to mid-teen percentages of annual net sales. In addition, Biogen will make payments up to $592.5 million across the discovery programs, along with royalties in the mid single digits to low teen percentages of annual net sales.

Biogen obtains worldwide commercialization rights for the XLRS and XLRP programs. AGTC has an option to share development costs and profits after the initial clinical trial data are available, and an option to co-promote the second of these products to be approved in the United States. AGTC will lead the clinical development programs of XLRS through product approval and of XLRP through the completion of first-in-human trials. Biogen will support the clinical development costs, subject to certain conditions, following the first-in-human study for XLRS and IND-enabling studies for XLRP. Under the manufacturing license, Biogen will receive an exclusive license to use AGTC's proprietary technology platform to make AAV vectors for up to six genes, three of which are in AGTC's discretion, in exchange for payment of milestones and royalties.

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The transaction is subject to customary closing conditions, including the expiration of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 in the United States, and is expected to close in the third calendar quarter of 2015.

AGTC will host a live webcast presentation and conference call on [July # at ##:## a.m. EDT] to discuss the collaboration. The webcast can be accessed at ir.agtc.com/events.cfm or by dialing [(###) ###-#### (US) or (###) ###-#### (outside of the US)] fifteen minutes prior to the start of the call. The passcode is [######.] The webcast will be archived on the AGTC website.

**About Gene Therapy**
Gene therapy is an evolving field of medicine in which faulty genes are corrected in cells. Genes control heredity and provide the basic biological code for determining a cell's specific functions. The most common form of gene therapy involves using DNA that encodes a functional, therapeutic gene to replace a defective gene. In gene therapy, the healthy copy of a defective gene is packaged within a vector, a biological delivery mechanism which is used to transport the genetic information into the diseased cells within the body. Once the gene is delivered into the correct cell, a therapeutic protein is naturally made by the cell from the therapeutic gene.

**About Adeno-Associated Virus (AAV) Vectors**
AAV vectors have emerged as an attractive approach for gene therapy since they can deliver the genes for therapeutic proteins to accessible tissues in the body. Several AAV gene therapy products are in late-stage clinical development, and one product is approved in the EU.

**About Biogen**
Through cutting-edge science and medicine, Biogen discovers, develops and delivers to patients worldwide innovative therapies for the treatment of neurodegenerative diseases, hematologic conditions and autoimmune disorders. Founded in 1978, Biogen is one of the world's oldest independent biotechnology companies, and patients worldwide benefit from its leading multiple sclerosis and innovative hemophilia therapies. For product labeling, press releases and additional information about the company, please visit www.biogen.com.

**About AGTC**
AGTC is a clinical-stage biotechnology company that uses its proprietary gene therapy platform to develop products designed to transform the lives of patients with severe diseases in ophthalmology. AGTC's lead product candidates focus on X-linked retinoschisis, achromatopsia and X-linked retinitis pigmentosa, which are inherited orphan diseases of the eye, caused by mutations in single genes that significantly affect visual function and currently lack effective medical treatments. AGTC is also using its gene therapy expertise to expand into disease indications with large market opportunity such as wet AMD and other ophthalmology and orphan indications.

**Biogen Safe Harbor**
This press release contains forward-looking statements, including statements about the potential benefits and advancements that may be achieved through the collaboration with AGTC and the expected timing of the closing the transactions. These statements may be identified by words such as "believe," "expect," "may," "plan," "potential," "will" and similar expressions, and are based on Biogen's current beliefs and expectations. These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements. Risks and uncertainties that may cause actual results to differ materially include, among others: uncertainty inherent in the regulatory review process and satisfaction of other closing conditions relating to the transactions; uncertainty regarding the ability to achieve the expected benefits from the proposed collaboration, including as a result of risks and uncertainties associated with drug development and commercialization, reliance on third parties over which Biogen may not always have full control and other risks associated with collaborations; and other risks and uncertainties that are described in the Risk Factors section of Biogen's most recent annual or quarterly report filed with the Securities and Exchange Commission. Any forward-looking statements speak only as of the date of this press release and Biogen assumes no obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

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**AGTC Safe Harbor**

This release contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs. Forward-looking statements include information concerning the expected timing of the closing of the transactions contemplated by the proposed collaboration, possible or assumed future results of operations, business strategies and operations, preclinical and clinical product development and regulatory progress, potential growth opportunities, potential market opportunities and the effects of competition. Forward-looking statements include all statements that are not historical facts and can be identified by terms such as "anticipates," "believes," "could," "seeks," "estimates," "expects," "intends," "may," "plans," "potential," "predicts," "projects," "should," "will," "would" or similar expressions and the negatives of those terms. Actual results could differ materially from those discussed in the forward-looking statements, due to a number of important factors. Risks and uncertainties that may cause actual results to differ materially include, among others: uncertainty inherent in the regulatory review process and satisfaction of other closing conditions relating to the transactions; uncertainty regarding the ability to achieve the expected benefits from the proposed collaboration, including as a result of risks and uncertainties associated with drug development and commercialization, reliance on third parties over which AGTC may not always have full control and other risks associated with collaborations; and other risks and uncertainties that are described under the heading "Risk Factors" in AGTC's Annual Report on Form 10-K for the fiscal year ended June 30, 2014, as filed with the SEC. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent management's plans, estimates, assumptions and beliefs only as of the date of this release. Except as required by law, we assume no obligation to update these forward-looking statements publicly or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

###

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**EXHIBIT E**

TAX MATTER PARTNERSHIP TERMS

[***]

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**Exhibit 10.7**

***EXECUTION VERSION***

***CONFIDENTIAL***

# MANUFACTURING LICENSE AND TECHNOLOGY TRANSFER AGREEMENT

This Manufacturing License and Technology Transfer Agreement ("Agreement") is entered into as of July 1, 2015 (the "Execution Date"), and effective as of the Effective Date, by and between Applied Genetic Technologies Corporation, having a place of business at 11801 Research Drive, Suite D, Alachua, FL 32615 ("AGTC") and Biogen MA Inc. ("LICENSEE"), having a place of business at 250 Binney Street, Cambridge, MA 02142. AGTC and LICENSEE are referred to collectively hereinafter as the "Parties" and individually as a "Party".

## RECITALS

WHEREAS, AGTC has developed expertise and acquired intellectual property rights related to the design, development and manufacture of AAV Products for use in delivering gene therapeutics;

WHEREAS, simultaneously with the execution of this Agreement, the Parties are executing a Collaboration and License Agreement (the "Collaboration Agreement") under which the technology to be transferred under this Agreement is licensed to LICENSEE for different products and uses and a Common Stock Purchase Agreement under which shares of common stock of AGTC shall be issued to LICENSEE; and

WHEREAS, LICENSEE wishes to license from AGTC intellectual property related to the design, development and manufacture of AAV Products, and AGTC wishes to license to LICENSEE such intellectual property, each on the terms and conditions set forth herein;

NOW, THEREFORE, in consideration of the foregoing and the mutual promises and covenants hereinafter set forth, AGTC and LICENSEE, intending to be legally bound, hereby agree as follows:

## ARTICLE I
## DEFINITIONS

For purposes of this Agreement, all capitalized terms used herein and not otherwise defined shall have the meanings set forth below:

1.1 "1934 Act" has the meaning set forth in Section 12.5.

1.2 "AAV" means adeno-associated virus.

1.3 "AAV Product" means any product containing a recombinant AAV or AAV-based vector that delivers one or more transgenes or portions thereof to a human or animal subject.

1.4 "Additional Taxes" has the meaning set forth in Section 5.8(b).

1.5 "Affiliate" means, as of any point in time and for so long as such relationship continues to exist with respect to any Person, any other Person that controls, is controlled by or is under common control with such Person. A Person shall be regarded as in control of another Person if it (a) owns or controls more than fifty percent (50%) of the equity securities of the subject Person entitled to vote in the election of directors (or, in the case of a Person that is not a corporation, for the election of the corresponding managing authority); provided, however, that in such circumstance, the term "Affiliate" shall not include subsidiaries or other entities in which a Person owns a majority of the ordinary voting power necessary to elect a majority of the board of directors or other governing board, but is subject to a contractual or other restriction that causes such Person to be unable to elect such majority, until such time as such restriction is no longer in effect; or (b) possesses, directly or indirectly, the power to direct or cause the direction of the management or policies of an such Person (whether through ownership of securities or other ownership interests, by contract or otherwise).

1.6 "Agreement" has the meaning set forth in the Preamble.

1.7 "AGTC" has the meaning set forth in the Preamble.

1.8 "AGTC Indemnified Party" has the meaning set forth in Section 10.1.

1.9 "AGTC Protectable Product" means any AAV Product (a) for which AGTC or any of its Affiliates or licensees has, at the time of the Selection Response for any Gene of Interest, Developed such AAV Product at least to the point of [***] and (b) that has been disclosed to LICENSEE pursuant to Section 2.2. Any AAV Product that meets the requirements of clause (a) but has not been disclosed to LICENSEE pursuant to Section 2.2 shall not be an AGTC Protectable Product unless and until it is so disclosed.

1.10 "AGTC Third Party Agreement" means any agreement between AGTC (or any of its Affiliates) and any Third Party pursuant to which AGTC has acquired, or, during the Term, acquires, Control of any of the [***] Manufacturing Technology, including the Existing License Agreements.

1.11 "Audited Party" has the meaning set forth in Section 5.7(a).

1.12 "Auditing Party" has the meaning set forth in Section 5.7(a).

1.13 "Available Gene of Interest" means any Gene of Interest for which AGTC has not, at the time that a Selection Request is delivered by LICENSEE, (a) already granted rights to or entered into a fully executed term sheet (which may be a non-binding term sheet) contemplating the grant of rights to a Third Party that would preclude the granting of rights to LICENSEE for such Gene of Interest as a Selected Gene to the extent contemplated by this Agreement or (b) [***] for purposes of selecting a candidate AAV [***] involving such Gene of Interest.

1.14 "BLA" means a Biologics License Application (as defined in 21 C.F.R. 600 et. seq.), NDA, MAA or substantially similar application or submission filed with a Regulatory Authority in a country or group of countries, and any amendments thereto.

1.15 "Business Day" means a day other than a Saturday, Sunday or bank or other public holiday in New York, New York.

1.16 "Calendar Quarter" means the respective periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 or December 31, for so long as this Agreement is in effect.

1.17 "Calendar Year" means any calendar year ending on December 31.

1.18 "Change of Control" means, with respect to a Party, (a) a merger or consolidation of such Party with a Third Party that results in the voting securities of such Party outstanding immediately prior thereto, or any securities into which such voting securities have been converted or exchanged, ceasing to represent more than fifty percent (50%) of the combined voting power of the surviving entity or the parent of the surviving entity immediately after such merger or consolidation, or (b) a transaction or series of related transactions in which a Third Party, together with its Affiliates, becomes the beneficial owner of more than fifty percent (50%) of the combined voting power of the outstanding securities of such Party, or (c) the sale or other transfer to a Third Party of all or substantially all of such Party's business or assets relating to one or more Products or the [***] Manufacturing Technology.

1.19 "Clinical Trial" means a human clinical study conducted on sufficient numbers of human subjects that is designed to (a) establish that a pharmaceutical product is reasonably safe for continued testing, (b) investigate the safety and efficacy of the pharmaceutical product for its intended use, and to define warnings, precautions and adverse reactions that may be associated with the pharmaceutical product in the dosage range to be prescribed or (c) support Regulatory Approval of such pharmaceutical product or label expansion of such pharmaceutical product. Without limiting the foregoing, Clinical Trial includes any FIH Trial or Pivotal Trial.

1.20 "Collaboration Agreement" has the meaning set forth in the Recitals.

1.21 "Collaboration Program" has the meaning set forth in the Collaboration Agreement.

1.22 "Combination Product" means (a) any single product in finished form containing as active ingredients both a Product and one or more other pharmaceutically active compounds or substances (including, for the avoidance of doubt, a transgene other than a transgene of a Selected Gene), whether co-formulated or co-packaged (*i.e.*, within a single box or sales unit); or (b) any Product sold in combination with one or more other products (such as devices) or services for a single invoice price; or (c) any Product sold where the sale of the Product is only available with the purchase of other products or services (such other pharmaceutically active compounds or substances, or such other products or services referred to in clauses (a) through (c) hereof, the "Other Components").

1.23 "Commercialize" or "Commercializing" means to market, advertise, promote, distribute, offer for sale, sell, have sold, import, lease, export or otherwise commercialize a product, to conduct activities, other than Development and Manufacturing, in preparation for the foregoing activities, and to conduct post-approval studies. When used as a noun, "Commercialization" shall mean any and all activities involved in Commercializing.

1.24 "Commercially Reasonable Efforts" means, with respect to each Party, the efforts and resources typically used by biotechnology or biopharmaceutical companies similar in size and scope to such Party and its Affiliates to perform the obligation at issue, which efforts shall not be less than those efforts made with respect to other products at a similar stage of development or in a similar stage of product life, with similar developmental risk profiles, of similar market and commercial potential, taking into account the competitiveness of the market place, the proprietary position of the products, the regulatory structure involved, Regulatory Authority-approved labeling, product profile, the profitability of the applicable products (taking into account payments under this Agreement), issues of safety and efficacy, the likely timing of the product's entry into the market, the likelihood of receiving Regulatory Approval and other relevant scientific, technical and commercial factors.

1.25 "Competing Program" means any program involving the Development or Commercialization of an AAV Product [***] as an AGTC Protectable Product.

1.26 "Competitive Infringement" has the meaning set forth in Section 8.6(b).

1.27 "Confidential Information" means, with respect to each Party, all Know-How or other information, including proprietary information (whether or not patentable) regarding or embodying such Party's technology, products, business information or objectives, that is communicated in any way or form by or on behalf of the Disclosing Party to the Receiving Party or its permitted recipients, on or after the Effective Date of this Agreement, whether or not such Know-How or other information is identified as confidential at the time of disclosure, provided that Know-How or other information not identified as confidential by or on behalf of the Disclosing Party shall be deemed to be Confidential Information of the Disclosing Party if the Receiving Party knows, or should have had a reasonable expectation, that such Know-How or other information communicated by or on behalf of the Disclosing Party is Confidential Information of the Disclosing Party. The terms and conditions of this Agreement shall be considered Confidential Information of both Parties. Notwithstanding any provision of this Section 1.27 to the contrary, Confidential Information does not include any (a) Joint Know-How or (b) Know-How or information that: (i) was already known by the Receiving Party (other than under an obligation of confidentiality to the Disclosing Party) at the time of disclosure by or on behalf of the Disclosing Party; (ii) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party; (iii) became generally available to the public or otherwise part of the public domain after its disclosure to the Receiving Party, other than through any act or omission of the Receiving Party in breach of its obligations under this Agreement; (iv) was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation not to disclose such information to the Receiving Party; or (v) was independently discovered or

developed by or on behalf of the Receiving Party without the use of or access to any Confidential Information belonging to the Disclosing Party.

1.28 "Consented Gene" has the meaning set forth in Section 2.4.

1.29 "Continuing Party" has the meaning set forth in Section 8.5(b).

1.30 "Control" or "Controlled" means with respect to any intellectual property right (including any Patent Right, Know-How or other data, information or Materials), possession of the ability (whether by sole or joint ownership, license or otherwise, other than pursuant to the license grants under this Agreement) to grant, without violating the terms of any agreement with a Third Party, a license, access or other right in, to or under such intellectual property right. Notwithstanding anything in this Agreement to the contrary, a Party shall be deemed to not Control any Patent Rights or Know-How that are owned or controlled by a Third Party described in the definition of "Change of Control", or such Third Party's Affiliates, (a) prior to the closing of such Change of Control, except to the extent that any such Patent Rights or Know-How were developed prior to such Change of Control through the use of such Party's technology, or (b) after such Change of Control to the extent that such Patent Rights or Know-How are developed or conceived by such Third Party or its Affiliates (other than such Party) after such Change of Control without using or incorporating or having access to such Party's technology.

1.31 "Cost of Goods Sold" means, as to each Product, the fully burdened cost of such Product in final therapeutic form. The fully burdened cost of each Product will be determined in accordance with U.S. GAAP as applied by the Party performing or contracting for each stage of the Manufacturing process and will include direct labor, material, product testing costs and allocable overhead.

1.32 "Cost Share Product" has the meaning set forth in the Collaboration Agreement.

1.33 "Cover," "Covering" or "Covers" means, as to a product and Patent Rights, that, in the absence of a license granted under, or ownership of, such Patent Rights, the making, using, selling, offering for sale or importation of such product would infringe such Patent Rights or, as to a pending claim included in such Patent Rights, the making, using, selling, offering for sale or importation of such product would infringe such Patent Rights if such pending claim were to issue in an issued patent without modification.

1.34 "Declining Party" has the meaning set forth in Section 8.5(b).

1.35 "Develop" or "Developing" means to discover, research or otherwise develop a product, including conducting non-clinical and clinical research and development activities such as toxicology, pharmacology and other discovery efforts, test method development and stability testing, process development, formulation development, delivery system development, quality assurance and quality control development, statistical analysis, clinical studies (including pre-approval studies), regulatory affairs, pharmacovigilance and Regulatory Approval and clinical study regulatory activities (including regulatory

activities directed to obtaining pricing and reimbursement approvals). When used as a noun, "Development" shall mean any and all activities involved in Developing.

1.36 "Disclosing Party" has the meaning set forth in Section 12.1.

1.37 "Distributor" means any Third Party which purchases its requirements for Product in a country from LICENSEE or its Affiliates or Sublicensees and is appointed as a distributor to distribute, market and resell such Product in such country, even if such Third Party is granted ancillary rights to develop, package or obtain regulatory approvals of such Product in order to distribute, market or sell such Product in such country.

1.38 "Dollar" means the United States Dollar.

1.39 "Effective Date" means the Effective Date of the Collaboration Agreement (as defined therein).

1.40 "Event Milestone Payment" has the meaning set forth in Section 5.2.

1.41 "Execution Date" has the meaning set forth in the Preamble.

1.42 "Existing License Agreements" means those certain license agreements as may be amended from time to time listed on Schedule 1.42.

1.43 "Existing Licensors" means the licensors under the Existing License Agreements.

1.44 "FD&C Act" means the United States Federal Food, Drug, and Cosmetic Act, as amended, and the rules and regulations promulgated thereunder

1.45 "FDA" means the United States Food and Drug Administration or any successor agency thereto.

1.46 "Field" means the diagnosis, treatment or prevention of disease in humans or animals in any and all indications.

1.47 "FIH Trial" means, with respect to a Product, the first Clinical Trial of such Product.

1.48 "First Commercial Sale" means, with respect to any Product and with respect to any country of the Territory, the first sale of such Product by LICENSEE or an Affiliate or Sublicensee of LICENSEE to a Third Party in such country after such Product has been granted Regulatory Approval by the appropriate Regulatory Authority(ies) for Commercialization in such country.

1.49 "GAAP" means United States generally accepted accounting principles, consistently applied.

1.50 "Gene of Interest" means any gene target for which LICENSEE delivers a Selection Request under Section 2.1.

1.51 "Gene Therapy Product" means any product containing a virus-based vector that delivers one or more transgenes to a human or animal subject.

1.52 "Governmental Authority" means any court, agency, department, authority or other instrumentality of any national, state, county, city or other political subdivision.

1.53 "[***]" means [***].

1.54 "[***] Agreements" means the License Agreement, dated [***], by and between AGTC and [***], as may be further amended from time to time, and the License Agreement, dated [***], by and between AGTC and [***], as may be further amended from time to time.

1.55 "[***] Biological Material(s)" has the meaning set forth in Section 4.4(b)(i).

1.56 "[***] Claims" has the meaning set forth in Section 10.3(d).

1.57 "[***] Indemnitees" has the meaning set forth in Section 10.3(d).

1.58 "[***] Product" has the meaning set forth in Section 4.4(b)(i).

1.59 "[***] Virus" has the meaning set forth in Section 4.4(b)(i).

1.60 "HSR Clearance Date" has the meaning set forth in the Collaboration Agreement.

1.61 "[***] Defense Election" has the meaning set forth in Section 8.7(c)(i).

1. 62 "[***] Manufacturing" has the meaning set forth in Section 8.7(e).

1. 63 "[***] Manufacturing Know-How" means all proprietary Know-How, other than Joint Know-How, (a) that (i) AGTC or any of its Affiliates Control as of the Execution Date or (ii) comes into the Control of AGTC or any of its Affiliates during the Term, provided that, in the case of any Know-How under this clause (ii) that comes into the Control of AGTC or its Affiliates through a license to Third Party IP Rights, LICENSEE has elected to take a sublicense to such Third Party IP Rights under Section 8.7(b)(i), (b) that relates to the production, manufacture, or expression of recombinant AAV using an [***] helper virus and (c) that is disclosed to LICENSEE.

1.64 "[***] Manufacturing Patent Right" means any Patent Right, other than a Joint Patent Right, (a) that (i) AGTC or any of its Affiliates Control as of the Execution Date or (ii) comes into the Control of AGTC or any of its Affiliates during the Term, provided that, in the case of any Patent Right under this clause (ii) that comes into the Control of AGTC or its Affiliates through a license to Third Party IP Rights, LICENSEE has elected to take a sublicense to such Third Party IP Rights under Section 8.7(b)(i) and (b) that claims or discloses any [***] Manufacturing Know-How. Notwithstanding the foregoing, Schedule 1.64 sets forth the [***] Manufacturing Patent Rights as of the Execution Date, and will be updated on or prior to the Schedule Revision Date to include additional Patent Rights that becomes [***] Manufacturing Patent Rights after the Execution Date, if any. In addition,

Schedule 1.64 shall be updated by the Patent Representatives on a semi-annual basis to include additional Patent Rights that become [***] Manufacturing Patent Rights after the Schedule Revision Date, provided that any [***] Manufacturing Patent Right that is not listed on Schedule 1.64, but is otherwise described in this Section 1.64, shall still be considered an [***] Manufacturing Patent Right hereunder.

