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 December 31, 2018

Or

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

For the transition period from to

Commission file number 001-15771

ABEONA THERAPEUTICS INC.

(Exact name of registrant as specified in its charter)

Delaware	83-0221517
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
1330 Avenue of the Americas, 33rd Floor, New York, NY	10019
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: (646) 813-4712

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

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

Common Stock, $0.01 par value
Title of each Class
Nasdaq Capital Markets
Name of each exchange on which registered

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

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

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

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

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

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

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

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

The aggregate market value of the voting and non-voting common equity held by non-affiliates computed by reference to the average bid and asked price of such common equity, as of June 30, 2018, was approximately $445,125,000.

The number of shares outstanding of the registrant's common stock as of March 13, 2019 was 47,949,694 shares.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive Proxy Statement relating to our 2019 Annual Meeting of Stockholders are incorporated by reference into Part III of this Annual Report on Form 10-K where indicated. Such Proxy Statement will be filed with the Securities and Exchange Commission within 120 days after the end of the fiscal year to which this report relates.

TABLE OF CONTENTS

FORWARD-LOOKING STATEMENTS

This Form 10-K (including information incorporated by reference) contains statements that express our opinions, expectations, beliefs, plans, objectives, assumptions or projections regarding future events or future results and therefore are, or may be deemed to be, "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Words such as "expects," "anticipates," "intends," "plans," "believes," "could," "would," "seeks," "estimates," and variations of such words and similar expressions, and the negatives thereof, are intended to identify such forward-looking statements. We caution readers not to place undue reliance on any such "forward-looking statements," which speak only as of the date made, and advise readers that these forward-looking statements are not guarantees of future performance and involve certain risks, uncertainties, estimates, and assumptions by us that are difficult to predict. Various factors, some of which are beyond our control, could cause actual results to differ materially from those expressed in, or implied by, such forward-looking statements. All such forward-looking statements, whether written or oral, and whether made by us or on our behalf, are expressly qualified by these cautionary statements and any other cautionary statements that may accompany the forward-looking statements. In addition, we disclaim any obligation to update any forward-looking statements to reflect events or circumstances after the date of this report, except as may otherwise be required by the federal securities laws.

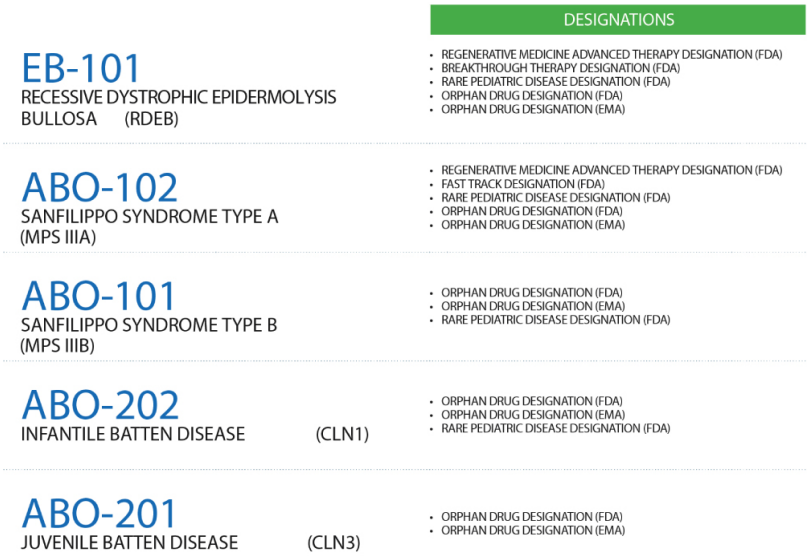
By their nature, these forward-looking statements involve risks and uncertainties. We have identified the following important factors that could cause actual results to differ materially from those discussed in our forward-looking statements. Such factors may be in addition to the risks described in Part I, Item 1A, Risk Factors; Part II, Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations; and other parts of this Form 10-K. These factors include: our estimates regarding expenses, future revenues, capital requirements, and needs for additional financing; our ability to raise capital; our ability to fund our operating expenses and capital expenditure requirements for at least the next 12 months with our existing cash and cash equivalents; our expectation that we will continue to incur losses; our belief that we will expend substantial funds to conduct research and development programs; our belief in our future ability to achieve profitability at all or on a sustained basis; our expected cash burn rate; the dilutive effect that raising additional funds by selling additional equity securities would have on the relative equity ownership of our existing investors; our belief that we have a rich pipeline of products and product candidates; our belief in our ability to continue to develop our novel AAV-vector gene therapy platform technology to target cells related to neurologic disorders, cystic fibrosis and eye disorders in human subjects; our belief that EB-101 could potentially benefit patients with recessive dystrophic epidermolysis bullosa ("RDEB"); our ability to initiate a Phase III clinical trial for patients with RDEB and complete enrollment of patients into the trial; our belief that AAV treatment could potentially benefit patients with MPS IIIA and B; our ability to add clinical sites and identify additional patients for our Phase I/II clinical trial for patients with MPS IIIA and B; our ability to continue to secure and maintain regulatory designations for our product candidates; our ability to develop manufacturing capability compliant with current good manufacturing practices for our product candidates; our ability to manufacture gene therapy products and produce an adequate product supply to support clinical trials and potentially future commercialization; our ability to secure timely regulatory review related to our clinical program; our belief in the adequacy of the data from clinical trials in EB-101 and expansion cohort of our Phase I/II clinical trial in ABO-102 (AAV-SGSH) for MPS IIIA, together with the data generated in the program to date, to support regulatory approvals; our intellectual property position and our ability to obtain, maintain and enforce intellectual property protection and exclusivity for our proprietary assets; the rate and degree of market acceptance of our product candidates for any indication once approved; our estimates regarding the size of the potential markets for our product candidates, the strength of our commercialization strategies and our ability to serve and supply those markets; our ability to meet our obligations contained in license agreements to which we are party; and the terms of future licensing arrangements or collaborations.