1.65 "[***] Manufacturing Technology" means the [***] Manufacturing Know-How and the [***] Manufacturing Patent Rights.

1.66 "IND" means an Investigational New Drug Application submitted under the FD&C Act, or an analogous application or filing with any analogous agency or Regulatory Authority outside of the United States under any analogous foreign Law for the purposes of obtaining permission to conduct human clinical studies.

1.67 "Indemnified Party" has the meaning set forth in Section 10.4.

1.68 "Indemnifying Party" has the meaning set forth in Section 10.4.

1.69 "Initial Licensed Product" has the meaning set forth in the Collaboration Agreement.

1.70 "Initial Licensed Program" has the meaning set forth in the Collaboration Agreement.

1.71 "Insolvency Event" has the meaning set forth in Section 13.4.

1.72 "Insolvent Party" has the meaning set forth in Section 13.4.

1.73 "Invented" means the act of invention by inventors, in accordance with statutes and regulations regarding inventorship as established under United States patent law, including case law, rules and guidelines associated therewith. "Invent" or "Invents" have correlative meanings.

1.74 "JHU" means John Hopkins University.

1.75 "JHU Inventors" has the meaning set forth in Section 10.3(c).

1.76 "Joint [***] Manufacturing Improvement Know-How" means Joint Know-How that constitutes an improvement or enhancement to the [***] Manufacturing Technology.

1.77 "Joint [***] Manufacturing Improvement Patent Right" means any Patent Right that claims or discloses any Joint [***] Manufacturing Improvement Know-How that is Invented jointly by or on behalf of (i) on the one hand, AGTC or any of its Affiliates or Sublicensees and (ii) on the other hand, LICENSEE or any of its Affiliates or Sublicensees, in each case, in the course of conducting activities under this Agreement.

1.78 "Joint [***] Manufacturing Improvement Technology" means the Joint [***] Manufacturing Improvement Know-How and the Joint [***] Manufacturing Improvement Patent Rights.

1.79 "Joint Know-How" means Know-How that is conceived, discovered, invented, created, made or reduced to practice or tangible medium jointly by or on behalf of (i) on the one hand, AGTC or any of its Affiliates or Sublicensees and (ii) on the other hand, LICENSEE or any of its Affiliates or Sublicensees, in each case, in the course of conducting activities under this Agreement.

1.80 "Joint Patent Right" means any Patent Right that claims or discloses Know-How that is Invented jointly by or on behalf of (i) on the one hand, AGTC or any of its Affiliates or Sublicensees and (ii) on the other hand, LICENSEE or any of its Affiliates or Sublicensees, in each case, in the course of conducting activities under this Agreement.

1.81 "Joint Technology" means the Joint Know-How and the Joint Patent Rights.

1.82 "Know-How" means intellectual property, data, results, pre-clinical and clinical protocols and study data, chemical structures, chemical sequences, information, inventions, know-how, formulas, trade secrets, techniques, methods, processes, procedures and developments, whether or not patentable; except that Know-How does not include Patent Rights claiming any of the foregoing. For clarity, "Know-How" does not include any Materials.

1.83 "Knowledge" means, with respect to AGTC, the then, actual knowledge, after inquiry of patent counsel, but without any other duty of inquiry, of the Chief Executive Officer, Chief Financial Officer, Chief Medical Officer, Chief Business Officer, Chief Scientific Officer and Senior Director – Process Development, Senior Director – Research and Pre-Clinical Studies and any other person performing substantially the same functions as any of the foregoing.

1.84 "Law" means any law, statute, rule, regulation, order, judgment or ordinance of any Governmental Authority.

1.85 "Liability" has the meaning set forth in Section 10.1.

1.86 "LICENSEE" has the meaning set forth in the Preamble.

1.87 "LICENSEE [***] Manufacturing Improvement Know-How" means any Know-How, other than Joint Know-How, that is conceived, discovered, invented, created, made or reduced to practice or tangible medium by or on behalf of LICENSEE or any of its Affiliates or Sublicensees in the course of conducting activities under this Agreement, that constitutes an improvement or enhancement to the [***] Manufacturing Know-How.

1.88 "LICENSEE [***] Manufacturing Improvement Patent Right" means any Patent Right, other than a Joint Patent Right, that claims or discloses any LICENSEE [***] Manufacturing Improvement Know-How that is Invented by or on behalf of LICENSEE or any of its Affiliates or Sublicensees in the course of conducting activities under this Agreement.

1.89 "LICENSEE [***] Manufacturing Improvement Technology" means the LICENSEE [***] Manufacturing Improvement Know-How and the LICENSEE [***] Manufacturing Improvement Patent Rights.

1.90 "LICENSEE Indemnified Party" has the meaning set forth in Section 10.2.

1.91 "LICENSEE Patent Challenge" has the meaning set forth in Section 13.2(b).

1.92 "MAA" means a Marketing Authorization Application for the applicable Product under the centralized European procedure.

1.93 "Major EU Market Countries" means the following countries: [***].

1.94 "Manufacture" or "Manufacturing" means activities directed to making, producing, manufacturing, processing, filling, finishing, packaging, labeling, quality control testing and quality assurance release, shipping or storage of a product.

1.95 "Marketing Application" means an application, submitted to a Regulatory Authority in any jurisdiction, for Regulatory Approval required in order to Commercialize a product as a drug, including a BLA.

1.96 "Materials" means any biological or chemical materials in each case, that are necessary or useful to exploit the licenses granted to LICENSEE under this Agreement including, but not limited to, cell lines (*e.g.*, parental cell lines and any non-commercially available cell lines, for example, the [***]), appropriate rep-cap-, gene of interest- and any other related [***] seed stocks, material-specific reference materials (particularly vectors, plasmids, starting constructs, and any reference plasmid or vector comprising rep2 and cap2), and platform assay reference materials including controls and reagents that are not readily available as standard commercial items.

1.97 "Milestone/Royalty Option" has the meaning set forth in the Collaboration Agreement.

1.98 "NDA" means a New Drug Application (as more fully described in 21 C.F.R. Parts 314 et seq. or its successor regulation).

1.99 "Net Sales" means, with respect to a Product in a country in the Territory, the gross amount invoiced by LICENSEE, its Affiliates or Sublicensees for the sale or other disposition of such Product in such country to Third Parties (including Distributors, wholesalers and end-users), less the following deductions:

(a) sales returns and allowances actually paid, granted or accrued on the Product, including trade, quantity, prompt pay and cash discounts and any other adjustments, including those granted on account of price adjustments or billing errors;

(b) credits or allowances given or made for rejection, recall, return or wastage replacement of, and for uncollectible amounts on, Product or for rebates or retroactive price reductions (including Medicare, Medicaid, managed care and similar types of rebates and chargebacks);

(c) taxes, duties or other governmental charges levied on or measured by the billing amount for Product, as adjusted for rebates and refunds, including without limitation pharmaceutical excise taxes (such as those imposed on a Product by the United States Patient Protection and Affordable Care Act of 2010 and other comparable laws), but which shall not include any tax, duty, or other charge imposed on or measured by net income (however denominated) or any franchise taxes, branch profits taxes, or similar tax;

(d) charges for freight, customs and insurance directly related to the distribution of the Product and wholesaler and Distributor administration fees; and

(e) other future similar deductions, taken in the ordinary course of business or in accordance with GAAP and LICENSEE's standard practices;

to the extent such deductions: (i) are reasonable and customary, (ii) included in the gross invoiced sales price for the Product or otherwise directly paid, allowed, accrued, or incurred by such Party, its Affiliates or Sublicensees with respect to the sale of such Product (iii) applicable and in accordance with standard allocation procedures, (iv) have not already been deducted or excluded, (v) are incurred in the ordinary course of business in type and amount consistent with good industry practice, and (vi) except with respect to the uncollectible amounts and pharmaceutical excise taxes described in subsections (b) and (c) above, are determined in accordance with, and as recorded in revenues under, GAAP. Net Sales shall not be imputed to transfers of Product without consideration or for nominal consideration for use in any Clinical Trial, or for any bona fide charitable, compassionate use or indigent patient program purpose where Products are sold at or below Cost of Goods Sold or as a sample. For the avoidance of doubt, in the case of any transfer of any Product between or among LICENSEE and its Affiliates or Sublicensees for resale, Net Sales shall be determined based on the sale made by such Affiliate or Sublicensee to a Third Party.

Notwithstanding the foregoing, in the event a Product is sold as a component of a Combination Product in any country in the Territory in any Calendar Quarter, Net Sales shall be calculated by multiplying the Net Sales of the Combination Product (calculated in the same manner as set forth above as if the Combination Product were a Product) in such country during such Calendar Quarter by the fraction A/(A+B), where A is the average Net Sales of the Product when sold separately in such country during such Calendar Quarter and B is the average Net Sales of the Other Components included in the Combination Product (calculated in the same manner as set forth above as if the Other Components were Product) when sold separately in such country during such Calendar Quarter. In the event that no separate sales of the Product or any Other Components included in a Combination Product are made by LICENSEE, its Affiliates or Sublicensees in a country during a Calendar Quarter in which such Combination Product is sold in such country, the average Net Sales in the above described equation shall be replaced with reasonable good faith estimate of the fair market value, as mutually determined by the Parties, of the Product and each of the Other Components included in such Combination Product.

1.100 "Non-Disclosing Party" has the meaning set forth in Section 12.7.

1.101 "Orphan Drug Designation" means a grant by the FDA of a request for designation under Section 526 of the FD&C Act, as amended by section 2 of the Orphan Drug Act (sections 525-528 (21 U.S.C. 360aa-360dd)) in the United States or any analogous grant by a Regulatory Authority in any other country in the Territory.

1.102 "Orphan Drug Exclusivity" means, with respect to a Product, a grant of a period of marketing exclusivity by a Regulatory Authority for such Product in connection with an Orphan Drug Designation for such Product.

1.103 "Other Components" shall have the meaning set forth in Section 1.22.

1.104 "Out-of-Pocket Costs" means, with respect to a Party, costs and expenses paid by such Party to Third Parties (or payable to Third Parties and accrued in accordance with GAAP), other than Affiliates or employees of such Party.

1.105 "Party" or "Parties" has the meaning set forth in the Preamble.

1.106 "Patent Representative" has the meaning set forth in Section 8.3(a).

1.107 "Patent Rights" means the rights and interests in and to issued patents and pending patent applications in any country, jurisdiction or region (including inventor's certificates and utility models), including all provisionals, non-provisionals, substitutions, continuations, continuations-in-part, divisionals, renewals and all patents granted thereon, and all reissues, reexaminations, extensions, confirmations, revalidations, registrations and patents of addition thereof, including supplementary protection certificates, PCTs, pediatric exclusivity periods and any foreign equivalents to any of the foregoing.

1.108 "Person" means an individual, sole proprietorship, partnership, limited partnership, limited liability partnership, corporation, limited liability company, business trust, joint stock company, trust, incorporated association, joint venture or similar entity or organization, including a government or political subdivision or department or agency of a government.

1.109 "Pivotal Trial" means a human Clinical Trial of a Product which is intended to be sufficient for obtaining Regulatory Approval, or is according to 21 C.F.R. §312.21(c), as amended, or its equivalent, as appropriate, in foreign jurisdictions.

1.110 "Price Approval" means, in any country where a Governmental Authority authorizes reimbursement for, or approves or determines pricing for, pharmaceutical products, receipt (or, if required to make such authorization, approval or determination effective, publication) of such reimbursement authorization or pricing approval or determination (as the case may be).

1.111 "Product" means any AAV Product that delivers one or more Selected Genes (or any transgene thereof including any variant, fragment, derivative or modification thereof, in any form) and (b) with respect to which, absent the license granted to LICENSEE in Section 3.1(b), the Manufacture by LICENSEE as contemplated under this Agreement would infringe a Valid Claim of the [***] Manufacturing Patent Rights or misappropriate [***] Manufacturing Know-How.

1.112 "Receiving Party" has the meaning set forth in Section 12.1.

1.113 "Regulatory Approval" means the technical, medical and scientific licenses, registrations, authorizations and approvals (including approvals of BLAs, supplements and amendments, pre- and post- approvals, pricing and third party reimbursement approvals, and labeling approvals) of any Regulatory Authority, necessary for the commercial manufacture, distribution, marketing, promotion, offer for sale, use, import, export or sale of a pharmaceutical product in a regulatory jurisdiction. For the sake of clarity, Regulatory Approval shall not be achieved for a Product in a country until all applicable Price Approvals and other Third Party reimbursement approvals have also been obtained by LICENSEE or its designee for such Product in such country.

1.114 "Regulatory Authority" means with respect to a country in the Territory, any national (*e.g.*, the FDA), supra-national (*e.g.*, the European Commission, the Council of the European Union, or the European Medicines Agency), regional, state or local regulatory agency, department, bureau, commission, council or other Governmental Authority involved in the granting of a Regulatory Approval for pharmaceutical products in such country or countries.

1.115 "Residual Knowledge" means knowledge, techniques, experience and Know-How that are (a) included in any Confidential Information owned or Controlled by the Disclosing Party and (b) retained in the unaided memory of any employee or representative of the Receiving Party as part of a body of knowledge that is not limited to such Confidential Information, after having authorized access to such Confidential Information, provided that such employee or representative has not accessed any written or electronic records or other embodiments of any Confidential Information of the Disclosing Party for use of such Confidential Information outside of this Agreement. A person's memory will be considered to be unaided if the person (i) has not made any effort to memorize or assist the recollection of the Confidential Information for the purpose of retaining and subsequently using or disclosing it, (ii) is not relying on the external records, documents or embodiments of the Disclosing Party's Confidential Information, or notes taken on the foregoing and (iii) is not knowingly disclosing what such person knows to be the Confidential Information of the Disclosing Party. In no event, however, will Residual Knowledge include any knowledge, techniques, experience and Know-How to the extent (at any time, for such time) within the scope of any Patent Right owned or Controlled by the Disclosing Party.

1.116 "Royalty Term" means with respect to any particular Product in any particular country in the Territory, the period of time beginning on the First Commercial Sale of such Product in such country and extending until the latest of (a) the expiration of the last to expire of any Valid Claim included in any [***] Manufacturing Patent Right in such country which Valid Claim Covers the Manufacture of such Product in such country; (b) the expiration or loss of Orphan Drug Exclusivity that applies to such Product in such country; or (c) the tenth (10th) anniversary of the First Commercial Sale of such Product in such country.

1.117 "Sales Milestone Payment" has the meaning set forth in Section 5.3.

1.118 "Schedule Revision Date" means the earlier of (a) the fifth (5th) day following the HSR Clearance Date and (b) the day on or after the HSR Clearance Date on which AGTC provides to LICENSEE either (i) AGTC's supplemental or additional schedules (if any) pursuant to the proviso in the first sentence of Section 9.2, the agreed-upon updated schedule of [***] Manufacturing Patent Rights, if any, and a notice that no further supplemental, additional or updated schedules will be provided or (ii) instead of providing any such supplemental, additional or updated schedules, a notice that no further supplemental, additional or updated schedules will be provided.

1.119 "Selected Gene" means any of the six (6) gene targets selected by LICENSEE and confirmed by AGTC pursuant to Article II and listed on Schedule 1.119 (including any allelic or other functional variants thereof).

1.120 "Selection Confirmation" has the meaning set forth in Section 2.3.

1.121 "Selection Date" has the meaning set forth in Section 2.3.

1.122 "Selection Fee" has the meaning set forth in Section 5.1.

1.123 "Selection Request" has the meaning set forth in Section 2.1.

1.124 "Selection Response" has the meaning set forth in Section 2.2.

1.125 "Specification" means a list of tests, references to analytical procedures, and appropriate acceptance criteria which are numerical limits, ranges, or other criteria for the tests described, which establishes the set of criteria to which a drug substance, drug product, or materials at other stages of its Manufacture or with respect to other drug substances, drug products or materials should conform to be considered acceptable for its intended use.

1.126 "Sublicensee" means (i) with respect to LICENSEE or its Affiliate, a Third Party, other than a Distributor, to whom LICENSEE or its Affiliate has, directly or through multiple tiers, granted a right under the [***] Manufacturing Technology or the Joint Technology to make, use, develop, sell, offer for sale or import a Product in a country or otherwise exercise its rights or perform its obligations under this Agreement, and (ii) with respect to AGTC or its Affiliate, a Third Party, other than a Distributor, to whom AGTC or its Affiliate has, directly or through multiple tiers, granted a right under the LICENSEE [***] Manufacturing Improvement Technology or the Joint Technology to exercise its rights or perform its obligations under this Agreement.

1.127 "Sued Party" has the meaning set forth in Section 8.7(c)(i).

1.128 "Tax Authority" has the meaning set forth in Section 5.8(a).

1.129 "Technology" means Know-How and Patent Rights.

1.130 "Term" has the meaning set forth in Section 13.1.

1.131 "Territory" means all countries of the world.

1.132 "Third Party" means any Person other than LICENSEE, AGTC or their respective Affiliates.

1.133 "Third Party IP Rights" has the meaning set forth in Section 8.7(b)(i).

1.134 "UAB" means the Board of Trustees, directors, officers, students, agents, contractors and employees of the University of Alabama at Birmingham.

1.135 "UAB Agreement" means the Non-Exclusive License Agreement with Sublicensing Terms, dated January 19, 2006, as amended March 28, 2014 and June 29, 2015, as may be further amended from time to time, by and between AGTC and UAB.

1.136 "UAB Indemnified Party" has the meaning set forth in Section 10.3(f).

1.137 "UABRF" means The UAB Research Foundation.

1.138 "UF/JHU Agreement" means the Standard Exclusive License Agreement With Sublicensing Terms (also known as Agreement A3288), dated October 7, 2003, as amended November 2004, February 25, 2009, March 30, 2010, December 17, 2013 and July 1, 2015, as may be further amended from time to time, by and among AGTC, UFRF and JHU.

1.139 "UFRF" means University of Florida Research Foundation, Inc.

1.140 "Valid Claim" means a claim of (a) an issued and unexpired patent, which claim has not been revoked or held unenforceable, unpatentable or invalid by a decision of a court or other governmental agency of competent jurisdiction, which is not appealable or has not been appealed within the time allowed for appeal, and which has not been abandoned, disclaimed, denied or admitted to be invalid or unenforceable through reissue, re-examination or disclaimer or otherwise, or (b) a patent application for a patent that has been pending less than [***] from the earliest date on which such patent application claims priority and which claim has not been cancelled, withdrawn or abandoned or finally rejected by an administrative agency action from which no appeal can be taken. If a claim of a patent application that ceased to be a Valid Claim due to the passage of time later issues, then it will again be a Valid Claim effective as of the issuance of such patent.

## ARTICLE II
## SELECTION OF SELECTED GENES

2.1 During the Term, LICENSEE may request and select up to three (3) Available Genes of Interest and three (3) Consented Genes as Selected Genes under this Agreement, in accordance with the procedures and subject to the conditions set forth in this Article II. In the event LICENSEE wishes to make any such selection, LICENSEE shall submit to AGTC a written request, which request shall state the Gene of Interest (the "Selection Request"). For the avoidance of doubt, the Selection Request and all information contained therein shall be the Confidential Information of LICENSEE.

2.2 As soon as practicable following AGTC's receipt of any Selection Request, but in any event, within fifteen (15) days of such receipt, AGTC shall notify LICENSEE if such Gene of Interest is an Available Gene of Interest or a Consented Gene (the "Selection Response"). If such Gene of Interest is an Available Gene of Interest or a Consented Gene, AGTC may, in its sole discretion, list in the Selection Response any then-current products that meet the requirements of clause (a) of the definition of "AGTC Protectable Products" as set forth in Section 1.9, and no other information about such products. AGTC shall promptly notify LICENSEE if, at any time during the Term, AGTC ceases the Development and Commercialization of a product listed as an AGTC Protectable Product in any Selection Response and, effective as of such notice, such product shall cease to be an AGTC Protectable Product hereunder. For the avoidance of doubt, the Selection Response and all information contained therein shall be the Confidential Information of AGTC.

2.3 Subject to Section 2.4, LICENSEE shall have the right to select any Available Gene of Interest or Consented Gene as a Selected Gene hereunder upon notice to AGTC and payment of the Selection Fee pursuant to this Section 2.3. Within thirty (30) days of receipt of the Selection Response confirming that the proposed gene target is an Available Gene of Interest (or, if Section 2.4 applies, confirming that such gene target is a Consented Gene), LICENSEE shall send to AGTC a confirmation notice which either notifies AGTC that LICENSEE does wish to make such selection (the "Selection Confirmation") or notifies AGTC that LICENSEE does not wish to make such selection. Effective immediately upon payment by LICENSEE of the Selection Fee, to be made within forty-five (45) days of the Selection Confirmation (such date of effectiveness, the "Selection Date"), (i) Schedule 1.119 shall be revised to include such Selected Gene and shall be deemed to be incorporated into and amend this Agreement as of the Selection Date, superseding the previous Schedule 1.119 and (ii) such gene specified on such revised Schedule 1.119 shall be deemed to be a Selected Gene.

2.4 Notwithstanding anything to the contrary, after such time as three (3) Available Genes of Interest have been deemed Selected Genes pursuant to Section 2.3, with respect to any Selection Request for any additional Gene of Interest provided by LICENSEE under Section 2.1, AGTC may determine in its sole discretion (regardless of whether such Gene of Interest is an Available Gene of Interest) whether to consent to LICENSEE's selection of such Gene of Interest as a Selected Gene (any such consented gene target, a "Consented Gene"). AGTC shall inform LICENSEE of such determination in the Selection Response under Section 2.2.

2.5 The right of LICENSEE to submit Selection Requests for consideration by AGTC shall continue until such time as six (6) Genes of Interest selected by LICENSEE pursuant to this Article II have become Selected Genes, and shall thereafter terminate.

2.6 For clarity, any gene target that is or was the subject of a Collaboration Program under the Collaboration Agreement shall not be considered a Selected Gene under this Agreement.

ARTICLE III
LICENSES

3.1 License Grants.

(a) Non-Exclusive Research Grants to LICENSEE. Subject to the terms and conditions of this Agreement, during the Term, AGTC, on behalf of itself and its Affiliates, hereby grants to LICENSEE a non-exclusive, royalty-free, fully paid-up license in the Territory, with no right to grant sublicenses, under the [***] Manufacturing Technology and the Materials transferred hereunder, solely for internal, non-commercial research purposes.

(b) Commercial Grant to LICENSEE. Subject to the terms and conditions of this Agreement, AGTC, on behalf of itself and its Affiliates, hereby grants to LICENSEE an exclusive license (exclusive even as to AGTC and its Affiliates), with the right to grant sublicenses through multiple tiers pursuant to Section 3.2, under the [***] Manufacturing Technology and the Materials transferred hereunder, to Manufacture and have Manufactured Products, and to use, have used, Develop, have Developed, Commercialize, have Commercialized, import, have imported, export and have exported such Products in the Field in the Territory.

(c) Grant to AGTC of LICENSEE [***] Manufacturing Improvement Technology.

(i) Subject to the terms and conditions of this Agreement and effective as of the Effective Date, LICENSEE, on behalf of itself and its Affiliates, hereby grants to AGTC a non-exclusive, worldwide, royalty-bearing license, with the right to grant sublicenses through multiple tiers, under the LICENSEE [***] Manufacturing Improvement Technology to Manufacture and have Manufactured Gene Therapy Products other than Products, and to use, have used, Develop, have Developed, Commercialize, have Commercialized, import, have imported, export and have exported such Gene Therapy Products.

(ii) If any Gene Therapy Product sold by AGTC, its Affiliates or Sublicensees or the Manufacture thereof by AGTC, its Affiliates or Sublicenses is Covered by a Valid Claim of a LICENSEE [***] Manufacturing Improvement Patent Right licensed to AGTC under this Section 3.1(c) in the country in which such Gene Therapy Product is made, used or sold, then on a country-by-country basis AGTC will pay to LICENSEE a royalty at a rate to be agreed upon by the Parties of up to [***] of net sales (as determined in accordance with Section 3.1(c)(iv) and calculated in accordance with Section 1.99, which definition of Net Sales shall apply *mutatis mutandis* to such calculation) of such Gene Therapy Product on a country-by-country and Gene Therapy Product-by-Gene Therapy Product basis, until the latest of (a) the expiration of the last to expire of any Valid Claim included in any Patent Right licensed to AGTC under this Section 3.1(c) in such country which Valid Claim Covers the Manufacture of such Gene Therapy Product in such country, (b) the expiration of all periods of Orphan Drug Exclusivity that apply to such Gene Therapy Product in such country or (c) the tenth (10th) anniversary of the First Commercial Sale of such Gene Therapy Product in such country.

(iii) Such royalties shall be paid in accordance with the provisions of Section 5.5, Section 5.8, Section 5.9 and Section 5.10, which shall apply *mutatis mutandis* to payments made by AGTC pursuant to this Section 3.1(c), provided, however, that if AGTC licenses or has prior to the Effective Date licensed, intellectual property rights from one or more Third Parties, in either case, which intellectual property rights are necessary or useful to, and are actually used at any time to, exercise the license under Section 3.1(c)(i), whether directly or through any AGTC Affiliate or Sublicensee, then any royalties otherwise payable to LICENSEE under Section 3.1(c)(ii) shall be reduced by [***] of the royalties paid to Third Parties pursuant to any such Third Party licenses arising out of and directly attributable and proportionately allocated to the exercise of the license under Section 3.1(c)(i), provided that in no event shall any royalty payable to LICENSEE under this Section 3.1(c) be reduced to less than [***] (unless the royalty rate determined under Section 3.1(c)(ii) or Section 3.1(c)(iv) is less than [***], in which case no royalty reduction will apply); provided, however, that any amounts paid under such Third Party license that are not used to reduce a payment due hereunder as a result of the foregoing limitations may be carried over to reduce subsequent payments due under this Section 3.1(c).

(iv) If the Parties are unable to agree upon the applicable royalty rate within thirty (30) days of the commencement of discussions regarding such royalty rate, then the Parties shall select a mutually agreed external neutral expert with significant and relevant experience to decide upon a commercially reasonable royalty rate of up to [***], which external neutral expert shall not have previously served as an employee of either Party or, within the two (2) years prior to the external neutral expert's engagement by the Parties pursuant to this Section 3.1(c), as a consultant or third party expert for either Party. The Parties shall cooperate with such external neutral expert to enable such external neutral expert to reach a decision as quickly as possible. The decision of the external neutral expert shall be final, non-appealable and binding on the Parties. LICENSEE and AGTC shall share equally the costs and fees of such external neutral expert regardless of the decision by the external neutral expert.

(d) Joint Technology. Subject to the terms and conditions of this Agreement, each Party, on behalf of itself and its Affiliates, hereby grants to the other Party a non-exclusive, worldwide, royalty-free, fully paid-up, irrevocable license, with the right to grant sublicenses through multiple tiers, under its interest in the Joint Technology, to use, have used, Develop, have Developed, Manufacture, have Manufactured, Commercialize, have Commercialized, import, have imported, export and have exported products or processes, provided, however, that, until the expiration of the last-to-expire Royalty Term (for purposes of this Section 3.1(d), as defined in the Collaboration Agreement) for the Initial Licensed Products under the Collaboration Agreement (treating, for this purpose, any Cost Share Product as an Initial Licensed Product for which AGTC has exercised the Milestone/Royalty Option), or the earlier termination of the Collaboration Agreement with respect to both Initial Licensed Programs, LICENSEE may not (a) use any Joint [***] Manufacturing Improvement Technology, or (b) license, assign or transfer its interest in any Joint [***] Manufacturing Improvement Technology, to a Third Party, in each case ((a) and (b)), for use in a program involving an AAV Product [***], as demonstrated to LICENSEE through written records provided on an annual basis or more frequently as requested by LICENSEE if LICENSEE has a bona fide intent to license, assign or transfer its interest in any such Joint Technology, and AGTC will thereafter inform LICENSEE if it discontinues development activities with respect to a program previously identified by AGTC pursuant to this Section 3.1(d).

3.2 Sublicenses. Subject to the restrictions set forth on Schedule 3.2, LICENSEE shall have the right to grant sublicenses through multiple tiers to one or more of its Affiliates and to one or more Sublicensees of any and all rights granted to LICENSEE under this Agreement by AGTC, provided that in no event may LICENSEE grant a sublicense, and LICENSEE shall use reasonable efforts to ensure that none of its Affiliates or their respective Sublicensees grant a sublicense, of any of the rights licensed under Section 3.1(b) with respect to a Product to any Person that, as of the date of the sublicense grant, has publicly disclosed, or otherwise disclosed to LICENSEE, that it is (i) Developing or Commercializing a product in a program that constitutes a Competing Program as of the date of the sublicense grant if at such time LICENSEE is Developing such Product or (ii) Commercializing a product in a program that constitutes a Competing Program as of the date of the sublicense grant if at such time LICENSEE is Commercializing such Product, in each case of (i) or (ii) without AGTC's prior written consent, which AGTC may give in its sole discretion. Each such sublicense shall be subject and subordinate to, and consistent with, the terms and conditions of this Agreement. The engagement of any Sublicensee in compliance with this Section 3.2 shall not relieve LICENSEE of its obligations under this Agreement. LICENSEE shall remain responsible for actions or omissions of its Sublicensees and LICENSEE's breaches under this Agreement that are caused by its Sublicensee's breach of any sublicense agreement (or delay caused by such breach). LICENSEE shall provide a redacted copy of each sublicense to AGTC promptly following execution of such sublicense.

3.3 Retained Rights. AGTC shall at all times retain the unrestricted right, under all Technology and other intellectual property Controlled by AGTC, subject to any other agreements with LICENSEE or with a Third Party (i) to research and develop the [***] Manufacturing Technology itself or with Third Parties (which right, for purposes of clarity, shall not include the right to research or Develop Products in the Field), (ii) to use the [***] Manufacturing Technology for any purpose outside the Field and (iii) to use the [***] Manufacturing Technology to Develop, Manufacture or Commercialize any products in the Field other than Products.

3.4 No Implied Rights. Except as expressly provided in this Agreement, neither Party shall be deemed to have granted the other Party any license or other right with respect to any intellectual property of such Party.

3.5 Existing License Agreements.

(a) The rights granted to LICENSEE, its Affiliates or Sublicensees under this Agreement are subject and subordinate to the terms and conditions of the Existing License Agreements, including the coordination of prosecution or enforcement of Patent Rights or other intellectual property rights under the applicable agreement.

(b) LICENSEE shall be entitled to grant a sublicense under its sublicense rights in the [***] Agreements in conjunction with a license to technology owned or controlled by LICENSEE that (i) is included in or useful for the making of [***] Products and (ii) is intended to be included in or used in the manufacture of [***] Products by the Sublicensee. LICENSEE shall only be entitled to sublicense its rights under each [***] Agreement on the terms set forth in in Section 2.3 of such [***] Agreement.

(c) It is understood that the United States Government (through any of its agencies or otherwise) has funded research, [***] during the course of or under which certain of the inventions of the [***] Manufacturing Patent Rights licensed to AGTC under the UF/JHU Agreement were conceived or made. The United States Government is entitled, as a right, under the provisions of 35 U.S.C. §202-212 and applicable regulations of Title 37 of the Code of Federal Regulations, to a non-exclusive, nontransferable, irrevocable, paid-up license to practice or have practiced the inventions of such [***] Manufacturing Patent Rights for governmental purposes. Any license under the Patent Rights in the UF/JHU Agreement granted to LICENSEE in this Agreement shall be subject to such right.

(d) LICENSEE shall include the following provisions in any sublicense to a Sublicensee, revised as appropriate to apply to such Sublicensee as it applies to LICENSEE, to the extent such [***] Manufacturing Technology is sublicensed and to the extent such provision applies to AGTC's licensors of such [***] Manufacturing Technology: Sections 3.5, 4.4, 5.6, 5.7, 8.10, 9.3, 9.4, 9.5, 9.6, 9.7, 10.3, 10.5, 12.6(c), 14.1, 14.8 and 14.14. The Parties acknowledge and agree that in the event that any Technology is included in the licenses granted to LICENSEE under this Agreement pursuant to Section 8.7(b)(i), additional obligations and restrictions may need to be included in this Agreement prior to such Technology being included in such licenses. Without limiting the foregoing, upon LICENSEE's election to take a sublicense under Section 8.7(b)(i) to any Technology, the Parties shall update Schedule 3.2 to include any restrictions on LICENSEE's right to sublicense such Technology.

3.6 Other Programs. AGTC understands and acknowledges that LICENSEE may have present or future initiatives or opportunities, including initiatives or opportunities with Third Parties, involving similar products, programs, technologies or processes that may compete with a product, program, technology or process covered by this Agreement. AGTC acknowledges and agrees that nothing in this Agreement will be construed as a representation, warranty, covenant or inference that LICENSEE will not itself Develop, Manufacture or Commercialize or enter into business relationships with one or more Third Parties to Develop, Manufacture or Commercialize products, programs, technologies or processes that are similar to or that may compete with any product, program, technology or process covered by this Agreement (including products targeting a Gene of Interest or a Selected Gene), provided that LICENSEE will not use AGTC's Confidential Information in breach of this Agreement.

## ARTICLE IV
## TRANSFER AND ASSISTANCE

4.1 Initial Technology Transfer. Within the time periods set forth in a technology transfer plan to be agreed by the Parties within sixty (60) days after the Effective Date, AGTC shall transfer to LICENSEE at [***]'s sole expense, to the extent not already transferred to LICENSEE under the Collaboration Agreement, a true and complete copy as reasonably practicable of (a) data embodying any [***] Manufacturing Know-How, (b) other tangible embodiments of [***] Manufacturing Know-How and (c) documentation necessary or useful to evaluate the Materials including (i) data safety sheets, (ii) history (provenance) of cell lines and viral seed stocks, (iii) development reports (e.g., process development, specifications, stability data for cell and viral banks, suspension and serum-free media adaptation of cell lines, media development, viral

clearance studies, and viral inactivation studies, (iv) [***]-assisted manufacturing process development reports, risk-assessments, CQAs, CPPs, control strategies, process flow diagrams, process instructions/SOPs, (v) all analytical characterization reports of each Material, (vi) analytical methods to characterize materials including assay development reports, specifications, and method description/SOP, (vii) reference information for any reference standards or material-specific reagents for assays for Materials, (viii) fully annotated maps for vectors and plasmids referenced in the Materials, and (ix) any environmental, product or process related risk assessments performed in relation to the Materials, in each case ((a) through (c)), that is necessary or useful to enable LICENSEE to practice its licenses and rights under this Agreement, in such format as LICENSEE may reasonably request (including by download of digital files to a secure website or e-room designated and controlled by LICENSEE, to which AGTC has equivalent access).

4.2 Ongoing Technology Transfers. The Parties shall conduct a transfer [***], or more frequently at such time as new material [***] Manufacturing Technology or LICENSEE [***] Manufacturing Improvement Technology, as applicable, comes into a Party's Control, in accordance with a technology transfer plan, to transfer to the other Party (i) if the transferee Party is LICENSEE, any and all tangible Know-How within the [***] Manufacturing Technology, and (ii) if the transferee Party is AGTC, any and all tangible Know-How within the LICENSEE [***] Manufacturing Improvement Technology, in each case ((i) and (ii)), to the extent not already transferred to the transferee Party under this Agreement or the Collaboration Agreement, to the extent necessary or useful to enable LICENSEE to practice the licenses and rights under this Agreement and in such format as the transferee Party may reasonably request (including, if the transferee Party is LICENSEE, by download of digital files to a secure website or e-room designated and controlled by LICENSEE, to which AGTC has equivalent access). Further, AGTC shall make appropriate personnel available to LICENSEE at reasonable times and places, including by telephone during normal business hours, and upon reasonable prior notice for the purpose of assisting LICENSEE to understand and use the [***] Manufacturing Technology for the Development, Manufacture, Commercialization and use of Products in accordance with this Agreement. Any activities under this Section 4.2 shall be conducted at AGTC's sole expense.

4.3 Transfer of Materials. AGTC shall provide to LICENSEE the Materials to the extent not already transferred under the Collaboration Agreement. Prior to the commencement of Manufacturing of any Product by LICENSEE, AGTC shall transfer to LICENSEE, at LICENSEE's request, any Materials specific to such Product and reasonable quantities of Materials that are not specific to such Product that are necessary or useful to enable LICENSEE to practice its license and rights under this Agreement. LICENSEE shall, subject to the terms and retained rights included in the Existing License Agreements as set forth in Section 4.4, have sole ownership of the Materials delivered to LICENSEE under this Section 4.3, or, if AGTC cannot transfer ownership of such Materials to LICENSEE, AGTC shall, and hereby does, transfer to LICENSEE all of AGTC's right, title and interest in and to such Materials. All Materials shall be used only in the fulfillment of obligations or exercise of rights under this Agreement and solely under the control of LICENSEE, shall not be used or delivered by the LICENSEE to or for the benefit of any Third Party (other than a permitted subcontractor or Sublicensee) without the prior written consent of AGTC, and, except with respect to any Materials provided by AGTC to the LICENSEE hereunder for use in a Clinical Trial, shall not be used in research or testing involving

human subjects, unless expressly agreed. All Materials supplied under this Section 4.3 are supplied "as is", with no warranties of fitness for a particular purpose and must be used with prudence and appropriate caution in any experimental work, since not all of their characteristics may be known. The transfer of Materials under this Section 4.3 shall be conducted at AGTC's expense.

4.4 Restrictions on Use and Transfer of Materials. Schedule 4.4 sets forth the Materials to which each of the following restrictions applies. Upon any transfer under Section 4.3 of any Third Party Materials not listed on Schedule 4.4, AGTC will notify LICENSEE of any restrictions applicable to such Materials.

(a) In General. LICENSEE shall, and shall direct its Affiliates and Sublicensees to, use the Materials solely in connection with exercising its rights and performing its obligations under this Agreement. Except as expressly agreed by the Parties, LICENSEE shall not (1) reverse engineer the Materials, to the extent prohibited by an AGTC Third Party Agreement related to such Materials, (2) transfer the Materials to a Third Party, except to a permitted subcontractor or Sublicensee, (3) transfer the Materials outside of LICENSEE's or its Affiliate's or Sublicensee's facilities or (4) file any patent application or claim inventorship for Materials that are proprietary to AGTC and are provided to LICENSEE under this Article IV. LICENSEE shall at all times, and direct its Affiliates and Sublicensees to at all times, comply with all applicable Laws regarding the use, storage and handling of the Material.

(b) [***] Biological Materials.

(i) LICENSEE acknowledges that all rights, title and interest in and to all materials scheduled in the [***] Agreements, together with all progeny, mutants, replicates and derivatives (modified or unmodified) thereof (collectively, the "[***] Biological Material(s)") shall be owned solely and exclusively by [***]. For clarity, the [***] Biological Materials do not include (a) any virus produced by AGTC, LICENSEE, or their respective Affiliates or sublicensees through the use of the [***] Biological Materials, provided that such virus does not contain any [***] Biological Materials or any functional portion or functional fragment thereof (a "[***] Virus") or (b) any product produced by a [***] Virus (a "[***] Product").

(ii) LICENSEE acknowledges that AGTC is required to inform [***] of any [***] Biological Material created by LICENSEE that is different from, and a modification to, the [***] Biological Material listed in part (a) of Schedule 4.4. LICENSEE shall not use the [***] Biological Material other than in accordance with the rights expressly granted by the applicable [***] Agreement. LICENSEE shall not sell or otherwise transfer any [***] Biological Material to any Affiliate or Third Party, except in connection with a sublicense granted in accordance with the provisions of this Agreement. The [***] Biological Material shall not be used in humans. All of the [***] Biological Material is experimental in nature and shall be used with prudence and appropriate caution since not all of their characteristics are known. LICENSEE acknowledges that, as between AGTC and [***], all right, title and interest in and to all [***] Viruses, [***] Products, and any intellectual property applying thereto or to the production thereof, shall be owned solely and exclusively by AGTC. For the avoidance of doubt, nothing herein prohibits or is intended to prohibit the use of the [***] Products in humans.

(iii) Except as expressly provided herein, nothing in this Agreement will be construed to confer any ownership interest, license or other rights upon LICENSEE by implication, estoppel or otherwise as to any technology, intellectual property rights, products or biological materials of [***], or any other entity, regardless of whether such technology, intellectual property rights, products or biological materials are dominant, subordinate or otherwise related to any [***] Biological Material or the other Materials listed in part (a) of Schedule 4.4.

(iv) LICENSEE shall not enter into any agreement under which LICENSEE grants to or otherwise creates in itself or any Affiliate or Third Party a security interest in any [***] Agreement or its rights under any [***] Agreement and any such security interest shall be null and void and of no legal effect. This limitation shall apply to any [***] Biological Material or the other Materials listed in part (a) of Schedule 4.4.

(c) [***]. The use of any cell line listed on part (b) of Schedule 4.4 licensed under the Agreement, [***], and as may be further amended shall be subject to the following terms: (i) LICENSEE shall only have the right to distribute and license [***] and not [***] and (ii) shall be subject to the terms and conditions included in Schedule 4.4(c), which terms and conditions allow for commercial use, despite references to "research purposes only".

(d) UF/JHU Materials. LICENSEE acknowledges that any Materials listed on part (c) of Schedule 4.4 under the Materials Use Agreement, dated March 13, 2014 by and between the University of Florida Board of Trustees, JHU and AGTC, shall at all times remain the property of the University of Florida Board of Trustees and JHU. With respect to such Materials, LICENSEE may transfer such Materials to its Affiliates or Third Parties to the extent necessary for said Affiliates or Third Parties to manufacture for LICENSEE (i) AAV or (ii) the raw materials and components used in connection with the preparation of AAV. LICENSEE shall provide to AGTC written notification of the identity of any such Third Party that receives such Materials from LICENSEE along with a certification that such transfer is in compliance with this Section 4.4(d) within thirty (30) days of such transfer.

## ARTICLE V
## CONSIDERATION

5.1 Selection Fee. For each Selected Gene listed on Schedule 1.119, LICENSEE shall pay to AGTC a non-refundable fee of [***] (the "Selection Fee") within forty-five (45) days after the date of the Selection Confirmation.

5.2 Event Milestone Payments. In partial consideration for AGTC's development of the [***] Manufacturing Technology and the grant of rights hereunder, LICENSEE shall pay AGTC the amounts set forth below within forty-five (45) days of the first occurrence of each event described below for the first Product with respect to each Selected Gene to achieve such event (each, an "Event Milestone Payment").