PART I

ITEM 1. BUSINESS

Business

Abeona Therapeutics Inc. (together with our subsidiaries, "we," "our," "Abeona" or the "Company"), a Delaware corporation, is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Our lead programs include EB-101, an autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa ("RDEB"), ABO-102, an adeno-associated virus ("AAV")-based gene therapy for Sanfilippo syndrome type A ("MPS IIIA"), and ABO-101 an AAV-based gene therapy for Sanfilippo syndrome type B ("MPS IIIB"). We also are developing ABO-202 and ABO-201, which are AAV-based gene therapies for the CLN1 and CLN3 forms of Batten Disease, respectively, ABO-401 for the treatment of cystic fibrosis, and ABO-5OX for the treatment of retinal diseases. In addition, we are developing next generation AAV-based gene therapy though our novel AIM™ vector platform programs. Our product candidates are eligible for orphan drug designation, breakthrough therapy designation, or other expedited review processes in the U.S., Europe or Japan. We hold several U.S. and EU regulatory designations for product candidates as follows:



Our Mission and Strategy

Abeona is at the forefront of cell and gene therapy research and development. We are a fully-integrated company featuring therapies in clinical development, in-house manufacturing facilities, a robust pipeline, and scientific, clinical, and commercial leadership. We see our mission as working together to create, develop, manufacture and deliver cell and gene therapies for people impacted by serious diseases. We partner with leading academic researchers, patient advocacy organizations and caregivers to bring therapies that address the underlying cause of a broad spectrum of rare genetic diseases where no effective treatment options exist today.

Since our last fiscal year, we made significant progress toward fulfilling our goal of harnessing the promise of genetic medicine to transform the lives of people impacted by serious diseases and redefine the standard of care through gene and cell therapies. Our strategy to achieve this goal consists of:

Advancing our Clinical Cell and Gene Therapy Programs and Research and Development with a Focus on Rare and Orphan Diseases.

We have three programs in clinical development—EB-101, ABO-101 and ABO-102—and a pipeline of additional earlier stage programs. Our programs are focused on rare serious diseases. Through our cell and gene therapy expertise in research and development, we are positioned to rapidly

introduce meaningful therapeutics to transform the standard of care in devastating diseases and establish our leadership position in the field.

Applying Novel Next Generation AIM™ Vector Technology to Develop New In-Vivo Gene Therapies.

We are researching and developing the next generation of adeno-associated virus ("AAV") gene therapy using our novel capsids developed from the AIM™ Vector Technology Platform. We aim to continue to develop chimeric AAV capsids capable of improved tissue targeting for various indications and potentially evading immunity to wildtype AAV vectors.