[***]

Each of the Event Milestone Payments set forth above shall be payable one time only for each Selected Gene (regardless of the number of Products for such Selected Gene with respect to which, or the number of times with respect to any such Product, the specified event milestone occurs). No Event Milestone Payments shall be payable for any subsequent Product for the same Selected Gene regardless of the number of such Products developed. Notwithstanding anything to the contrary, if more than one Selected Gene is included in a single Product, each Event Milestone Payment shall be payable only once upon the first achievement by such Product of the applicable event milestone, provided that, if LICENSEE later uses any one of such Selected Genes in a separate Product, each Event Milestone Payment shall be payable again upon the first achievement by such separate Product of the applicable event milestone. For clarification, if one Product replaces another Product in development for a given Selected Gene, such replacement Product, as applicable, shall only be subject to Event Milestone Payments that have not previously been triggered by one or more prior Products for such Selected Gene, as applicable.

If for any reason, with respect to a Selected Gene, (i) milestone 1 does not occur prior to the occurrence of any of the subsequent milestones (ii) milestone 2 does not occur before the occurrence of any of the subsequent milestones, (iii) milestone 3 does not occur before milestone 6, (iv) milestone 4 does not occur before milestone 7 or (v) milestone 5 does not occur before milestone 8, then upon achievement of the later event milestone, Event Milestone Payments shall be payable both for the event milestone achieved and any earlier event milestone that was bypassed.

5.3 Sales Milestones Payments. In addition to the Event Milestone Payments described in Section 5.2, in consideration of the rights granted to LICENSEE hereunder, and subject to the terms and conditions of this Agreement, LICENSEE shall pay AGTC the following one-time payments (each, a "Sales Milestone Payment") when aggregate Net Sales of all Products for a given Selected Gene, in a Calendar Year in the Territory first reach the respective thresholds indicated below:

[***]

LICENSEE shall make any Sales Milestone Payment payable with respect to a Calendar Year within sixty (60) days after the end of the applicable Calendar Quarter in which such cumulative Net Sales for such Calendar Year were achieved, and such payment shall be accompanied by a report identifying the applicable Products, the relevant countries, Net Sales of each Product for each such country, and the amount payable to AGTC under this Section 5.3. In the event that more than one of the previously unmet sales milestones are achieved in a Calendar Year with respect to a Product for a given Selected Gene, then all of the Sales Milestone Payments corresponding to the sales milestones met in such year shall be owed to AGTC.

5.4 Royalties.

(a) In consideration for the license granted to LICENSEE under Section 3.1(b), LICENSEE, on a Product-by-Product and country-by-country basis shall, during the Royalty Term for such Product, pay to AGTC a royalty on Net Sales of (a) [***] plus (b) all royalties, if any, payable to the Existing Licensors pursuant to the Existing License Agreements as a result of Net Sales hereunder; provided that, in any case, the royalties due hereunder shall not exceed [***] of Net Sales.

(b) Following expiration of the Royalty Term for any Product in a country, no further royalties shall be payable in respect of sales of such Product in such country and, thereafter the license granted to LICENSEE under Section 3.1(b) with respect to such Product in such country shall be a fully paid-up, perpetual, exclusive, irrevocable, royalty-free license.

(c) Any obligation to pay royalties under this Agreement shall be imposed only once with respect to any sale of any Product.

(d) Subject to the provisions of Section 5.4(a), AGTC shall be solely responsible for all obligations (including any royalty or other obligations that relate to the [***] Manufacturing Technology) under the Existing License Agreements and any other agreements with Third Parties that are in effect as of the Effective Date. Solely to the extent that LICENSEE elects to take a sublicense under Section 8.7(b)(i) under any license to Third Party IP Rights that AGTC or any of its Affiliates enters into during the Term, LICENSEE shall be responsible for any payment obligations under the applicable AGTC Third Party Agreements arising out of the Development, Manufacture, Commercialization or use of any Product, provided that any upfront payments under such AGTC Third Party Agreements shall be allocated equitably by AGTC in good faith and proportionately among the applicable Products and other relevant programs of AGTC and its Affiliates. AGTC shall be solely responsible for all other obligations under any such AGTC Third Party Agreements. Notwithstanding anything to the contrary, in the event that LICENSEE obtains a direct license from any licensor under an AGTC Third Party Agreement upon termination of such AGTC Third Party Agreement pursuant to Section 13.6, then, if AGTC had been paying all amounts due under such AGTC Third Party Agreement prior to such termination, any payments otherwise payable to AGTC under Section 5.4(a) with respect to a Product shall be reduced by [***] of the payments paid to Third Parties pursuant to any such Third Party licenses arising out of and directly attributable to the Development, Manufacture, Commercialization or use of such Product without any limitation described in this Section 5.4.

(e) On a country-by-country and Product-by-Product basis, any royalty otherwise payable to AGTC under this Agreement with respect to Net Sales of such Product in such country shall be reduced by [***] at any time when (a) there is no Valid Claim included in the [***] Manufacturing Patent Rights in such country that Covers the Manufacture of such Product and (b) there is no Orphan Drug Exclusivity, or Orphan Drug Exclusivity has terminated, with respect to such Product in such country.

(f) In the event that the Royalty Term for any Product extends beyond the [***] anniversary of the First Commercial Sale of such Product solely because the Manufacture of such Product is Covered by a Valid Claim of an [***] Manufacturing Patent Right Controlled by AGTC under an AGTC Third Party Agreement that AGTC enters into during the Term, then, for the remainder of the Royalty Term, any royalty payments otherwise payable to AGTC under this Agreement with respect to such Product shall be reduced to an amount equal to [***]. Notwithstanding anything to the contrary, this Section 5.4(f) shall not apply in the event that the Manufacture of such Product is Covered by a Valid Claim of an [***] Manufacturing Patent Right Controlled by AGTC under an AGTC Third Party Agreement that AGTC enters into during the Term, but for which AGTC provided all or substantially all of the funding that contributed to the invention Covered by such Valid Claim.

5.5 Reports; Payments. Within ten (10) days of the end of each Calendar Quarter, AGTC shall deliver to LICENSEE a report setting forth the royalty rates, if any, that are payable to the Existing Licensors pursuant to the Existing License Agreements for each item of [***] Manufacturing Technology. Within sixty (60) days of the end of each Calendar Quarter, at any time during the Term in which LICENSEE is making royalty payments to AGTC for any Products under Section 5.4, LICENSEE shall deliver to AGTC a report setting forth for the most recently completed Calendar Quarter, the following information, on a Product-by-Product, country-by-country and Territory-wide basis: (a) Net Sales of each such Product, (b) the basis for any adjustments to the royalty payable for the sale of any such Product and (c) the royalty due hereunder for the sale of each such Product. No such reports shall be due for any such Product before the First Commercial Sale of such Product. The total royalty due for the sale of all such Products during such Calendar Quarter shall be remitted at the time such report is made.

5.6 Books and Records.

(a) Each Party shall maintain, consistent with its then-current internal policies and practices, and cause its Affiliates, Sublicensees, employees and subcontractors to maintain, consistent with its internal policies and applicable Law, [***], records and laboratory notebooks, inventory, purchase and invoice records and Manufacturing records with respect to the Products in sufficient detail and in a good scientific manner appropriate for (i) inclusion in filings with Regulatory Authorities, and (ii) obtaining and maintaining intellectual property rights and protections, including Patent Rights. Such records and laboratory notebooks shall be complete and accurate in all material respects and shall fully and properly reflect all work done, data and developments made, and results achieved. Each Party shall allow, and cause its Affiliates, Sublicensees, employees and subcontractors to allow, the other Party, to the extent necessary for such regulatory or intellectual property protection purposes, inspect or copy such records, subject to redaction by such Party.

(b) Each Party shall keep and shall cause its Affiliates and Sublicensees to keep complete and accurate books and accounts of record in connection with the sale of Products, including without limitation, sales analysis, general ledgers, financial statements, and tax returns, in each case, in accordance with GAAP and such Party's then-current accounting procedures and in sufficient detail to permit accurate determination of all figures necessary for verification of amounts to be paid under this Agreement. Each Party shall, and shall cause its Affiliates and Sublicensees to, maintain such records for a period of at least six (6) years after the end of the Calendar Quarter in which they were generated.

5.7 Audits.

(a) Upon reasonable advance written notice by a Party (the "Auditing Party") and not more than once in each Calendar Year (except for cause), the other Party (the "Audited Party") and its Affiliates shall permit, and shall use reasonable efforts to cause their Sublicensees to permit the Auditing Party or Existing Licensors (or an attorney or CPA of such licensor), or an independent certified public accounting firm of internationally recognized standing, selected by the Auditing Party and reasonably acceptable to the Audited Party, to have access during normal business hours to such of the records of the Audited Party and its Affiliates and, if applicable, their

Sublicensees as may be reasonably necessary to verify the accuracy of the applicable royalty or milestone payments hereunder. No year may be audited more than once, except for cause. The accounting firm will enter a confidentiality agreement reasonably acceptable to the Audited Party governing the use and disclosure of the Audited Party's information disclosed to such firm, and such firm shall disclose to the Auditing Party only whether the information provided by the Audited Party to the Auditing Party as described in clauses (a) through (b) above was accurate, and the specific details concerning any discrepancies, which information shall be Confidential Information of the Audited Party.

(b) Unless disputed by either Party in good faith, if such accounting firm concludes that any payments paid by a Party to the other Party during the audited period were more or less than the amount actually due, the underpaying Party shall pay any additional amounts due, or the overpaid Party will refund any amounts overpaid, as applicable, in each case plus interest as set forth in Section 5.10, within forty-five (45) days after the date the written report of the accounting firm so concluding is delivered to the Parties. The written report will be binding on the Parties absent clear error. The fees charged by such accounting firm shall be paid by the Auditing Party; provided, however, that if the audit results in a payment adjustment of more than five percent (5%), then the Audited Party shall pay the reasonable fees and expenses charged by such accounting firm. The Auditing Party shall treat all financial information disclosed by its accounting firm pursuant to this Section 5.7(b) as Confidential Information of the Audited Party for purposes of Article XII of this Agreement.

(c) In the event of a good faith dispute by either Party regarding the result of an audit made pursuant to this Section 5.7(c), the Parties shall agree in good faith on an alternative independent certified public accounting firm of internationally recognized standing to perform a second audit. If such audit is requested by the Audited Party because the Audited Party was found by the initial audit to have underpaid and the second audit confirms that the Audited Party underpaid, then the Audited Party shall bear all costs associated with the second audit. If such audit is requested by the Auditing Party because the Audited Party was found by the initial audit to have overpaid and the second audit confirms that the Audited Party overpaid, then the Auditing Party shall bear all costs associated with the second audit. Notwithstanding the above, in the event that the second audit confirms the findings of the first audit, the requesting Party shall pay. No over or under payment indicated by the initial audit shall be payable in the event of a dispute until the second audit is complete and such second audit shall be binding on the Parties, with any under or over payment determined thereby, plus interest as set forth in Section 5.10, being payable within thirty (30) days after the date the written report of the accounting firm so concluding is delivered to both Parties.

5.8 Taxes.

(a) Withholdings. AGTC shall provide such information and documentation to LICENSEE as are reasonably requested by LICENSEE that are necessary for LICENSEE to determine if any withholding taxes apply to any payments to be made by LICENSEE to AGTC. LICENSEE shall only make such withholding payments to the extent required by applicable Law and shall subtract such required withholding payments that are actually paid by LICENSEE to the appropriate Governmental Authority responsible for the collection of such withholding tax (such a

Governmental Authority, a "Tax Authority") from the payments due to AGTC. For avoidance of doubt, AGTC shall not be responsible for any interest, penalties or additions to tax attributable to LICENSEE'S failure to timely make any such required withholding payments. LICENSEE shall promptly submit to AGTC appropriate proof of payment by LICENSEE to the appropriate Tax Authority of the required withholding taxes. At the request of AGTC, LICENSEE shall give AGTC such reasonable assistance, which shall include the provision of appropriate certificates of such deductions and withholding payments made, together with other supporting documentation as may be required by the relevant Tax Authority, to enable AGTC to claim exemption from such withholding tax or to obtain a repayment thereof or a reduction thereof, and shall provide such additional documentation from time to time as is reasonably requested by AGTC in connection with any of the foregoing. LICENSEE shall use commercially reasonable efforts to minimize any such withholdings.

(b) Additional Taxes. The amount of any payment to be made by LICENSEE to AGTC pursuant to this Agreement shall be increased for any sales, value added or similar taxes (any such taxes, "Additional Taxes") required to be collected by AGTC from LICENSEE. LICENSEE shall provide such information and documentation to AGTC as are reasonably requested by AGTC for AGTC to determine the amount of any Additional Taxes that apply to any payments to be made by LICENSEE to AGTC, and to satisfy any applicable reporting obligations related to such Additional Taxes.

(c) The Parties agree that the provisions of this Section 5.8 shall also apply to payments made by AGTC to LICENSEE, if any, under this Agreement, in which case this Section 5.8 shall be read by replacing all references to "AGTC" with "LICENSEE" and all references to "LICENSEE" WITH "AGTC."

5.9 Payment Method and Currency Conversion. All payments to be made by a Party to the other Party hereunder shall be in immediately available funds via either a bank wire transfer, an ACH (automated clearing house) mechanism, or any other means of electronic funds transfer, at the payee Party's election, to a bank account to be designated by the payee Party in a notice at least ten (10) days before the payment is due. All amounts payable and calculations under this Agreement shall be in United States Dollars. As applicable, Net Sales and any royalty deductions shall be translated into United States Dollars at the exchange rate used by LICENSEE for public financial accounting purposes in accordance with GAAP. If, due to restrictions or prohibitions imposed by national or international authority, payments cannot be made as provided in this Article V, the Parties shall consult with a view to finding a prompt and acceptable solution, and LICENSEE will deal with such monies as AGTC may lawfully direct.

5.10 Late Payments. If a Party does not receive payment of any sum due to it on or before the due date therefor set forth in this Agreement, simple interest shall thereafter accrue on the sum due to the Party from the due date until the date of payment at a per-annum rate of [***] over the then-current prime rate reported in *The Wall Street Journal* or the maximum rate allowable by applicable Laws, whichever is lower.

## ARTICLE VI
## DEVELOPMENT AND COMMERCIALIZATION DILIGENCE

6.1 Diligence Obligations. LICENSEE shall be responsible for all Development and Commercialization activities with respect to the Products, and for all costs and expenses associated therewith, and shall use Commercially Reasonable Efforts to Develop and Commercialize a Product with respect to each Selected Gene in the Territory. LICENSEE shall, and shall use commercially reasonable efforts to cause its Affiliates and Sublicensees to, comply with all applicable Laws, including without limitation, obtaining all necessary licenses, permits and approvals in each region in the Territory where Commercialization activities occur.

6.2 Diligence Reports. By December 31st of each Calendar Year after selection of a Selected Gene in accordance with this Agreement, LICENSEE shall deliver to AGTC an up-to-date report containing summaries of the following items with respect to such Product, as applicable: (a) a status update with respect to research, pre-clinical, clinical and CMC matters for such Product and (b) the plan for Development and Commercialization activities for such Product across all relevant functions for the following year.

## ARTICLE VII
## REGULATORY MATTERS.

7.1 Ownership of Regulatory Documentation. LICENSEE will own all INDs, Orphan Drug Designations, BLAs and related documentation submitted to any Regulatory Authority and all Regulatory Approvals with respect to the Products.

7.2 Responsibilities. LICENSEE will be solely responsible, in LICENSEE's sole discretion, for all regulatory matters relating to the Products, including (i) overseeing, monitoring and coordinating all regulatory actions, communications and filings with, and submissions to, Regulatory Authorities with respect to the Products; (ii) interfacing, corresponding and meeting with Regulatory Authorities with respect to the Products; (iii) submitting and maintaining all regulatory filings with respect to the Products; and (iv) maintaining and submitting all records required to be maintained or required to be submitted to any Regulatory Authority with respect to the Products, provided that, if such matter would set a regulatory precedent for Specifications for the Manufacture of AAV Products during the period of time that Regulatory Approval for at least [***] AAV Products has not been obtained by either Party or their respective Affiliates or sublicensees or [***] years from the first Regulatory Approval achieved for such AAV Products, if earlier, then such matter may only be decided by mutual agreement of the Parties.

## ARTICLE VIII
## INTELLECTUAL PROPERTY

8.1 Ownership of Intellectual Property.

(a) Ownership of Inventions. Each Party shall own all right, title and interest in and to: (i) any and all inventions, developments or discoveries made solely by its or its Affiliates' employees, agents or independent contractors in connection with their activities under this Agreement; (ii) any and all Patent Rights claiming any invention, development or discovery

described in clause (i) of this Section 8.1; and (iii) any and all Know-How embodied by or in any invention, development or discovery described in clause (i) of this Section 8.1. Inventorship shall be determined in accordance with United States patent laws.

(b) Ownership of Joint Know-How and Joint Patent Rights. The Parties shall jointly own any Joint Technology. Subject to the license grant under Section 3.1(b) and the Parties' other rights and obligations under this Agreement, each Party shall be free to exploit Joint Patent Rights and Joint Know-How pursuant to the license grant set forth in Section 3.1(d), including granting a license under such Joint Technology without accounting to the other Party in accordance with Section 3.1(d).

8.2 Personnel Obligations. Each employee, agent or independent contractor (including all subcontractors) of a Party or its respective Affiliates performing work under this Agreement shall, prior to commencing such work, be bound by invention assignment obligations, including: (i) promptly reporting any invention, discovery, process or other intellectual property right; (ii) presently assigning to the applicable Party or Affiliate all of his or her right, title and interest in and to any invention, discovery, process or other intellectual property; (iii) cooperating in the preparation, filing, prosecution, maintenance and enforcement of any patent or patent application; and (iv) performing all acts and signing, executing, acknowledging and delivering any and all documents required for effecting the obligations and purposes of this Agreement. It is understood and agreed that such invention assignment agreement need not reference or be specific to this Agreement.

8.3 Patent Representatives.

(a) Within thirty (30) days of the Effective Date, each Party will appoint a patent representative as the point person to manage that Party's review and comment on (a) Patent Rights being prepared, filed, prosecuted and maintained subject to the provisions in this Article VIII and (b) materials for publications, subject to the provisions in Sections 12.6 and 12.7 (the "Patent Representative"). Each Party shall be permitted to appoint a new Patent Representative upon written notice to the other Party. The Patent Representatives will meet on a regular basis at a frequency to be agreed from time to time by the Patent Representatives, but no less than [***] per year, and will (i) determine by mutual agreement whether intellectual property arising out of activities performed under this Agreement is [***] Manufacturing Technology, Joint Technology (including Joint [***] Manufacturing Improvement Technology) or LICENSEE [***] Manufacturing Improvement Technology, (ii) determine whether any such Technology has previously been conceived, discovered, invented, created, made or reduced to practice or tangible medium in the performance of either Party's rights or obligations under the Collaboration Agreement, (iii) determine by mutual agreement to update Schedule 1.64 and (iv) facilitate the exchange of information between the Parties in matters related to intellectual property.

(b) In the event the Patent Representatives cannot reach an agreement on any matter to be determined by the Patent Representatives pursuant to this Section 8.3 within thirty (30) days, such dispute shall be escalated to each Party's respective Head of Manufacturing (or person performing the functions of such role, or his/her designee) for resolution. Following such thirty (30)-day period, either Head of Manufacturing may elect to obtain an opinion on such matter

from an independent outside patent counsel mutually agreed by the Patent Representatives, the costs of which shall be borne equally by the Parties. If either Head of Manufacturing elects to obtain such an opinion, the Heads of Manufacturing shall consider such opinion, but such opinion shall not be binding on the Parties. If the Heads of Manufacturing are unable to reach agreement with respect to such decision within fifteen (15) days of (i) the date of escalation of the dispute, if neither Head of Manufacturing elects to obtain an opinion of outside patent counsel or (ii) receipt of the opinion of outside patent counsel, if a Head of Manufacturing elects to obtain such an opinion, such dispute shall be escalated to the Chief Executive Officer of each Party (or his/her nominee), and such Chief Executive Officers (or their nominees, as applicable) will meet promptly to attempt to resolve the dispute by good faith negotiations. In the event that such dispute is escalated to the CEOs (or their nominees, as applicable), the Heads of Manufacturing shall (x) obtain a non-binding opinion of independent outside patent counsel as set forth in this Section 8.3(b), if they have not already obtained such an opinion in accordance with this Section 8.3(b), and (y) provide such opinion to the CEOs (or their nominees, as applicable) for their consideration.

8.4 Invention Disclosure. LICENSEE shall notify AGTC in writing within sixty (60) days of any inventions that are directly related to activities performed under this Agreement, for the sole purpose of AGTC's compliance with reporting requirements under the Existing License Agreements and determining whether any rights to such inventions must be granted back to any Existing Licensor pursuant to the Existing License Agreements.

8.5 Patent Prosecution. Each Party shall be solely responsible for the preparation, filing prosecution and maintenance of Patent Rights owned or Controlled by such Party, subject to the following:

(a) [***] Manufacturing Patent Rights, Joint [***] Manufacturing Improvement Patent Rights and LICENSEE [***] Manufacturing Improvement Patent Rights.

(i) As between the Parties, AGTC shall, at its own expense, prepare, file, prosecute and maintain all [***] Manufacturing Patent Rights, Joint [***] Manufacturing Improvement Patent Rights and LICENSEE [***] Manufacturing Improvement Patent Rights, in all countries determined by AGTC, after consultation with LICENSEE. AGTC shall keep LICENSEE advised on the status of the prosecution of all patent applications included within such Patent Rights and the maintenance of any issued patents included within such Patent Rights. Further, AGTC shall consult and reasonably cooperate with LICENSEE with respect to the preparation, filing, prosecution and maintenance of such Patent Rights, including: (i) allowing LICENSEE a reasonable opportunity and reasonable time to review and comment regarding such drafts before any applicable filings are submitted to any relevant patent office or Governmental Authority; and (ii) considering in good faith any reasonable comments offered by LICENSEE in any final filings submitted by AGTC to any relevant patent office or Governmental Authority, to the extent such comments are intended to prevent any detrimental effect on the prosecution and maintenance of any Patent Rights owned or controlled by LICENSEE.

(ii) If AGTC elects not to file a patent application included in the Joint [***] Manufacturing Improvement Patent Rights or the LICENSEE [***] Manufacturing Improvement Patent Rights in any country or elects to cease the prosecution or maintenance of any

such Patent Right in any country, then AGTC shall provide LICENSEE with written notice immediately, but not less than thirty (30) days before any action is required, upon the decision to not file or continue the prosecution of such patent application or maintenance of such patent. In the event AGTC has provided notice to LICENSEE as described in the preceding sentence, AGTC shall permit LICENSEE, in LICENSEE's sole discretion, to file or continue prosecution or maintenance of any such Patent Right in such country at LICENSEE's expense, provided that [***], and provided, further, that, if LICENSEE has the right to file or continue prosecution or maintenance of such Patent Right, LICENSEE shall consult with AGTC with respect to the preparation, filing, prosecution and maintenance of such Patent Rights, including: (a) allowing AGTC a reasonable opportunity and reasonable time to review and comment regarding such drafts before any applicable filings are submitted to any relevant patent office or Governmental Authority, (b) reflecting any reasonable comments offered by AGTC in any final filings submitted by LICENSEE to any relevant patent office or Governmental Authority and (c) not taking any position with respect to such Patent Right that would be reasonably likely to adversely affect the scope, validity or enforceability of any of the other Patent Rights owned or Controlled by AGTC without the prior written consent of AGTC, which consent shall not be unreasonably withheld.

(b) Other Joint Patent Rights. In the event the Parties make any Joint Know-How (other than Joint [***] Manufacturing Improvement Know-How), the Patent Representatives shall promptly meet to discuss and determine whether to seek patent protection thereon. LICENSEE shall have the first right, but not the obligation, to prepare, file, prosecute and maintain any Joint Patent Right (other than any Joint [***] Manufacturing Improvement Patent Right, which, for clarity, shall be governed by Section 8.5(a)) throughout the world using patent counsel selected by LICENSEE and reasonably acceptable to AGTC. LICENSEE shall give AGTC an opportunity to review the text of any application with respect to such Joint Patent Right before filing, shall consult with AGTC with respect thereto, and shall supply AGTC with a copy of the application as filed, together with notice of its filing date and serial number. LICENSEE shall keep AGTC reasonably informed of the status of the actual and prospective patent filings (including, without limitation, the grant of any Joint Patent Rights), and shall provide advance copies of any official correspondence related to the filing, prosecution and maintenance of such patent filings. AGTC shall reimburse LICENSEE for [***] of the reasonable Out-of-Pocket Costs incurred by LICENSEE in preparing, filing, prosecuting and maintaining such Joint Patent Rights, which reimbursement will be made pursuant to invoices submitted by LICENSEE to AGTC no more often than once per Calendar Quarter. If either Party (the "Declining Party") at any time declines to share in the costs of filing, prosecuting and maintaining any such Joint Patent Right, on a country by country basis, the Declining Party shall provide the other Party (the "Continuing Party") with thirty (30) days' prior written notice to such effect, in which event, the Declining Party shall (i) have no responsibility for any expenses incurred in connection with such Joint Patent Right after the end of such thirty (30) day period and (ii) if the Continuing Party elects to continue prosecution or maintenance, the Declining Party, upon the Continuing Party's request, shall execute such documents and perform such acts, at the Continuing Party's expense, as may be reasonably necessary (A) to assign to the Continuing Party all of the Declining Party's right, title and interest in and to such Joint Patent Right and (B) to permit the Continuing Party to file, prosecute and maintain such Joint Patent Right.

8.6 Enforcement of Patent Rights.

(a) Notice. If either Party becomes aware of any potential infringement, anywhere in the world, of any issued Patent Right within the [***] Manufacturing Patent Rights, the Joint Patent Rights or the LICENSEE [***] Manufacturing Improvement Patent Rights, such Party will promptly notify the other Party in writing to that effect. Any such notice shall include any available evidence to support an allegation of infringement by such Third Party.

(b) Enforcement of [***] Manufacturing Patent Rights, Joint [***] Manufacturing Improvement Patent Rights and LICENSEE [***] Manufacturing Improvement Patent Rights. Except as otherwise provided in this Section 8.6(b), AGTC shall have the sole right, but not the obligation, in its sole discretion to defend, take action to obtain a discontinuance of infringement or bring suit against a Third Party infringer of any [***] Manufacturing Patent Right, Joint [***] Manufacturing Improvement Patent Right or LICENSEE [***] Manufacturing Improvement Patent Right, provided that AGTC shall keep LICENSEE reasonably informed of AGTC's strategy with respect to any such action and shall consider LICENSEE's comments with respect to such strategy in good faith. AGTC shall have the right to cause LICENSEE to join AGTC as a party plaintiff to any such suit, at AGTC's expense, where such joinder is necessary for the enforcement of any such Patent Right. In the case of a Third Party infringer developing, manufacturing or commercializing an AAV Product that is competitive to a Product in the same indication and targeting the same gene (a "Competitive Infringement") of any such [***] Manufacturing Patent Right, Joint [***] Manufacturing Improvement Patent Right or LICENSEE [***] Manufacturing Improvement Patent Right, unless AGTC has notified LICENSEE that it does not wish to bring such action or does not bring such action within the period of time set by court decree, the Parties shall jointly take action to obtain a discontinuance of infringement or bring suit in a Competitive Infringement. Alternatively, if AGTC has notified LICENSEE that it does not wish to join such action or does not join within a period of time set by court decree, LICENSEE may take such action without AGTC in which case LICENSEE shall have the right to cause AGTC to join LICENSEE as a party plaintiff in such suit, at LICENSEE's expense, where joinder is necessary for enforcement of the Patent Right. Each Party shall bear its own expenses in connection with any action taken by a Party pursuant to this Section 8.6(b). Any recovery obtained by AGTC as a result of any proceeding that is not a Competitive Infringement proceeding shall be retained by AGTC. Any recovery obtained by either Party as a result of any Competitive Infringement proceeding against a Third Party infringer shall be allocated as follows:

(i) such recovery shall first be used to reimburse each Party pro rata for all litigation costs in connection with such litigation paid by that Party; and

(ii) LICENSEE shall retain [***] and AGTC shall retain [***] of the remaining portion of any such recovery.

(c) Enforcement of Other Joint Patent Rights. Except as otherwise provided in this Section 8.6(c), LICENSEE shall have the first right, but not the obligation, to take action to obtain a discontinuance of infringement or bring suit against a Third Party infringer in a Competitive Infringement of any Joint Patent Right that is not a Joint [***] Manufacturing Improvement Patent Right. LICENSEE shall have the right to cause AGTC to join LICENSEE as

a party plaintiff to any such suit, at LICENSEE's expense, where such joinder is necessary for the enforcement of any such Joint Patent Right. If, ninety (90) days after the date of notice given pursuant to Section 8.6(a), LICENSEE has not obtained a discontinuance of infringement of such Joint Patent Right, filed suit against any such Third Party infringer of such Joint Patent Right or provided AGTC with information and arguments demonstrating to AGTC's reasonable satisfaction that there is insufficient basis for the allegation of such infringement of such Joint Patent Right, then AGTC shall have the right, but not the obligation, to bring suit against such Third Party infringer of such Joint Patent Right. With respect to any infringement of a Joint Patent Right that is not a Joint [***] Manufacturing Improvement Patent Right, where such infringement is not a Competitive Infringement, the Parties shall determine by mutual agreement (a) whether to take action to obtain a discontinuance of infringement or bring suit against a Third Party infringer and (b) which Party shall take control of such action or suit. Each Party shall bear its own expenses in connection with any action taken by a Party pursuant to this Section 8.6(c). Any recovery obtained by either Party as a result of any such proceeding against a Third Party infringer shall be allocated as follows:

(i) Such recovery shall first be used to reimburse each Party for all litigation costs in connection with such litigation paid by that Party; and

(ii) if the recovery arose out of a Competitive Infringement proceeding, then LICENSEE shall retain [***] and AGTC shall retain [***] of the remaining portion of any such recovery; and if the recovery arose out of any proceeding that is not a Competitive Infringement proceeding, then the Parties shall share the remaining portion of such recovery equally.

(d) Settlements. With respect to any action, suit, proceeding or claim involving a Patent Right under Section 8.6(b) (solely in the case of a Competitive Infringement) or Section 8.6(c), the enforcing Party shall not consent to the entry of any judgment or enter into any settlement with respect to such an action or suit without the prior written consent of the other Party (which consent shall not unreasonably be withheld or delayed).

(e) Cooperation. Each Party shall cooperate (including by executing any documents required to enable the other Party to initiate such litigation) with the other Party in any suit for infringement of any such Patent Right brought by the other Party against a Third Party in accordance with this Section 8.6, and shall have the right to consult with the other Party and to participate in and be represented by independent counsel in such litigation. Neither Party shall incur any liability to the other Party as a consequence of such litigation or any unfavorable decision resulting therefrom, including any decision holding any such Patent Right invalid or unenforceable.

8.7 Infringement and Third Party Licenses.

(a) Infringement of Third Party Patents. If the exploitation of the [***] Manufacturing Technology under this Agreement by LICENSEE or any of its Affiliates or Sublicensees is alleged by a Third Party to infringe such Third Party's Patent Rights or other intellectual property rights, the Party becoming aware of such allegation shall promptly notify the

other Party. Additionally, if either Party determines that, based upon the review of any Third Party Patent Right or other Third Party intellectual property rights, it may be desirable to obtain a license from such Third Party with respect thereto so as to avoid any potential claim of infringement by such Third Party against either Party or their respective Affiliates or Sublicensees, such Party shall promptly notify the other Party of such determination and initiate discussions to determine whether such a license is desirable.

(b) Negotiating Third Party Licenses.

(i) Either Party shall have the right to obtain a license under one or more Patent Rights or other intellectual property rights owned or controlled by a Third Party that are necessary or useful to exploit the [***] Manufacturing Technology (collectively, "Third Party IP Rights"), provided that, (a) if AGTC is the licensee, AGTC is granted a sublicensable license under such Third Party IP Rights permitting AGTC and LICENSEE and their respective Affiliates and sublicensees to practice such Third Party IP Rights in connection with the performance of any of their respective obligations or the exercise of any of their respective rights under this Agreement, under terms and conditions that, to the extent applicable to LICENSEE as a sublicensee of such Third Party IP Rights, are not more onerous in any material respect on LICENSEE than those contained in this Agreement and (b) if LICENSEE is the licensee, [***], to the extent applicable to AGTC as a sublicensee of such Third Party IP Rights, are not more onerous in any material respect on AGTC than those contained in this Agreement. Upon entry into any such agreement, the contracting Party shall promptly provide a copy of such agreement to the other Party and, in the case where AGTC is the contracting Party, AGTC shall provide LICENSEE with a proposed allocation of upfront payments contemplated by Section 5.4(d). In the case of any such agreement entered into by AGTC, LICENSEE may, but shall not be required to, at any time after LICENSEE receives such copy, elect to take a sublicense to such Third Party IP Rights by notice to AGTC, and thereafter LICENSEE's payment obligations under Section 8.7(b)(ii) shall apply, and the Know-How and Patent Rights included in such sublicense shall thereafter be deemed [***] Manufacturing Technology.

(ii) LICENSEE shall be responsible for any payments under any such agreement that LICENSEE enters into during the Term. Any payments under any such agreement that AGTC enters into during the Term shall be treated in accordance with the provisions of Section 5.4(d).

(c) Third Party Infringement Suit.

(i) If a Third Party sues LICENSEE or any of LICENSEE's Affiliates or Sublicensees (each Person so sued being referred to herein as a "Sued Party"), alleging that the exploitation of the [***] Manufacturing Technology by LICENSEE or any of LICENSEE's Affiliates or Sublicensees during the Term and pursuant to this Agreement infringe or will infringe such Third Party's Patent Rights, then, if such suit is an indemnifiable claim under Section 10.2, such suit shall, at LICENSEE's election, be subject to the indemnification provisions of Article X. If LICENSEE does not seek indemnification under Section 10.2 with respect to such suit, or if such suit is not an indemnifiable claim, then, to the extent such action involves [***] Manufacturing Technology, LICENSEE shall so notify AGTC and AGTC shall have the first

right, but not the obligation to defend against any such action, at its own expense and LICENSEE shall have the sole right, but not the obligation to defend the remainder of such action, provided that, if AGTC makes an [***] Defense Election, AGTC shall keep LICENSEE reasonably informed of AGTC's strategy with respect to such action and shall consider LICENSEE's comments with respect to such strategy in good faith. AGTC shall notify LICENSEE within five (5) days of notice of such suit whether AGTC wishes to exercise its first right as set forth in the preceding sentence (AGTC's election to exercise such first right, an "[***] Defense Election"). If AGTC notifies LICENSEE that it will not make an [***] Defense Election, or if AGTC does not respond within such five (5) day period, LICENSEE shall have the sole right to defend such action, in its own name, and any such defense shall be at LICENSEE's expense. Upon the defending Party's request, the other Party may, in its sole discretion, consent to join, and will join if necessary under applicable Law, in any such action at the defending Party's expense and cooperate with the defending Party at the defending Party's expense. If AGTC fails to defend against any such action for which AGTC has made an [***] Defense Election, then AGTC shall provide LICENSEE with sufficient notice to enable LICENSEE to assume the defense of such action, and shall indemnify LICENSEE against any Liabilities arising from AGTC's failure to provide such timely notice or any action or inaction that prejudices LICENSEE's ability to defend such action. In such event, LICENSEE shall have the right to defend such action, in its own name, and any such defense shall, subject to the preceding sentence, be at LICENSEE's expense.

(ii) Each Party shall reasonably cooperate in any such action at the defending Party's expense and in connection with the defending Party's defense of any such Third Party infringement suit, each Party shall provide reasonable assistance to the defending Party for such defense. All activities under this Section 8.7(c) shall be conducted at the expense of the Party defending against any action pursuant to this Section 8.7(c). In the event that more than one Party is defending against such action, the Parties shall cooperate in good faith and coordinate defense strategy.

(iii) Any recovery obtained by LICENSEE as a result of any proceeding under Section 8.7(c)(i) in which AGTC has not made an [***] Defense Election shall be retained by LICENSEE. Any recovery obtained by AGTC as a result of any proceeding under Section 8.7(c)(i) for which AGTC has made an [***] Defense Election shall be allocated as follows:

(A) such recovery shall first be used to reimburse each Party pro rata for all litigation costs in connection with such litigation paid by that Party;

(B) AGTC shall retain the portion of the recovery allocable to the defense of the [***] Manufacturing Technology if set forth in the judgment awarded in such action and otherwise as mutually agreed by the Parties; and

(C) LICENSEE shall retain the remainder of the recovery.

(iv) With respect to any action, suit, proceeding or claim involving an [***] Manufacturing Patent Right under this Section 8.7(c), the defending Party shall not enter into a settlement with respect to such action or suit without the prior written consent of the other

Party if such settlement includes an admission of culpability or infringement by such other Party or a payment obligation by such other Party, or imposes material restrictions on such other Party.

(v) In any proceeding under Section 8.7(c)(i) for which AGTC has not made an [***] Defense Election, subject to Article X, LICENSEE shall be solely responsible for any Liabilities incurred in connection with such action. In any proceeding under Section 8.7(c)(i)for which AGTC has made an [***] Defense Election, subject to Article X, AGTC shall be solely responsible for any Liabilities incurred and allocable to the [***] Manufacturing Technology if set forth in the judgment awarded in such action and otherwise as mutually agreed by the Parties.

(d) Administrative Actions by Third Parties. Each Party shall promptly notify the other Party in the event of any administrative action involving any [***] Manufacturing Patent Right, Joint Patent Right or LICENSEE [***] Manufacturing Improvement Patent Right of which it becomes aware, including any nullity, revocation, reexamination, opposition, interference, inter partes and post-grant review or compulsory license proceeding. AGTC shall have the first right, but no obligation, to defend against any such action, in its own name and at its own expense. Upon AGTC's request, LICENSEE may, in its sole discretion, consent to join, and will join if necessary under applicable Law, in any such action at AGTC's expense and cooperate with AGTC at AGTC's expense. If AGTC fails to defend against any such action within ten (10) days of notice thereof, then LICENSEE shall have the right to defend such action, in its own name, and any such defense shall be at LICENSEE's expense. In such event, AGTC shall reasonably cooperate, upon LICENSEE's request, in any such action at LICENSEE's expense.

(e) Administrative Actions Against Third Parties. Each Party shall promptly notify the other Party in the event it wishes to initiate an administrative action against a Third Party involving a Patent Right claiming any use, production, manufacture, or expression of a recombinant AAV using [***] (in which the Patent Right does not claim a recombinant AAV or [***] comprising [***] or the use thereof) ("[***] Manufacturing"), including any nullity, revocation, reexamination, opposition, interference, inter partes and post-grant review or compulsory license proceeding. AGTC shall have the sole right to initiate any such action involving [***] Manufacturing, in its own name and at its own expense. Upon AGTC's request, LICENSEE may, in its sole discretion, consent to join, and will join if necessary under applicable Law, in any such action at AGTC's expense and cooperate with AGTC at AGTC's expense. LICENSEE shall have the sole right to initiate any such action involving any Patent Right that does not cover [***] Manufacturing. AGTC, upon LICENSEE's request, may, in its sole discretion, consent to join, and will join if necessary under applicable Law, in any such action at LICENSEE's expense and cooperate with LICENSEE at LICENSEE's expense.

(f) Paragraph IV Notices. Each Party shall immediately give written notice to the other of any certification of which it becomes aware filed pursuant to any statutory or regulatory requirement in any country in the Territory similar to 21 U.S.C. § 355(b)(2)(A)(iv) or § 355(j)(2)(A)(vii)(IV) (or any amendment or successor statute thereto) claiming that any [***] Manufacturing Patent Right, Joint Patent Right or LICENSEE [***] Manufacturing Improvement Patent Right covering any Product is invalid or that infringement will not arise from the Development, Manufacture, use or Commercialization in the Territory of such Product by a Third

Party. Upon the giving or receipt of such notice, the provisions of Section 8.6 with respect to division of enforcement responsibilities shall apply, *mutatis mutandis*, with respect to any infringement action against such Third Party. In each case, the Party with the right to bring an infringement action shall notify the other Party at least ten (10) days prior to the date set forth by statute or regulation of its intent to exercise, or not exercise, this right. Any infringement action against a Third Party arising under this Section 8.7(f) shall be governed by the provisions of Section 8.6. Without limiting any provision of Section 8.6, in order to establish standing in connection with any action under this this Section 8.7(f), upon the request of the Party bringing the action, the other Party shall reasonably cooperate in any such action at the expense of the Party bring the action and shall timely commence or join in any such action at the request and expense of the Party bringing the action.

8.8 Patent Term Restoration. The Parties shall reasonably cooperate with each other in obtaining patent term restoration in any country in the Territory under any statute or regulation equivalent or similar to 35 U.S.C. § 156, where applicable to the [***] Manufacturing Patent Rights, Joint Patent Rights or LICENSEE [***] Manufacturing Improvement Patent Rights. If any election with respect to seeking such patent term restoration is to be made in any country in the Territory, with respect to an [***] Manufacturing Patent Right, Joint Patent Right or LICENSEE [***] Manufacturing Improvement Patent Right, then AGTC shall make such election (including, without limitation, by filing supplementary protection certificates and any other extensions that are now or in the future become available) and LICENSEE shall abide by such election and cooperate, as reasonably requested by AGTC, in connection with the foregoing (including, without limitation, by providing appropriate information and executing appropriate documents).

8.9 Recording. If either Party deems it necessary or desirable for any reason to register or record this Agreement or evidence of this Agreement with any patent office or other appropriate Governmental Authority(ies) in one or more jurisdictions in the Territory, the other Party shall reasonably cooperate to execute and deliver to such Party any documents accurately reflecting or evidencing this Agreement that are necessary or desirable, in such Party's reasonable judgment, to complete such registration or recordation. The registering or recording Party shall reimburse the other Party for all reasonable Out-of-Pocket Costs, including attorneys' fees, incurred by such other Party in complying with the provisions of this Section 8.9.

8.10 Patent Marking. LICENSEE shall apply patent markings that meet all requirements of U.S. law Title 35 of United States Code, including without limitation, 35 U.S.C. §287, with respect to all Products subject to this Agreement. LICENSEE shall mark the Products sold in the United States with all applicable United States patent numbers. All Products shipped to or sold in other countries shall be marked in such manner as to conform with the patent laws and practice of the country of manufacture or sale. Any Products subject to Patent Rights under an Existing License Agreement that are sold or produced in the United States shall be Manufactured substantially in the United States to the extent required by applicable Law. LICENSEE shall take all reasonable action necessary on its part as a licensee of any Patent Rights under an Existing License Agreement to enable the Existing Licensors to satisfy their respective obligations to the United States government under Title 35 of the United States Code.