Establishing Leadership Position in Commercial-Scale Gene and Cell-Therapy Manufacturing.

We established current Good Manufacturing Practice ("cGMP"), clinical-scale manufacturing capabilities for gene-corrected cell therapy and AAV-based gene therapies in our state-of-the-art Cleveland, OH facility. We believe that our platform provides us with distinct advantages, including flexibility, scale, reliability, and the potential for reduced development risk, cost, and faster times to market. We have focused on establishing internal Chemistry, Manufacturing and Controls ("CMC") capabilities that drive value for the organization through process development, assay development and manufacturing. We have also deployed robust quality systems governing all aspects of product lifecycle from preclinical through commercial stage.

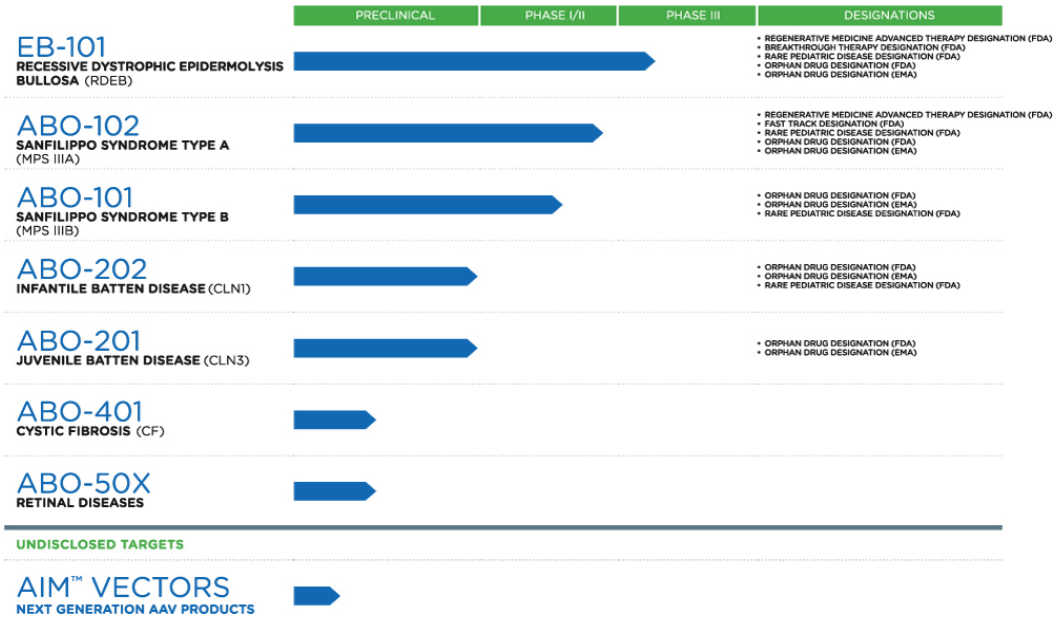
Establishing Additional Gene and Cell Therapy Franchises and Adjacencies through In-Licensing and Strategic Partnerships.

Abeona seeks to be the partner of choice in rare disease and has closely collaborated with leading academic institutions, key opinion leaders, patient foundations and industry partners to generate novel intellectual property, accelerate research and development, and understand the needs of patients and their families.

Maintaining and Growing IP Portfolio.

We strive to have a leading intellectual property portfolio. To that end, we seek patent rights for various aspects of our programs, including vector engineering and construct design, our production process, and all features of our clinical products including composition of matter and method of administration and delivery. We expect to continue to expand our intellectual property portfolio by aggressively seeking patent rights for promising aspects of our product engine and product candidates.

Our Pipeline



Our robust and diverse pipeline features early-stage and late-stage candidates with the potential to transform the treatment of devastating genetic diseases, and we are conducting clinical trials in the U.S. and abroad.

Our lead clinical programs include EB-101, an autologous, gene-corrected cell therapy for RDEB, ABO-102 (AAV-SGSH), an AAV-based gene therapy for MPS IIIA, and ABO-101 ("AAV NAGLU"), an AAV-based gene therapy forMPS IIIB. We are also developing ABO-201 ("AAV-CLN3") gene therapy for treatment of juvenile Batten disease ("CLN3"), ABO-202 ("AAV-CLN1") for treatment of infantile Batten disease ("CLN1"), ABO-401 for treatment of cystic fibrosis and ABO-50X for treatment of retinal diseases. In addition, we are developing next generation AAV-based gene therapy products utilizing a novel vector platform, AIM™, for additional disease areas.