## ARTICLE IX
## REPRESENTATIONS AND WARRANTIES

9.1 Mutual Representations. Except as may be disclosed in Schedule 9.1, which may be updated within five (5) days following the HSR Clearance Date, each of AGTC and LICENSEE hereby represents, warrants and covenants to the other Party as of the Execution Date and the Effective Date as follows:

(a) it is a corporation duly organized, validly existing and in good standing under the laws of the state of its incorporation;

(b) it (i) has the requisite power and authority and the legal right to enter into this Agreement and to perform its obligations hereunder, (ii) has the requisite resources and expertise to perform its obligations hereunder and (iii) has taken all requisite action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder;

(c) this Agreement has been duly executed and delivered on behalf of such Party, and constitutes a legal, valid, binding obligation, enforceable against such Party in accordance with its terms;

(d) it has obtained all necessary consents, approvals and authorizations of all Governmental Authorities and other persons or entities required to be obtained by such Party in connection with the execution and delivery of this Agreement;

(e) the execution, delivery and performance by such Party of this Agreement and its compliance with the terms and provisions hereof does not and will not conflict with or result in a breach of any of the terms and provisions of or constitute a default under (i) a loan agreement, guaranty, financing agreement, agreement relating to one or more Patent Rights or other agreement or instrument binding or affecting it or its property; (ii) the provisions of its charter or operative documents or bylaws; or (iii) any order, writ, injunction or decree of any court or Governmental Authority entered against it or by which any of its property is bound

(f) it has not, and will not, after the Execution Date and during the Term, grant any right to any Third Party that would conflict with the rights granted to the other Party or would be inconsistent with its obligations hereunder; and

(g) it shall at all times comply with all material Laws applicable to its activities under this Agreement.

9.2 Representations, Warranties and Covenants of AGTC. In addition to the representations, warranties and covenants made by AGTC elsewhere in this Agreement, except as disclosed in Schedule 9.2 as may be updated in accordance with this Section 9.2, and subject to the scope of the license grants and retained rights and other exclusions set forth in this Agreement, AGTC hereby represents, warrants and covenants to LICENSEE (i) as of the Execution Date and the Effective Date (provided that AGTC may (1) supplement Schedule 9.2 or (2) add one or more new schedules or exhibits to this Section 9.2 with respect to the applicable representation and warranty made as of

the Effective Date in each case ((1) and (2)) within five (5) days following the HSR Clearance Date, but any such supplement or new schedule may only contain information arising after the Execution Date and may not correct, modify or delete any information set forth in any such schedule on the Execution Date):

(a) it owns or Controls the [***] Manufacturing Technology;

(b) it has sufficient right, power and authority to grant all of the right, title and interest in the licenses granted or to be granted to LICENSEE under this Agreement;

(c) the issued [***] Manufacturing Patent Rights are, to its Knowledge, valid and enforceable patents and it has not received written notice challenging the extent, validity or enforceability of the [***] Manufacturing Patent Rights (including by way of example through the institution or written threat of institution of interference, nullity, opposition, inter partes or post grant review or similar invalidity proceedings before the United States Patent and Trademark Office or any analogous foreign Governmental Authority;

(d) all terms and conditions of the Existing License Agreements applicable to LICENSEE in its role as sublicensee or otherwise required to be included in sublicense agreements under the Existing License Agreements are expressly set forth in this Agreement;

(e) it will not without LICENSEE's written consent, amend any AGTC Third Party Agreement in a manner that materially adversely affects the rights granted to LICENSEE hereunder or AGTC's ability to fully perform its obligations hereunder;

(f) it will promptly furnish LICENSEE with copies of all (i) amendments of the AGTC Third Party Agreements and (ii) correspondence with or from licensors under the AGTC Third Party Agreements to the extent material to LICENSEE or the rights granted to LICENSEE or LICENSEE's Affiliates under this Agreement;

(g) Schedule 1.64 contains a complete and correct list of all [***] Manufacturing Patent Rights owned or otherwise Controlled by AGTC or its Affiliates (and indicating which entity owns or Controls each Patent Right and which are owned and which are Controlled);

(h) it has, and to its Knowledge, its licensors have, complied with all material respects with all applicable Laws, including, with respect to any issued patents and pending patent applications (excluding United States Provisional patent applications) any disclosure requirements of the USPTO or any other Governmental Authority, in connection with the filing, prosecution, and maintenance of the [***] Manufacturing Patent Rights, and it has, and to its Knowledge, its licensors have, timely paid all filing and renewal fees payable with respect to the [***] Manufacturing Patent Rights for which it controls prosecution and maintenance;

(i) it has obtained, or caused its Affiliates, as applicable, to obtain, assignments from inventors of all inventorship rights to the [***] Manufacturing Patent Rights that are owned by AGTC or such Affiliates and, to AGTC's Knowledge, there has been no failure on the part of any licensor of the [***] Manufacturing Patent Rights that are licensed by AGTC to obtain

assignments from the inventors of all inventorship rights to such licensed [***] Manufacturing Patent Rights, and to AGTC's Knowledge, all assignments of inventorship rights relating to the [***] Manufacturing Patent Rights are valid and enforceable, and the inventorship of the [***] Manufacturing Patent Rights owned by AGTC, and to AGTC's Knowledge, of the [***] Manufacturing Patent Rights licensed to AGTC, is properly identified on each patent or patent application; and

(j) it will file, prosecute and maintain the [***] Manufacturing Patent Rights during the Term, provided that it shall have no obligation to file, prosecute and maintain such patent rights [***].

9.3 Special Exceptions for Existing Licensors. Notwithstanding anything to the contrary in this Agreement, nothing in this Agreement shall be construed as:

(a) a warranty or representation by UFRF as to the validity or scope of any right included in the [***] Manufacturing Patent Rights licensed under the UF/JHU Agreement;

(b) a warranty or representation that anything made, used, sold or otherwise disposed of under the license granted in the UF/JHU Agreement will or will not infringe patents of Third Parties;

(c) an obligation to bring or prosecute actions or suits against Third Parties for infringement of [***] Manufacturing Patent Rights granted in the UF/JHU Agreement;

(d) an obligation to furnish any Know-How not provided in [***] Manufacturing Patent Rights granted in the UF/JHU Agreement or any services other than those specified in the UF/JHU Agreement; or

(e) a warranty or representation by UFRF that it will not grant licenses to others to make, use or sell products not covered by the claims of the [***] Manufacturing Patent Rights granted in the UF/JHU Agreement which may be similar and/or compete with products made or sold by LICENSEE.

9.4 Additional Covenant and Representation of LICENSEE.

(a) In addition to the representations, warranties and covenants made by LICENSEE elsewhere in this Agreement, LICENSEE hereby covenants to AGTC that LICENSEE shall not encumber, other than under sublicenses as expressly permitted under this Agreement, or otherwise grant a security interest in, any of the AGTC Technology to any Third Party.

(b) LICENSEE represents and warrants that it will comply, and will ensure that its Affiliates comply, with all local, state and international laws and regulations relating to the [***] Biological Material and to the development, manufacture, use, sale and importation of [***] Viruses and [***] Products. Without limiting the foregoing, LICENSEE represents and warrants that it will comply with all United States export control laws and regulations with respect to [***]

Biological Material and any [***] Viruses and [***] Products developed or made through the use thereof.

9.5 UFRF Disclaimer. EXCEPT AS OTHERWISE EXPRESSLY SET FORTH IN THE UF/JHU AGREEMENT, UFRF MAKES NO REPRESENTATIONS AND EXTENDS NO WARRANTIES OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING BUT NOT LIMITED TO WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, AND VALIDITY OF PATENT RIGHTS CLAIMS, ISSUED OR PENDING. UFRFASSUMES NO RESPONSIBILITIES WHATSOEVER WITH RESPECT TO USE, SALE, OR OTHER DISPOSITION BY LICENSEE, ITS SUBLICENSEE(S), OR THEIR VENDEES OR OTHER TRANSFEREES OF PRODUCTS INCORPORATING OR MADE BY USE OF INVENTIONS LICENSED UNDER SUCH AGREEMENT.

9.6 Duties of the Parties. None of the licensors under the UF/JHU Agreement are commercial organizations. They are institutes of research and education. Therefore, such licensors have no ability to evaluate the commercial potential of any [***] Manufacturing Patent Rights or processes or other license or rights granted in such Agreement. It is therefore incumbent upon LICENSEE to evaluate the rights and products in question, to examine the materials and information provided by such licensors, and to determine for itself the validity of any [***] Manufacturing Patent Rights or processes licensed under such Agreement, its freedom to operate, and the value of any such [***] Manufacturing Patent Rights or processes or other rights granted.

9.7 Representations by JHU. JHU has represented to AGTC that it has good and marketable title to its interest in the inventions claimed under [***] Manufacturing Patent Rights licensed under the UF/JHU Agreement with the exception of certain retained rights of the United States government, which may apply if any part of the JHU research was funded in whole or in part by the United States Government. JHU does not warrant the validity of any patents or that practice under such patents shall be free of infringement. EXCEPT AS EXPRESSLY SET FORTH IN THIS SECTION 9.7, LICENSEE, AND LICENSEE'S AFFILIATES AND SUBLICENSEE(S) AGREE THAT THE [***] MANUFACTURING PATENT RIGHTS LICENSED UNDER THE UF/JHU AGREEMENT ARE PROVIDED "AS IS", AND THAT JHU MAKES NO REPRESENTATION OR WARRANTY WITH RESPECT TO THE PERFORMANCE OF SUCH LICENSED PRODUCT(S) AND LICENSED PROCESSES INCLUDING THEIR SAFETY, EFFECTIVENESS, OR COMMERCIAL VIABILITY. JHU DISCLAIMS ALL WARRANTIES WITH REGARD TO SUCH PRODUCT(S) AND PROCESSES(S) LICENSED UNDER THE UF/JHU AGREEMENT, INCLUDING, BUT NOT LIMITED TO, ALL WARRANTIES, EXPRESSED OR IMPLIED, OF MERCHANTABILITY AND FITNESS FOR ANY PARTICULAR PURPOSE. NOTWITHSTANDING ANY OTHER PROVISION OF THIS AGREEMENT, JHU ADDITIONALLY DISCLAIMS ALL OBLIGATIONS AND LIABILITIES ON THE PART OF JHU AND INVENTORS, FOR DAMAGES, INCLUDING, BUT NOT LIMITED TO, DIRECT, INDIRECT, SPECIAL AND CONSEQUENTIAL DAMAGES, ATTORNEYS' AND EXPERTS' FEES, AND COURT COSTS (EVEN IF JHU HAS BEEN ADVISED OF THE POSSIBILITY OF SUCH DAMAGES, FEES OR COSTS), ARISING OUT OF OR IN CONNECTION WITH THE MANUFACTURE, USE, OR SALE OF THE PRODUCT(S) AND PROCESSES LICENSED UNDER THIS AGREEMENT. LICENSEE, AND LICENSEE'S AFFILIATES AND SUBLICENSEE(S) ASSUME ALL RESPONSIBILITY

AND LIABILITY FOR LOSS OR DAMAGE CAUSED BY A PRODUCT AND/OR PROCESS MANUFACTURED, USED, OR SOLD BY LICENSEE, ITS SUBLICENSEE(S) AND AFFILIATED COMPANIES WHICH IS A LICENSED PRODUCT(S) OR LICENSED PROCESSES AS DEFINED IN THE UF/JHU AGREEMENT.

9.8 Representation by Legal Counsel. Each Party hereto represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption shall exist or be implied against the Party which drafted such terms and provisions.

9.9 Disclaimer. THE FOREGOING WARRANTIES OF EACH PARTY ARE IN LIEU OF ANY OTHER WARRANTIES, EXPRESS OR IMPLIED, INCLUDING ANY IMPLIED WARRANTIES OF MERCHANTABILITY OR ANY IMPLIED WARRANTIES OF FITNESS FOR A PARTICULAR PURPOSE, ALL OF WHICH ARE HEREBY SPECIFICALLY EXCLUDED AND DISCLAIMED.

9.10 No Guarantee of Success. LICENSEE and AGTC acknowledge and agree that nothing in this Agreement will be construed as representing any estimate or projection of (a) the successful Development or Commercialization of any Product under this Agreement, (b) the number of Products that will or may be successfully Developed or Commercialized under this Agreement, (c) anticipated sales or the actual value of any Products that may be successfully Developed or Commercialized under this Agreement or (d) the damages, if any, that may be payable if this Agreement is terminated for any reason. Neither Party makes any representation, warranty or covenant, either express or implied, that (i) it will successfully Develop, Manufacture, Commercialize or, other than is expressly required under Article VI, continue to Commercialize any Product in any country, (ii) if Commercialized, that any Product will achieve any particular sales level, whether in any individual country or cumulatively throughout the Territory or (iii) other than is expressly required under Article VI, that either Party will devote, or cause to be devoted, any level of diligence or resources to Developing or Commercializing any Product in any country, or in the Territory in general.

## ARTICLE X
## INDEMNIFICATION; INSURANCE

10.1 Indemnification by LICENSEE. LICENSEE will indemnify, defend and hold harmless AGTC, each of its Affiliates and each licensor of the [***] Manufacturing Technology, and each of its and its Affiliates' or such licensor's employees, officers, directors, trustees and agents and inventors of [***] Manufacturing Technology licensed under the UAB Agreement (each, an "AGTC Indemnified Party") from and against any and all liability, loss, damage, expense (including reasonable attorneys' fees and expenses) and cost (collectively, a "Liability") that the AGTC Indemnified Party may be required to pay to one or more Third Parties resulting from or arising out of:

(a) any claims of any nature arising out of the Development, Manufacture, Commercialization, consumption or use of any Product by or on behalf of, LICENSEE (other than

by any AGTC Indemnified Party), or under the authority of LICENSEE including without limitation death of or injury to any Person or out of damage to property, other than claims for which AGTC is required to indemnify LICENSEE pursuant to Section 10.2; or

(b) the breach by LICENSEE of any of its representations, warranties, covenants or obligations set forth in this Agreement;

except, in each case, to the extent such Liabilities are caused by the recklessness, negligence or intentional misconduct of AGTC or any AGTC Indemnified Party.

10.2 Indemnification by AGTC. AGTC will indemnify, defend and hold harmless LICENSEE, its Affiliates, Sublicensees, Distributors and each of its and their respective employees, officers, directors and agents (each, a "LICENSEE Indemnified Party") from and against any and all Liabilities that the LICENSEE Indemnified Party may be required to pay to one or more Third Parties resulting from or arising out of:

(a) the breach by AGTC of any of its representations, warranties, covenants or obligations set forth in this Agreement; or

(b) any claim that the practice of the [***] Manufacturing Technology to Develop, Manufacture, Commercialize or use any Product infringes or misappropriates any issued patent or other proprietary right owned or possessed by any Third Party, other than any such claim to the extent that (i) it is based on the practice of the [***] Manufacturing Technology in combination with Technology other than [***] Manufacturing Technology that is utilized in the Development, Manufacture, Commercialization or use of any Product as a result of LICENSEE's exercise of its final decision-making authority or (ii) it arises from LICENSEE's election not to take a license or sublicense to any Technology under Section 8.7(b)(i);

except, in each case, to the extent such Liabilities are caused by the recklessness, negligence or intentional misconduct of LICENSEE or any LICENSEE Indemnified Party.

10.3 Special Indemnification by LICENSEE for Existing License Agreements.

(a) LICENSEE shall, at all times during the term of this Agreement and thereafter, indemnify, defend and hold UFRF, the Florida Board of Governors, the University of Florida Board of Trustees, the University of Florida, and each of their directors, officers, employees, and agents, and the inventors of the any Patent Rights licensed to AGTC under the UFRF Existing License Agreements, regardless of whether such inventors are employed by the University of Florida at the time of the claim, harmless against all claims and expenses, including legal expenses and reasonable attorneys' fees, whether arising from a Third Party claim or resulting from UFRF's enforcing this indemnification clause against LICENSEE arising out of the death of or injury to any person or persons or out of any damage to property and against any other claim, proceeding, demand, expense and liability of any kind whatsoever (other than patent infringement claims) resulting from the production, manufacture, sale, use, lease, consumption, marketing, or advertisement of Products or use of any processes licensed hereunder or arising from any right or obligation of LICENSEE hereunder. Notwithstanding the above, UFRF at all times reserves the right to retain counsel of its own to defend UFRF's, the Florida Board of Governors', the University of Florida Board of Trustees', the University of Florida's, and the inventor's interests.

(b) LICENSEE warrants that it now maintains and will continue to maintain liability insurance coverage appropriate to the risk involved in producing, manufacturing, selling, marketing, using, leasing, consuming, or advertising the products subject to this Agreement. Notwithstanding the foregoing, LICENSEE may self-insure to the extent that it self-insures for its other products.

(c) JHU and [***] who are employees of JHU (hereinafter "JHU Inventors") will have no legal liability exposure to Third Parties if JHU does not license the Products and processes licensed under the UF/JHU Agreement, and any royalties JHU and the JHU Inventors may receive is not adequate compensation for such legal liability exposure. Furthermore, JHU and JHU Inventors will not, under the provisions of the UF/JHU Agreement or otherwise, have control over the manner in which LICENSEE or its Affiliates or its Sublicensees or those operating for its account or Third Parties who purchase Products and processes licensed under the UF/JHU Agreement from any of the foregoing entities, develop, manufacture, market or practice the inventions of such Products and processes. Therefore, LICENSEE, and its Affiliates and Sublicensees shall indemnify, defend with counsel reasonably acceptable to JHU, and hold JHU, The Johns Hopkins Health Systems, their present and former trustees, officers, JHU Inventors, agents, faculty, employees and students harmless as against any judgments, fees, expenses, or other costs arising from or incidental to any product liability or other lawsuit, claim, demand or other action brought as a consequence of the practice of said inventions by any of the foregoing entities, whether or not JHU or said JHU Inventors, either jointly or severally, is named as a party defendant in any such lawsuit and whether or not JHU or the JHU Inventors are alleged to be negligent or otherwise responsible for any injuries to persons or property. Practice of the inventions covered by such Products and processes, by an Affiliate or an agent or a Sublicensee or a Third Party on behalf of or for the account of LICENSEE or by a Third Party who purchases such Products and processes from LICENSEE, shall be considered LICENSEE's practice of said inventions for purposes of this Section 10.3(c). The obligation of LICENSEE to defend and indemnify as set out in this Section 10.3(c) shall survive the termination of this Agreement or the UF/JHU Agreement, shall continue even after assignment of rights and responsibilities to an Affiliate or Sublicensee, and shall not be limited by any other limitation of liability elsewhere in this Agreement or the JHU/UF Agreement.

(d) LICENSEE shall indemnify, defend and hold harmless [***] and its current and former directors, governing board members, trustees, officers, faculty, medical and professional staff, employees, students, and agents and their respective successors, heirs and assigns (collectively, the "[***] Indemnitees") from and against any claim, liability, cost, expense, damage, deficiency, loss or obligation of any kind or nature (including reasonable attorneys' fees and other costs and expenses of litigation) by or owed to a Third Party, based upon, arising out of, or otherwise relating to the activities of LICENSEE, its Affiliates and Sublicensees under this Agreement, including any cause of action relating to product liability concerning any product, process, or service made, used, sold or performed pursuant to any right or license granted under this Agreement (collectively, the "[***] Claims"); provided, however, that LICENSEE's indemnification obligations hereunder shall not apply to any [***] Claim to the extent that it is attributable to the gross negligence or willful misconduct of any [***] Indemnitee.

(e) LICENSEE shall, at its own expense, provide attorneys reasonably acceptable to [***] to defend against any actions brought or filed against any [***] Indemnitee hereunder with respect to the subject of indemnity contained herein, whether or not such actions are rightfully brought. Any [***] Indemnitee seeking indemnification hereunder shall promptly notify LICENSEE of such [***] Claim; provided that any failure of or delay in such notification shall not affect LICENSEE's indemnification obligation unless and to the extent such failure or delay is materially prejudicial to LICENSEE. The [***] Indemnitees shall provide LICENSEE, at LICENSEE's expense, with reasonable assistance and full information with respect to such [***] Claim and give LICENSEE sole control of the defense of any [***] Claim. Neither LICENSEE nor [***] shall settle any [***] Claim without the prior written consent of the other, which consent shall not be unreasonably withheld.

(f) LICENSEE and its Sublicensees shall, at all times during the term of the UAB Agreement and thereafter, indemnify, defend and hold UABRF and UAB and the inventors of the [***] Manufacturing Patent Rights licensed under the UAB Agreement (each a "UAB Indemnified Party") harmless against all claims and expenses, including legal expenses and reasonable attorneys' fees, arising out of the death of or injury to any person or persons or out of any damage to property and against any other claim, proceeding, demand, expense and liability of any kind whatsoever (other than patent infringement claims) resulting from the production, manufacture, sale, use, lease, consumption or advertisement of Products arising from any right or obligation of LICENSEE or any Sublicensee under the UAB Agreement or for LICENSEE's or any Sublicensee's breach of terms and conditions herein except to the extent that such claims are due to the gross negligence or willful misconduct of a UAB Indemnified Party. Notwithstanding the above, UABRF at all times reserves the right to retain counsel of its own to defend UABRF's UAB's and the inventors' interests. UABRF has agreed to promptly notify AGTC in writing of any such claim, and AGTC shall promptly notify LICENSEE of such notification, and LICENSEE shall manage and control, at its own expense, the defense of such claim and its settlement. LICENSEE agrees not to settle any such claim against UABRF without UABRF's written consent where such settlement would include any admission of liability on the part of UABRF, where the settlement would impose any restriction on the conduct of UABRF of any of its activities, or where the settlement would not include an unconditional release of UABRF from all liability for claims that are the subject matter of such claim.

10.4 Indemnification Procedure. Each Party will notify the other Party in writing in the event it becomes aware of a claim for which indemnification may be sought hereunder. In case any proceeding (including any governmental investigation) shall be instituted involving any Party in respect of which indemnity may be sought pursuant to this Article X, such Party (the "Indemnified Party") shall promptly notify the other Party (the "Indemnifying Party") in writing and the Indemnifying Party and Indemnified Party shall meet to discuss how to respond to any claims that are the subject matter of such proceeding. The Indemnified Party shall reasonably cooperate with the Indemnifying Party in defense of such matter. The Indemnifying Party, upon request of the Indemnified Party, shall retain counsel reasonably satisfactory to the Indemnified Party to represent the Indemnified Party and shall pay the fees and expenses of such counsel related to such proceeding. In any such proceeding, the Indemnified Party shall have the right to retain its own counsel, but the fees and expenses of such counsel shall be at the expense of the Indemnified Party unless (a) the Indemnifying Party and the Indemnified Party shall have mutually agreed to the

retention of such counsel, (b) the named parties to any such proceeding (including any impleaded parties) include both the Indemnifying Party and the Indemnified Party and representation of both Parties by the same counsel would be inappropriate due to actual or potential differing interests between them. All such fees and expenses shall be reimbursed as they are incurred. The Indemnifying Party shall not be liable for any settlement of any proceeding effected without its written consent, but, if settled with such consent or if there be a final judgment for the plaintiff, the Indemnifying Party agrees to indemnify the Indemnified Party from and against any loss or liability by reason of such settlement or judgment. The Indemnifying Party shall not, without the written consent of the Indemnified Party, effect any settlement of any pending or threatened proceeding in respect of which the Indemnified Party is, or could have been, a party and indemnity could have been sought hereunder by the Indemnified Party, unless such settlement includes an unconditional release of the Indemnified Party from all liability on claims that are the subject matter of such proceeding.

10.5 Insurance.

(a) Insurance Obligations of AGTC. AGTC will maintain, at its cost, reasonable insurance against liability and other risks associated with its activities contemplated by this Agreement, provided that, if AGTC is engaged in any Development activities with respect to the Products hereunder, AGTC will maintain, in force from thirty (30) days prior to enrollment of the first subject in a Clinical Trial, a Clinical Trials/product liability insurance policy providing coverage of at least [***] per claim and [***] annually in the aggregate. AGTC will furnish to LICENSEE evidence of such insurance upon request.

(b) Insurance Obligations of LICENSEE. LICENSEE, together with its Affiliates, will maintain, at its cost, reasonable insurance against liability and other risks associated with its activities contemplated by this Agreement, provided that, at a minimum, LICENSEE will maintain, in force from thirty (30) days prior to enrollment of the first subject in a Clinical Trial, a Clinical Trials/product liability insurance policy providing coverage of at least [***] per claim and [***] annually in the aggregate, and provided, further, that such coverage is increased to at least [***] at least thirty (30) days before LICENSEE initiates the First Commercial Sale of a Product. LICENSEE will furnish to AGTC evidence of such insurance upon request. Notwithstanding the foregoing, so long as (i) substantially all of LICENSEE's equity securities remain publicly traded on a nationally recognized stock exchange and (ii) LICENSEE or any Affiliate of LICENSEE is researching, developing and commercializing Products under this Agreement, LICENSEE may self-insure against liability and other risks associated with its and its Affiliates' activities under this Agreement to the extent that it self-insures in respect of its other products, but at a minimum will self-insure at levels that are consistent with levels customarily maintained against similar risks by similar companies in LICENSEE's industry.

(c) Upon request of AGTC or an Existing Licensor, LICENSEE will furnish to AGTC or such licensor with a certificate of insurance of each product liability insurance policy obtained.

## ARTICLE XI
## LIMITATIONS OF LIABILITY

11.1 EXCEPT WITH RESPECT TO LIABILITY ARISING FROM A BREACH OF ARTICLE XII, FROM ANY WILLFUL MISCONDUCT OR INTENTIONALLY WRONGFUL ACT, OR TO THE EXTENT SUCH PARTY MAY BE REQUIRED TO INDEMNIFY THE OTHER PARTY UNDER ARTICLE X, IN NO EVENT WILL EITHER PARTY OR ANY OF ITS RESPECTIVE AFFILIATES, AGENTS OR REPRESENTATIVES BE LIABLE UNDER THIS AGREEMENT FOR ANY SPECIAL, INDIRECT, INCIDENTAL OR CONSEQUENTIAL DAMAGES, WHETHER IN CONTRACT, WARRANTY, TORT, NEGLIGENCE, STRICT LIABILITY OR OTHERWISE, INCLUDING LOSS OF PROFITS OR REVENUE SUFFERED BY EITHER PARTY OR ANY OF ITS RESPECTIVE AFFILIATES, AGENTS OR REPRESENTATIVES. WITHOUT LIMITING THE GENERALITY OF THE FOREGOING (A) "CONSEQUENTIAL DAMAGES" WILL BE DEEMED TO INCLUDE, AND NEITHER PARTY WILL BE LIABLE TO THE OTHER PARTY OR ANY OF THE OTHER PARTY'S AFFILIATES, AGENTS, REPRESENTATIVES OR STOCKHOLDERS FOR, ANY DAMAGES BASED ON OR MEASURED BY, ANY EVENT MILESTONE PAYMENT DUE UPON ANY UNACHIEVED EVENT MILESTONE UNDER SECTION 5.2, ANY SALES MILESTONE PAYMENT DUE UPON ANY UNACHIEVED ANNUAL NET SALES LEVEL UNDER SECTION 5.3, ANY UNEARNED ROYALTIES UNDER SECTION 5.4, OR ANY OTHER UNEARNED, SPECULATIVE OR OTHERWISE CONTINGENT PAYMENTS PROVIDED FOR IN THIS AGREEMENT AND (B) "CONSEQUENTIAL DAMAGES" WILL BE DEEMED TO INCLUDE, AND NEITHER PARTY WILL BE LIABLE TO THE OTHER PARTY OR ANY OF THE OTHER PARTY'S AFFILIATES OR REPRESENTATIVES FOR, ANY DAMAGES BASED ON OR MEASURED BY THE OTHER PARTY'S, ITS AFFILIATES' OR ITS SUBLICENSEES' LOSS OF PROJECTED OR SPECULATIVE FUTURE SALES OF THE PRODUCT(S).

11.2 AGTC'S AND EXISTING LICENSORS' AGGREGATE CUMULATIVE LIABILITY FOR ANY CLAIMS ARISING OUT OF OR RELATED TO THIS AGREEMENT, OTHER THAN INDEMNIFIABLE CLAIMS UNDER ARTICLE X, SHALL NOT EXCEED SEVEN (7) TIMES THE AMOUNT PAID BY LICENSEE TO AGTC HEREUNDER.

## ARTICLE XII
## CONFIDENTIALITY

12.1 Confidentiality. Except to the extent expressly authorized by this Agreement or otherwise agreed in writing, the Parties agree that, during the Term and for [***] years thereafter (or indefinitely with respect to trade secrets), each Party (the "Receiving Party") receiving any Confidential Information of the other Party (the "Disclosing Party") hereunder shall: (a) keep the Disclosing Party's Confidential Information confidential; (b) not publish, or allow to be published, and shall not otherwise disclose, or permit the disclosure of, the Disclosing Party's Confidential Information in any manner not expressly authorized pursuant to the terms of this Agreement; and (c) not use, or permit to be used, the Disclosing Party's Confidential Information for any purpose other than as expressly authorized pursuant to the terms of this Agreement. Each Party shall be responsible for unauthorized disclosures by its agents, directors, officers, employees, consultants,

Affiliates and advisors, and any other Third Party to whom such Party discloses such Confidential Information, regardless of whether such disclosure to such Third Party was permitted. For the avoidance of doubt, the [***] Manufacturing Technology shall be the Confidential Information of AGTC.

12.2 Authorized Disclosure. Notwithstanding the foregoing provisions of Section 12.1, each Party may disclose Confidential Information belonging to the other Party to the extent such disclosure is reasonably necessary to:

(a) file or prosecute patent applications or regulatory filings as contemplated by this Agreement;

(b) prosecute or defend litigation;

(c) exercise its rights and perform its obligations hereunder, provided that such disclosure is covered by terms of confidentiality at least as restrictive as those set forth herein;

(d) allow AGTC to comply with the terms and conditions of any agreements with Third Party licensors of the [***] Manufacturing Technology and LICENSEE to comply with the terms and conditions of any Third Party licensors of Technology required for the Product, provided that such disclosure is covered by terms of confidentiality at least as restrictive as those set forth herein or, with respect to [***] Manufacturing Technology licensed under an Existing License Agreement, those set forth in the applicable Existing License Agreement; and

(e) comply with applicable Law.

In the event a Party shall deem it reasonably necessary to disclose Confidential Information belonging to the other Party pursuant to this Section 12.2, the Disclosing Party shall to the extent possible give reasonable advance written notice of such disclosure to the other Party and take all reasonable measures to ensure confidential treatment of such information.

12.3 SEC Filings and Other Disclosures. Either Party may disclose the terms of this Agreement (a) to the extent required to comply with applicable Law, including the rules and regulations promulgated by the United States Securities and Exchange Commission or any equivalent governmental agency in any country in the Territory, (b) in connection with a prospective acquisition, merger or financing for such Party, to prospective acquirers or merger candidates or to existing or potential investors or financing sources and (c) to any sublicensee, collaborator or potential sublicensee or permitted collaborator of such Party, provided that, in the case of clause (b) or (c), prior to such disclosure each such candidate, investor or financing source shall agree in writing to be bound by obligations of confidentiality and non-use no less restrictive in scope than those set forth in this Article XII; and provided, further, that in the case of clause (a), such Party shall initially submit the redacted version of the Agreement agreed to by the Parties in writing within ten (10) days after the Execution Date with a request for confidential treatment of all of the redacted portions of such attached Agreement. With respect to any subsequent disclosure regarding this Agreement by a Party as required to comply with applicable Law, including the rules and regulations promulgated by the United States Securities and Exchange Commission or any equivalent governmental agency in any country in the Territory (including in response to

comments from the Securities and Exchange Commission regarding a request for confidential treatment), such Party shall provide a copy of the intended disclosure to the other Party prior to filing of such disclosure, and the other Party shall have five (5) Business Days (or in the case of a Current Report on Form 8-K, two (2) Business Days) prior to the filing thereof to review such disclosure and provide comments to such Party. Such Party shall implement all reasonable comments provided by the other Party within such period, it being understood that each Party is solely responsible for the accuracy and completeness of all SEC disclosures made by such Party.

12.4 Residual Knowledge Exception. Notwithstanding any provision of this Agreement to the contrary, and subject to the terms and conditions of any pre-existing exclusive license granted by either Party to one or more Third Parties, Confidential Information will not include Residual Knowledge. Any use made by the Receiving Party of Residual Knowledge is on an "as is, where is" basis, with all faults and all representations and warranties disclaimed and at its sole risk. Notwithstanding the foregoing, nothing in this Section 12.4 shall (a) affect the obligations of either Party with respect to confidentiality obligations of Confidential Information under Article XII; (b) constitute, or be deemed to result in, a license under any Technology or other intellectual property right; or (c) affect any other rights or remedies a Party may have under this Agreement or otherwise.

12.5 Restrictions on Material Non-Public Information. Each Party acknowledges that it is aware that the United States securities laws prohibit certain Persons who have received material, non-public information with respect to a public company from purchasing or selling securities of that public company and from communicating such information to any other Person under circumstances in which it is reasonably foreseeable that such Person is likely to purchase or sell such securities. Each Party acknowledges that it is familiar with the United States Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder (collectively, the "1934 Act"); and agrees that it will neither use, nor cause or permit any person to use, any Confidential Information in contravention of the 1934 Act, including Rule l0b-5 and Rule 14e-3 thereunder, or other applicable securities laws.

12.6 Public Announcements; Publications.

(a) Coordination. The Parties will, from time to time and at the request of the other Party, discuss the general information content relating to this Agreement that may be publicly disclosed.

(b) Announcements. Neither Party will make any public announcement regarding this Agreement without the prior written approval of the other Party. For the sake of clarity, nothing in this Agreement shall prevent LICENSEE from making any scientific publication or public announcement concerning LICENSEE's Development, Manufacture or Commercialization activities with respect to any Product under this Agreement; provided, however, that, except as permitted under Section 12.2, LICENSEE shall not disclose any of AGTC's Confidential Information in any such publication or announcement without obtaining AGTC's prior written consent to do so and consult with AGTC if such scientific publication or public announcement involves the [***] Manufacturing Technology.

(c) Use of Names. LICENSEE shall not and shall ensure that its Affiliates and Sublicensees shall not:

(i) use the name or insignia of [***] or the name of any [***] officers, faculty, other researches or students, or any adaptation of such names, in any advertising, promotional or sales literature, including any press release or any document employed to obtain funds, without the prior written approval of [***]; this restriction shall not apply to any information required by law to be disclosed to any governmental entity;

(ii) use the names of UFRF, or the University of Florida, nor of any of either institutions employees, agents or Affiliates, nor the name of any inventor of Patent Rights under any UFRF Existing License Agreement, nor any adaptation of such names, in any promotional, advertising or marketing materials or any similar form of publicity, or to suggest any endorsement by such entities or individuals, without the prior written approval of UFRF in each case;

(iii) use the name of The Johns Hopkins University or the Johns Hopkins Health System or any of its constituent parts, such as the Johns Hopkins Hospital or any contraction thereof or the name of inventors in any advertising, promotional, sales literature or fundraising documents without prior written consent from an authorized representative of JHU; LICENSEE, Affiliates and Sublicensees shall allow at least seven (7) Business Days' notice of any proposed public disclosure for JHU's review and comment or to provide written consent; and

(iv) use UABRF's name, the name of any inventor of Patent Rights governed by the UAB Agreement, or the name of UAB in any sales promotion, advertising or any other form of publicity without the prior approval of UABRF, except as required by Law; should LICENSEE be required by regulatory or legal requirements to disclose the existence of this Agreement, any of the terms in the UAB Agreement or the names of UABRF or UAB, UABRF shall have thirty (30) days to review (i) redaction of terms, including but not limited to royalty rates, and milestone or other payments, and (ii) the manner in which the names of UABRF or UAB are used.

12.7 Publications. During the Term, each Party shall submit to the other Party (the "Non-Disclosing Party") for review and approval any proposed public announcement, academic, scientific or medical publication or presentation related to the Joint [***] Manufacturing Improvement Technology or the LICENSEE [***] Manufacturing Improvement Technology. Such review and approval will be conducted for the purposes of preserving the value of the [***] Manufacturing Technology, the rights granted to each Party hereunder and determining whether any portion of the proposed publication or presentation containing the Non-Disclosing Party's Confidential Information should be modified or deleted. Written copies of such proposed publication or presentation required to be submitted hereunder shall be submitted to the Non-Disclosing Party no later than thirty (30) days before submission for publication or presentation. The Non-Disclosing Party shall provide its comments with respect to such publications and presentations within ten (10) Business Days of its receipt of such written copy. The review period may be extended for an additional sixty (60) days in the event the Non-Disclosing Party can demonstrate reasonable need for such extension including for the

preparation and filing of patent applications. Notwithstanding anything to the contrary, the Non-Disclosing Party may require that the other Party redact the Non-Disclosing Party's Confidential Information from any such proposed publication or presentation. AGTC and LICENSEE will each comply with standard academic practice regarding authorship of scientific publications and recognition of contribution of other parties in any publication. With respect to any public announcement, academic, scientific or medical publication or presentation during the Term related to the [***] Manufacturing Technology, subject to any Third Party confidentiality obligations and other obligations to Third Parties with respect to such publication or presentation, AGTC shall furnish LICENSEE with a written copy of such proposed publication or presentation no later than thirty (30) days before submission for publication or presentation, redacted, as appropriate for Third Party confidential information.

## ARTICLE XIII
## TERM AND TERMINATION

13.1 Term. The term of this Agreement shall begin on the Effective Date and, unless earlier terminated with respect to a Selected Gene or a Product in accordance with this Article XIII, shall continue on Product-by-Product and country-by-country basis until the expiration of the Royalty Term in such country for such Product (the "Term").

13.2 Termination by AGTC.

(a) In the event that LICENSEE commits a material breach of its obligations under this Agreement and such material breach remains uncured for [***] days (or in the case of non-payment that constitutes a material breach, [***] days), measured from the date written notice of such material breach is given to LICENSEE, AGTC may, in its sole discretion, terminate this Agreement either for cause in its entirety or on a Selected Gene-by-Selected Gene or Product-by-Product basis with respect to the Selected Genes or Products to which such material breach directly relates, in each case, in one or more countries in the Territory, at any time during the Term after such [***] day period (or [***] day period in the case of non-payment), by giving written notice to LICENSEE; provided, however, that if any breach other than non-payment is not reasonably curable within [***] days and if LICENSEE is making a bona fide effort to cure such breach, such termination shall be delayed for so long as LICENSEE is continuing to make such bona fide efforts to cure such breach. The cure period shall be tolled pending resolution of any bona fide dispute between the Parties as to whether any such material breach has occurred.

(b) Except to the extent the following under this Section 13.2(b) is unenforceable under the law of the applicable jurisdiction where the applicable Patent Right is pending or issued, in the event that LICENSEE or any of its Affiliates, individually or in association with any other person or entity, initiates or assists in initiating or continuing a determination that any Patent Right owned or Controlled by AGTC is invalid or unenforceable or otherwise limit the scope of any such Patent Right (a "LICENSEE Patent Challenge") through any administrative, judicial or other similar proceeding with respect to such Patent Right in a particular jurisdiction, AGTC may either, at its sole discretion (i) terminate LICENSEE's licenses under this Agreement with respect to such Product to which such Patent Right relates or in its entirety upon [***] days' prior written notice to LICENSEE, unless such LICENSEE Patent Challenge is

dropped within such [***] day period; or (ii) elect, in lieu of termination, to convert the licenses to LICENSEE under this Agreement to non-exclusive licenses, but for purposes of clarity, the financial obligations of LICENSEE contained herein shall continue in full force and effect. In any event, LICENSEE shall reimburse AGTC for all costs incurred by AGTC, its Affiliates or their respective sublicensees in connection with such LICENSEE Patent Challenge upon written notice to LICENSEE. LICENSEE will include the obligations set forth in this Section 13.2(b) in any sublicenses of its rights under this Agreement and shall use reasonable efforts to ensure its Sublicensees' compliance with such obligations, provided that AGTC shall have no termination right under this Section 13.2(b) in the event of any failure by such a Sublicensee to comply with such obligations, unless (a) LICENSEE has not included such provision in the applicable sublicense and (b) such Sublicensee individually or in association with any other person or entity, initiates or assists in initiating or continuing a determination that any Patent Right owned or Controlled by AGTC is invalid or unenforceable or otherwise limits the scope of any such Patent Right. AGTC will be a third party beneficiary of such provisions in any sublicense agreement solely for the purpose of enforcing its rights under such sublicense provisions directly.

13.3 Termination by LICENSEE.

(a) At any time upon at least [***] days' written notice to AGTC, LICENSEE may terminate this Agreement on a Selected Gene-by-Selected Gene or Product-by-Product basis without cause, for any or no reason, which termination shall be effective after the expiration of such [***] day period.

(b) In the event that AGTC commits a material breach of its obligations under this Agreement and such material breach remains uncured for [***] days (or in the case of non-payment that constitutes a material breach, [***] days), measured from the date written notice of such material breach is given to AGTC, LICENSEE may, in its sole discretion, terminate this Agreement either for cause in its entirety or on a Selected Gene-by-Selected Gene or Product-by-Product basis with respect to the Selected Genes or Products to which such material breach directly relates, in each case, in one or more countries in the Territory, at any time during the Term after such [***] day period (or the applicable [***] day period), by giving written notice to AGTC; provided, however, that if any breach other than non-payment is not reasonably curable within [***] days and if AGTC is making a bona fide effort to cure such breach, such termination shall be delayed for so long as AGTC is continuing to make such bona fide efforts to cure such breach. The cure period shall be tolled pending resolution of any bona fide dispute between the Parties as to whether any such material breach has occurred.

13.4 Termination for Insolvency. To the extent permissible under applicable Law, in the event that either Party makes an assignment for the benefit of creditors, appoints or suffers appointment of an administrator, receiver or trustee over all or substantially all of its property to which this Agreement relates, files a petition under any bankruptcy or insolvency act or has any such petition filed against it which is not dismissed within [***] days of the filing thereof (each, an "Insolvency Event" and the Party undergoing such Insolvency Event, the "Insolvent Party"), then the other Party may terminate this Agreement effective immediately upon written notice to Insolvent Party. In the event of a rejection of this Agreement by the Insolvent Party or any trustee thereof under Section 365 of the Bankruptcy Code:

(a) All rights and licenses now or hereafter granted by the Insolvent Party to the other Party under or pursuant to this Agreement, including, for the avoidance of doubt, the licenses granted under Section 3.1, are, for all purposes of Section 365(n) of the Bankruptcy Code, licenses of rights to "intellectual property" as defined in the Bankruptcy Code. Upon the rejection of this Agreement by the Insolvent Party or any trustee thereof, the Insolvent Party, for itself and any successors or assigns, including any trustee, agrees that the other Party, as licensee of such rights under this Agreement, shall retain and may fully exercise all of its rights and elections under the Bankruptcy Code. The Insolvent Party shall, during the term of this Agreement, create and maintain current copies of all intellectual property licensed under this Agreement. Each Party acknowledges and agrees that "embodiments" of such intellectual property within the meaning of Section 365(n) include, without limitation, laboratory notebooks, product samples and inventory, research studies and data, all Marketing Applications and Regulatory Approvals and rights of reference therein, of the [***] Manufacturing Technology on the one hand or the LICENSEE [***] Manufacturing Improvement Technology on the other hand, and in either case, the Joint Technology. If (i) a case under the Bankruptcy Code is commenced by or against the Insolvent Party, (ii) this Agreement is rejected as provided in the Bankruptcy Code, and (iii) the other Party elects to retain its rights hereunder as provided in Section 365(n) of the Bankruptcy Code, the Insolvent Party (in any capacity, including debtor-in-possession) and its successors and assigns (including a trustee) shall:

(i) provide to the other Party all such intellectual property (including all embodiments thereof) in the possession of the Insolvent Party on terms agreed by the Parties, promptly upon the other Party's written request.