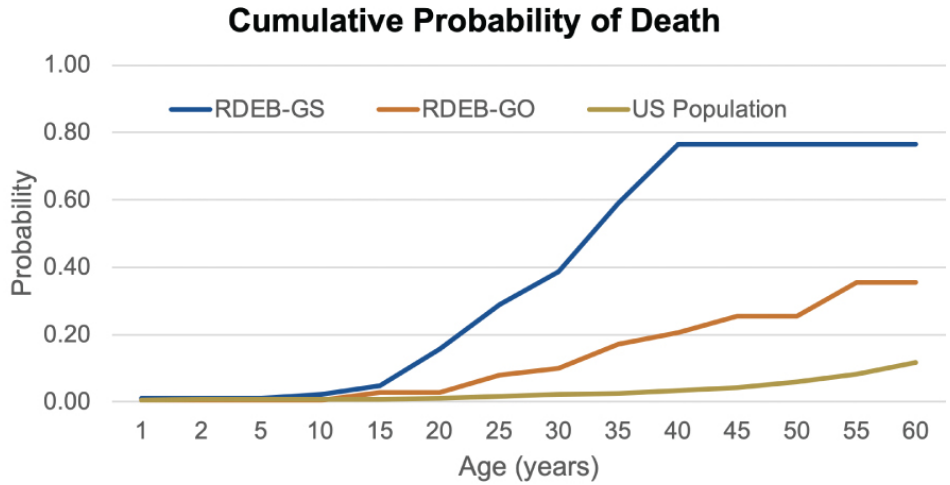
Developing Next Generation Cell and Gene Therapy

EB-101 for the Treatment of Recessive Dystrophic Epidermolysis Bullosa (RDEB)

Disease Overview

RDEB belongs to a group of genetic skin disorders known more broadly as epidermolysis bullosa ("EB"). Patients with RDEB have a defect in the COL7A1 gene resulting in the inability to produce Type VII collagen, which plays an important role in anchoring the dermal and epidermal layers of the skin. RDEB patients have extremely fragile skin resulting in severe, chronic, blistering, mutilating scarring of the hands and feet, joint contractures, strictures of the esophagus, a high risk of developing aggressive squamous cell carcinomas, infections, and risk premature death.

The two most common subtypes of RDEB are Recessive Dystrophic Epidermolysis Bullosa — Severe ("RDEB-GS"); and Recessive Dystrophic Epidermolysis Bullosa — Generalized Other ("RDEB-GO"). The two subtypes differ in the specific genetic mutation type which correlates with defects in Collagen VII formation and results in different phenotypes. Individuals with RDEB-GS produce little or no Collagen VII. They have generalized blistering from birth that results in extensive scarring, sparse hair, and blistering of the mucous membranes. Patients with RDEB-GO produce some functional, albeit abnormal, Collagen VII and therefore have more variable and generally less severe disease manifestation. Nevertheless, as described in natural history studies and in the NEBR registry (Fine, J., 2016), some patients with RDEB-GS and RDEB-GO have persistent blistering, severe systemic complications and are at a higher risk of premature death. As observed in the RDEB Registry, patients with RDER-GS and RDEB-GO have a twenty times and five times, respectively, greater probability of death at the age of 30 years than the general population.



Fine, J.D., and Registry, N.E.B. (1999). Epidermolysis Bullosa: Clinical, Epidemiologic, and Laboratory Advances, and the Findings of the National Epidermolysis Bullosa Registry.(Johns Hopkins University Press).

The incidence and prevalence of RDEB are not well defined. To date, the estimated incidence of 0.2-6.65 per million births and prevalence of 3.5-20.4 per million people have been primarily characterized by limited analyses of clinical databases or registries. Using genetic modeling of pathogenic variants of the COL7A1 gene, we estimate the incidence of RDEB to be 95 per million births in the US, of which 3% are RDEB-GS. Considering the relative mortality rates of RDEB-GS, RDEB-GO, and general population, we estimate a prevalence of RDEB from 700 to 4,000 patients in the US (most likely estimate of 2,500 patients), many of whom could benefit from a treatment such as EB-101. This estimate is similar to the number of RDEB patients registered with DEBRA, an advocacy group in the US devoted to the patient community with EB.

From a natural history study conducted by Stanford University in 2017 (Solis, D., et al., 2017), RDEB-GS and RDEB-GO patients have on average eight chronic and recurrent wounds of varying sizes per patient, with the majority of wounds being >20cm^2. Chronic wounds are defined as those that stay open for more than 12 weeks. Recurrent wounds partially heal but easily re-blister, with most wounds re-blistering within three weeks of healing. The larger wounds carry the highest burden, including pain, pruritis, and risk of infection.

Current Management of RDEB

At present, there are no approved treatments available for RDEB in the U.S or Europe. The management of RDEB currently consists of supportive wound care to limit contamination/infection and reduce mechanical forces that produce new blisters. Wound care usually includes treatment of new blisters by lancing and draining. Wounds are then dressed with a non-adherent material, covered with padding for stability and protection, and secured with an elastic wrap for integrity.

Individuals with RDEB have increased caloric and protein needs due to the increased energy utilized in wound healing, while oral intake is limited by oral and esophageal involvement. Infants and children with RDEB may require nutritional support, including a gastrostomy feeding tube. Anemia is typically treated with iron supplements and transfusions as needed.

We estimate that the annual cost of symptom supportive care for a RDEB patient is in the range of approximately $20,000 to $400,000 per year.

Surveillance is important for individuals with RDEB. Biopsies of abnormal-appearing wounds that do not heal may be recommended in some types of RDEB due to predisposition to squamous cell carcinoma. Screening for deficiencies of iron, zinc, vitamin D, selenium, and carnitine is typically recommended after the first year of life. Routine echocardiograms are recommended to identify dilated cardiomyopathy, and bone mineral density studies are recommended to identify osteoporosis. It is also typically recommended to avoid activities and bandages (including all adhesives) that may traumatize the skin.

Program Status

EB-101 is an autologous, gene-corrected cell therapy in which the normal COL7A1 gene is inserted into a patient's own skin cells (keratinocytes) and transplanted back to the patient to restore normal Type VII collagen expression and skin function. From preliminary clinical data and expert input, we expect EB-101 to be a potential treatment choice for most wounds, and currently the only product candidate being evaluated as a treatment for larger wounds. EB-101 has been granted both RMAT and Breakthrough designations, FDA and EU Orphan Drug designations and FDA Rare Pediatric Disease designation.

Results from a completed Phase I/II study (Phase I/II gene transfer for recessive dystrophic epidermolysis bullosa (NCT01263379)) that enrolled 7 patients with chronic RDEB wounds at Stanford University showed that EB-101 was well-tolerated and resulted in significant and durable wound healing (Siprashvili, Z., et al., 2016). The Phase I/II study showed significant and durable healing of large chronic wounds, with up to five years of follow-up. There have been no reported serious adverse events observed to date. Continuous Type VII collagen expression was observed for more than two years post treatment. There has been no detection of replication competent retrovirus (RCR) up to four years.

The Company expects to initiate a pivotal clinical trial evaluating the potential of EB-101 for the treatment of RDEB in the middle of 2019. The VITAL™ Study will be a multicenter, randomized, Phase III clinical trial assessing 10-15 patients treated with EB-101. The primary endpoint of the study will

be the proportion of EB-101 treated wounds with >50% healing at three months, compared with untreated wound site on the same patient. Secondary endpoints will include patient reported outcomes such as pain and itch and investigator global assessment of wounds.

ABO-102 and ABO-101 for the treatment of Mucopolysaccharidosis (MPS) III (Sanfilippo syndrome)

Disease Overview

MPS III (Sanfilippo syndrome) is a group of four inherited lysosomal storage diseases, described as type A, B, C or D, which cause enzyme deficiencies that result in the abnormal accumulation of glycosaminoglycans (sugars) in body tissues. The incidence of MPS III (all four types combined) is estimated to be 1 in 70,000 births.