(ii) not interfere with the non-insolvent Party's rights under this Agreement, or any agreement supplemental hereto, to such intellectual property (including such embodiments), including any right to obtain such intellectual property (or such embodiments) from another entity, to the extent provided in Section 365(n) of the Bankruptcy Code.

(b) All rights, powers and remedies of each Party provided herein are in addition to and not in substitution for any and all other rights, powers and remedies now or hereafter existing at law or in equity (including the Bankruptcy Code) in the event of the commencement of a case under the Bankruptcy Code with respect to the Insolvent Party. The Parties agree that they intend the following rights to extend to the maximum extent permitted by law, and to be enforceable under Bankruptcy Code Section 365(n) upon any rejection of this Agreement: the right of access on terms agreed by the Parties to any intellectual property (including all embodiments thereof) of the Insolvent Party which is necessary for the Manufacture, use, sale, import or export of Products.

13.5 Effects of Material Breach by AGTC in Lieu of Termination. Notwithstanding anything to the contrary, in the event of any material breach by AGTC of its obligations under this Agreement that remains uncured following the applicable cure period under Section 13.3(b), LICENSEE may elect, in lieu of terminating this Agreement in whole or in part as a result of such material breach, to reduce all further royalty and milestone payments payable by LICENSEE to AGTC under this Agreement, for a Product to which such material breach directly relates, by [***] of the amount otherwise payable to AGTC, after taking into account all applicable reductions set forth in Section

5.4, provided that in no event shall the royalties payable to AGTC under this Section 13.5 with respect to any Product in any Calendar Quarter be less than the sum of (A) the lesser of (a) the total royalty payments payable by AGTC to the Existing Licensors pursuant to the Existing License Agreements for such Product in such Calendar Quarter, and (b) [***] of Net Sales of such Product in such Calendar Quarter and (B) all payments under any AGTC Third Party Agreement that AGTC enters into during the Term and with respect to which LICENSEE has elected to take a sublicense under such AGTC Third Party Agreement pursuant to Section 8.7(b)(i). In the event of a termination by LICENSEE for AGTC's non-payment pursuant to Section 13.3(b), LICENSEE may credit the amount of the non-payment, together with interest that accrued pursuant to Section 5.10 from the first and any subsequent milestone or royalty payments due to AGTC under this Agreement until such amount is exhausted.

13.6 Termination of AGTC Third Party Agreements. In the event that any AGTC Third Party Agreement is terminated, so long as LICENSEE is not in default of any obligation under this Agreement, LICENSEE shall have rights to obtain a direct license to any AGTC Third Party Agreement to the extent expressly allowed and subject to the terms and conditions as expressly set forth in such AGTC Third Party Agreement.

13.7 Effect of Termination. Unless otherwise agreed in writing, no Party shall be released from any obligation accrued prior to termination or expiration of this Agreement. Upon termination or expiration of this Agreement in whole or with respect to a Selected Gene or a Product:

(a) LICENSEE shall (i) cease using all applicable terminated Products, Materials and [***] Manufacturing Technology and (ii) certify to AGTC within thirty (30) days after termination that LICENSEE has destroyed or returned to AGTC, at AGTC's sole election, all such items;

(b) any permitted sublicense of either Party shall, at the Sublicensee's option, survive such termination, provided that the Sublicensee is not in breach of any of its obligations under such sublicense and provided, further, that, in the case of a Sublicensee of LICENSEE, such Sublicensee has not initiated or assisted in the initiation or continuation of any LICENSEE Patent Challenge. In order to effect this provision, at the request of the Sublicensee, the licensor Party shall enter into a direct license with the Sublicensee on substantially the same terms as the sublicense, provided that the licensor Party shall not be required to undertake obligations in addition to those required by this Agreement, and that the licensor Party's rights under such direct license shall be consistent with its rights under this Agreement, taking into account the scope of the license granted under such direct license; and

(c) the licenses granted to LICENSEE in Section 3.1(b) with respect to such terminated Selected Gene or terminated Product shall terminate.

13.8 Survival. In addition to any other provisions expressly stated in this Agreement to survive expiration or termination of this Agreement, the following sections (and any other sections referenced therein for the corresponding time periods set forth therein) of this Agreement shall survive expiration or termination of this Agreement for any reason: Article I, Article XI, Article XII (for the time periods set forth therein), and Article XIV (other than Section 14.16), and

Sections 3.1(c), 3.1(d), 3.3, 3.4, 3.5(c), 3.6, 4.4, 5.6 (solely with respect to record-keeping for activities conducted under this Agreement prior to the effective date of termination), 5.7 (solely with respect to audits of records for activities conducted under this Agreement prior to the effective date of termination), 5.8, 5.9, 5.10, 8.1, 9.5, 9.7, 9.9, 9.10, 10.1, 10.2, 10.3, 10.4, 13.7 and 13.8.

## ARTICLE XIV
## MISCELLANEOUS

14.1 Assignment. Neither this Agreement nor any interest hereunder shall be assignable by either Party without the prior written consent of the other Party, except as follows: (a) either Party may, subject to the terms of this Agreement, assign its rights and obligations under this Agreement by way of sale of itself or the sale of the portion or substantially all of the portion of such Party's business to which this Agreement relates, through merger, sale of assets and/or sale of stock or ownership interest, provided that such sale is not primarily for the benefit of its creditors and (b) either Party may assign its rights and obligations under this Agreement to any of its Affiliates, provided that the assigning Party shall remain liable for all of its rights and obligations under this Agreement. The assigning Party shall promptly (and in any event within two (2) Business Days) notify the other Party of any assignment or transfer under the provisions of this Section 14.1. This Agreement shall be binding upon the successors and permitted assigns of the Parties and the name of a Party appearing herein shall be deemed to include the names of such Party's successors and permitted assigns to the extent necessary to carry out the intent of this Agreement. Any assignment not in accordance with this Section 14.1 shall be void. LICENSEE shall not assign this Agreement without the prior written consent of [***], except that LICENSEE may assign this Agreement to an Affiliate or a successor in connection with the merger, consolidation or sale of all or substantially all of its assets or that portion of its business to which this Agreement relates; provided, however, that any permitted assignee agrees in writing to be bound by the terms of this Agreement.

14.2 Force Majeure. Each Party shall be excused from the performance of its obligations under this Agreement to the extent that such performance is prevented by force majeure (defined below) and the nonperforming Party as promptly as practicable provides notice of the prevention to the other Party. Such excuse shall be continued so long as the condition constituting force majeure continues and the nonperforming Party takes Commercially Reasonable Efforts to remove the condition. For purposes of this Agreement, "force majeure" shall include conditions beyond the control of the Parties, including an act of God, voluntary or involuntary compliance with, or change in, any regulation, law or order of any government, omissions or delays in acting by any Regulatory Authority or other Governmental Authority or from the other Party, war, terrorism, civil commotion, riot, labor strike or lock-out, unavailability of raw materials, embargo, epidemic, failure or default of public utilities or common carriers, destruction of production facilities or materials by fire, flood, earthquake, storm or like catastrophe.

14.3 Correspondence and Notices.

(a) Ordinary Notices. Subject to the provisions of Section 14.3(b), correspondence, reports, documentation and any other communication in writing between the

Parties in the course of ordinary implementation of this Agreement shall be delivered by hand, sent by registered or certified mail (return receipt requested) postage prepaid or sent using a nationally recognized express courier service, in each case to the employee or representative of the other Party who is designated by such other Party to receive such written communication.

(b) Extraordinary Notices. Any notice or notification required or permitted to be provided pursuant to the terms and conditions of this Agreement (including, without limitation, any notice of force majeure, breach, termination, change of address, etc.) shall be in writing and shall be deemed given upon receipt if delivered personally or by facsimile transmission (receipt verified), five (5) days after deposited in the mail if mailed by registered or certified mail (return receipt requested) postage prepaid, or on the next Business Day if sent by overnight delivery using a nationally recognized express courier service and specifying next business day delivery (receipt verified), to the Parties at the following addresses or facsimile numbers (or at such other address or facsimile number for a Party as shall be specified by like notice; provided, however, that notices of a change of address shall be effective only upon receipt thereof):

All correspondence to LICENSEE shall be addressed as follows:

Biogen MA Inc.
225 Binney Street
Cambridge, Massachusetts 02142
Attn: General Counsel
Fax: (866) 546-2758

with a copy to:

Marc Rubenstein
Ropes & Gray LLP
Prudential Tower, 800 Boylston Street
Boston, MA 02199-3600
Telephone: 617-951-7826
Facsimile: 617-235-0706

All correspondence to AGTC shall be addressed as follows:

Applied Genetic Technologies Corporation
11801 Research Drive
Suite D
Alachua, Florida 32615
Attn: Larry Bullock, Chief Financial Officer

with a copy to:

Hemmie Chang

Foley Hoag LLP
Seaport West, 155 Seaport Boulevard
Boston, MA 02210-2600
Telephone: 617-832-1175
Facsimile: 617-832-7000

14.4 Amendment. No amendment, modification or supplement of any provision of this Agreement shall be valid or effective unless made in writing and signed by a duly authorized officer of each Party.

14.5 Waiver. No provision of this Agreement shall be waived by any act, omission or knowledge of a Party or its agents or employees except by an instrument in writing expressly waiving such provision and signed by a duly authorized officer of the waiving Party. The waiver by either of the Parties of any breach of any provision hereof by the other Party shall not be construed to be a waiver of any succeeding breach of such provision or a waiver of the provision itself.

14.6 Severability. If any clause or portion thereof in this Agreement is for any reason held to be invalid, illegal or unenforceable, the same shall not affect any other portion of this Agreement, as it is the intent of the Parties that this Agreement shall be construed in such fashion as to maintain its existence, validity and enforceability to the greatest extent permitted by law. In any such event, this Agreement shall be construed as if such clause of portion thereof had never been contained in this Agreement, and (after negotiation by the parties) there shall be deemed substituted therefor such provision as will most nearly carry out the intent of the Parties as expressed in this Agreement to the fullest extent permitted by applicable law.

14.7 Descriptive Headings. The descriptive headings of this Agreement are for convenience only and shall be of no force or effect in construing or interpreting any of the provisions of this Agreement.

14.8 Export Control. This Agreement is made subject to any restrictions concerning the export of products or technical information from the United States of America or other countries which may be imposed upon or related to AGTC or LICENSEE from time to time. Each Party agrees that it will not export, directly or indirectly, any technical information acquired from the other Party under this Agreement or any products using such technical information to a location or in a manner that at the time of export requires an export license or other governmental approval, without first obtaining the written consent to do so from the appropriate agency or other governmental entity. Specifically, each Party understands that the Arms Export Control Act (AECA), including its implementing International Traffic In Arms Regulations (ITAR) and the Export Administration Act (EAA), including its Export Administration Regulations (EAR), are some (but not all) of the laws and regulations that comprise the U.S. export laws and regulations. Each Party further understands that the U.S. export laws and regulations include (but are not limited to): (I) ITAR and EAR product/service/data-specific requirements; (2) ITAR and EAR ultimate destination-specific requirements; (3) ITAR and EAR end user-specific requirements; (4) ITAR and EAR end use-specific requirements; (5) Foreign Corrupt Practices Act; and (6) anti-boycott laws and

regulations. Each Party will comply with all then-current applicable export laws and regulations of the U.S. Government (and other applicable U.S. laws and regulations) pertaining to the patents and products licensed under the [***] Agreement (including any associated products, items, articles, computer software, media, services, technical data, and other information). Each Party certifies that it will not, directly or indirectly, export (including any deemed export), nor re-export (including any deemed re-export) such patents or products (including any associated products, items, articles, computer software, media, services, technical data, and other information) in violation of U.S. export laws and regulations or other applicable U.S. laws and regulations.

14.9 Governing Law. This Agreement, and all claims arising under or in connection therewith, shall be governed by and interpreted in accordance with the substantive laws of the State of Delaware, without regard to conflict of law principles thereof.

14.10 Entire Agreement. This Agreement, together with all related agreements referenced herein, constitutes and contains the complete, final and exclusive understanding and agreement of the Parties and cancels and supersedes any and all prior negotiations, correspondence, understandings and agreements, whether oral or written, between the Parties respecting the subject matter hereof and thereof, including that certain Mutual Confidentiality Agreement between the Parties dated May 27, 2014 which is hereby superseded and replaced in its entirety as of the Effective Date, and any Confidential Information disclosed by the Parties under such Mutual Confidentiality Agreement shall be treated in accordance with the provisions of Article XII.

14.11 Independent Contractors. Both Parties are independent contractors under this Agreement. Nothing herein contained shall be deemed to create an employment, agency, joint venture or partnership relationship between the Parties hereto or any of their agents or employees, or any other legal arrangement that would impose liability upon one Party for the act or failure to act of the other Party. Neither Party shall have any express or implied power to enter into any contracts or commitments or to incur any liabilities in the name of, or on behalf of, the other Party, or to bind the other Party in any respect whatsoever.

14.12 Counterparts. This Agreement may be executed in two (2) counterparts, each of which shall be an original and both of which shall constitute together the same document. Counterparts may be signed and delivered by facsimile, each of which shall be binding when received by the applicable Party.

14.13 Interpretation. Except where the context expressly requires otherwise, (a) the use of any gender herein shall be deemed to encompass references to either or both genders, and the use of the singular shall be deemed to include the plural (and vice versa), (b) the words “include”, “includes” and “including” shall be deemed to be followed by the phrase “without limitation,” (c) the word “will” shall be construed to have the same meaning and effect as the word “shall,” (d) any definition of or reference to any agreement, instrument or other document herein shall be construed as referring to such agreement, instrument or other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein), (e) any reference herein to any Person shall be construed to include the Person’s successors and assigns, (f) the words “herein”, “hereof” and “hereunder”, and words of similar import, shall be construed to refer to this Agreement in its

entirety and not to any particular provision hereof, (g) all references herein to Sections or Exhibits shall be construed to refer to Sections or Exhibits of this Agreement, and references to this Agreement include all Exhibits hereto, (h) the word "notice" shall mean notice in writing (whether or not specifically stated) and shall include notices, consents, approvals and other written communications contemplated under this Agreement, (i) provisions that require that a Party, the Parties or any committee hereunder "agree," "consent" or "approve" or the like shall require that such agreement, consent or approval be specific and in writing, whether by written agreement, letter, approved minutes or otherwise (but excluding e-mail and instant messaging), (j) references to any specific law, rule or regulation, or article, section or other division thereof, shall be deemed to include the then-current amendments thereto or any replacement or successor law, rule or regulation thereof, (k) any definition of or reference to any agreement, instrument or other document herein shall be construed as referring to such agreement, instrument or other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein), and (l) the term "or" shall be interpreted in the inclusive sense commonly associated with the term "and/or."

14.14 No Third Party Rights or Obligations. No provision of this Agreement shall be deemed or construed in any way to result in the creation of any rights or obligation in any Person not a Party to this Agreement, provided that each Person indemnified by either Party under Article X is an intended Third Party beneficiary for the sole purpose of enforcing such indemnification.

14.15 Remedies Cumulative. All rights and remedies of each Party under this Agreement will be cumulative and non-exclusive of any other rights or remedies available to such Party at law or in equity or provided for in this Agreement.

14.16 Further Actions. Each Party agrees to execute, acknowledge and deliver such further instruments, and to do all such other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

[*Signature page follows.*]

IN WITNESS WHEREOF, the Parties have executed this Agreement to be effective as of the Effective Date.

| **BIOGEN MA INC.** | | **APPLIED GENETIC TECHNOLOGIES CORPORATION** | |
|---|---|---|---|
| By | /s/ Douglas Williams | By | /s/ Susan B. Washer |
| Name: | Douglas Williams, Ph.D. | Name: | Susan B. Washer |
| Title: | Executive Vice President, Research and Development | Title: | President and CEO |

CONFIDENTIAL MATERIALS OMITTED AND FILED SEPARATELY WITH THE SECURITIES AND EXCHANGE COMMISSION. TRIPLE ASTERISKS [***] DENOTE OMISSIONS.

**SCHEDULE 1.42**

**EXISTING LICENSE AGREEMENTS**

UFRF/JHU Agreements

1. Standard Exclusive License Agreement With Sublicensing Terms (A3288), dated October 7, 2003, by and among AGTC, University of Florida Research Foundation, Inc. and Johns Hopkins University
    a. Amendment - November 2004 (First Amendment)
    b. Amendment - December 3, 2004 (Side Letter)
    c. Amendment - February 25, 2009 (Second Amendment)
    d. Amendment - March 30, 2010 (Third Amendment)
    e. Amendment - December 17, 2013 (Fourth Amendment)
    f. Amendment – July 1, 2015 (Omnibus Amendment)
2. [***] Agreement, dated March 13, 2014, by and among AGTC, University of Florida Board of Trustees and Johns Hopkins University
    a. Amendment – July 1, 2015 (Omnibus Amendment)

UAB Agreements

3. Non-Exclusive License Agreement with Sublicensing Terms [***], dated January 19, 2006, by and between AGTC and UAB Research Foundation .
    a. Amendment - March 28, 2014 (First Amendment)
    b. Amendment -June 29, 2015 (Second Amendment)
    c. Side Letter -June 29, 2015 (Request Letter)

[***] Agreements

[***]

[***] Agreements

[***]

## SCHEDULE 1.64

## [***] MANUFACTURING PATENT RIGHTS

"[***] Manufacturing Patent Rights" has the meaning set forth in Section 1.64 and, as of the Execution Date, consists of the following Patent Rights:

(i) AGTC Owned Patents

[***]

(ii) Co-Owned Patent Rights

[***]

(iii) In-licensed Patents

[***]

## SCHEDULE 1.119

## SELECTED GENES

[*To be included upon selection.*]

**SCHEDULE 3.2**

**SUBLICENSING RESTRICTIONS**

1. [***] Agreement

With respect to [***] Manufacturing Technology sublicensed to LICENSEE pursuant to the [***] Agreement, LICENSEE may grant further sublicenses to such [***] Manufacturing Technology through itself or its Affiliates to Third Parties, provided that each sublicense agreement: (a) shall incorporate by reference the terms and conditions of the [***]Agreement as set forth in this Agreement, (b) shall be consistent with the terms, conditions and limitations of the [***] Agreement, (c) shall name [***] and [***] as intended third party beneficiaries with respect to the indemnification obligations of the Sublicensee, (d) shall include a prohibition from further sublicensing the rights delivered thereunder, and (e) shall comply with the applicable provisions of Section 3.5(d) of this Agreement. LICENSEE agrees to provide a copy of each executed sublicense agreement to AGTC for delivery to [***] and [***] (which copy may be redacted for LICENSEE's, its Affiliate's or any Sublicensee's confidential information, for information regarding intellectual property that is unrelated to the [***] Manufacturing Patent Rights licensed under the [***] Agreement or other confidential information not necessary for [***] and [***] to ensure compliance with the [***] Agreement). Notwithstanding anything to the contrary, LICENSEE and any Sublicensee shall be free, without notice or consent, to engage distributors or to sublicense to contractors or collaborators for the purpose of manufacturing, research, development or any other purpose other than granting sublicense rights to commercialize or sell Products to Third Parties, provided that the provisions of this paragraph in this Schedule 3.2 shall be incorporated into each such sublicense agreement.

**SCHEDULE 4.4**

**THIRD PARTY MATERIALS**

(a)

[***] Biological Materials:

[***]

Other [***] Materials:

[***]

(b)

[***] Materials:

[***]

(c)

UF/JHU Materials:

[***]

## SCHEDULE 4.4(C)

## [***] RESTRICTIONS

[***]

**SCHEDULE 9.1**

**MUTUAL DISCLOSURE SCHEDULE**

(a) AGTC Disclosures

[***]

(b) LICENSEE Disclosures

[***]

**SCHEDULE 9.2**

**AGTC DISCLOSURE SCHEDULE**

[***]

**Exhibit 23.1**

**Consent of Independent Registered Public Accounting Firm**

We consent to the incorporation by reference in Registration Statement No. 333-198979 on Form S-8 and Registration No. 333-204064 on Form S-3 of Applied Genetic Technologies Corporation of our report dated September 10, 2018 relating to our audit of the financial statements and the financial statement schedule, which appear in this Annual Report on Form 10-K of Applied Genetic Technologies Corporation for the year ended June 30, 2018.

/s/ EY US LLP

Tampa, Florida
September 10, 2018

**Exhibit 23.2**

**Consent of Independent Registered Public Accounting Firm**

We consent to the incorporation by reference in Registration Statement No. 333-198979 on Form S-8 and Registration No. 333-225286 on Form S-3/A of Applied Genetic Technologies Corporation of our report dated September 13, 2017 relating to our audit of the financial statements and the financial statement schedule of Applied Genetic Technologies Corporation, appearing in this Annual Report on Form 10-K of Applied Genetic Technologies Corporation for the year ended June 30, 2018.

/s/ RSM US LLP

Raleigh, North Carolina
September 10, 2018

**Exhibit 31.1**

**CERTIFICATIONS**

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 10, 2018

By: /s/ Susan B. Washer
Susan B. Washer
Chief Executive Officer and President
(Principal Executive Officer)

**Exhibit 31.2**

**CERTIFICATIONS**

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 10, 2018

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, 2018 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 10, 2018

By: /s/ Susan B. Washer
Susan B. Washer
Chief Executive Officer and President (Principal Executive Officer)

Date: September 10, 2018

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.