Mucopolysaccharides are long chains of sugar molecules used in the building of connective tissues in the body. Lysosomes are responsible for a continuous process of replacing used materials and breaking them down for disposal. Children with MPS IIIA are missing a lysosomal enzyme that is essential in breaking down used mucopolysaccharides, specifically heparan sulfate ("HS"). The partially broken down heparan sulfate remains stored in cells in the body causing progressive lysosomal and cell damage and eventually cell death. Babies may show little sign of the disease, but as more cells become damaged, symptoms start to appear within the first few years of life.

In MPS III, the predominant glycosaminoglycan accumulation occurs in the brain and spinal cord, resulting in cognitive decline, motor dysfunction, and eventual death. Most patients with the rapidly progressing form of MPS III never reach a cognitive function above that of an unaffected 3-year-old child. Accumulation also occurs in other organs, leading to liver enlargement and soft tissue coarsening. To date, there is no cure for MPS III and treatments are largely supportive.

Program Status

Abeona is developing next-generation AAV-based gene therapies for MPS IIIA and MPS IIIB (Sanfilippo syndrome Type A and Sanfilippo syndrome Type B), ABO-102 and ABO-101, respectively, which involve a one-time intravenous administration that delivers a normal copy of the defective gene to cells of the central nervous system ("CNS") with the aim of reversing the effects of the genetic errors that cause the disease. Both viral constructs utilize the neurotropism of the serotype AAV9 to cross the blood brain barrier ("BBB") and deliver the functional copy of the gene to the CNS.

ABO-102 for MPS IIIA

Preclinical *in vivo* efficacy studies in animals with MPS IIIA showed that a single dose of ABO-102 significantly restored normal cell and organ function, corrected neurological deficits, increased neuromuscular control, and increased the lifespan by more than 100% one year after treatment compared with untreated control animals. These results are consistent with studies from several laboratories suggesting AAV treatment could potentially benefit patients with MPS III. In addition, safety studies conducted in animal models of MPS IIIA have demonstrated that delivery of ABO-102 is well tolerated with minimal side effects. ABO-102 was granted Fast Track and RMAT designations by the FDA, FDA and EU Orphan Drug designations and FDA Rare Pediatric Disease designation.

On December 6, 2018, we reported updated clinical data from the ongoing Phase I/II gene transfer clinical trial of scAAV9.U1a.hSGSH for Mucopolysaccharidosis IIIA (NCT02716246), our investigational gene therapy for the treatment of MPS IIIA. In the trial, subjects received a single intravenous injection of ABO-102 to facilitate systemic delivery, including to the CNS, of a corrective copy of the gene associated with onset and progression of MPS IIIA. Subjects were evaluated at multiple time points post-injection for safety assessments and signals of biopotency and clinical activity. The results showed a robust and durable reduction of heparan sulfate levels in the cerebrospinal fluid ("CSF"), denoting transgene expression in the CNS, and a durable reduction of liver volume. Effects were notable within 30 days post ABO-102 administration and remained stable with up to 2+ years of follow-up. No treatment related serious adverse events ("SAEs") have been reported to date.

Summary of MPS IIIA ABO-102 Phase I/II Study Data:

- 14 patients enrolled as of November 2018
- Clear dose-response and sustained reduction of heparan sulfate levels in CSF
- Sustained reduction in liver volume
- Encouraging neurocognitive signals seen in younger, higher functioning patients enrolled in cohort 3
- As of November 2018, follow up in cohort 1: (27-30 months); cohort 2: (19-21) months; and cohort 3: (1-16 months):
    - ABO-102 has been well tolerated to date
    - No serious drug related adverse events
    - ELISpot negative for the SGSH enzyme

We have amended the ongoing Phase I/II trial to enroll higher functioning patients who are likely to receive the most benefit from the treatment. We are planning to initiate a separate study of ABO-102 for patients with more advanced disease.

ABO-101 for MPSIIIB

In the ABO-101 program for MPS IIIB, subjects in our ongoing clinical study (Phase I/II gene transfer clinical trial of rAAV9.CMV.hNAGLU for Mucopolysaccharidosis (MPS) IIIB (NCT03315182)) receive a single, intravenous infusion of ABO-101, which uses an AAV vector to introduce the functional NAGLU gene to treat patients with MPS IIIB disease. Subjects will be evaluated at multiple time points post-injection for safety assessments and efficacy parameters. The clinical program is supported by a Natural History Study which included potential efficacy assessments consisting of neurocognitive evaluations, biochemical assays and MRI data generated over one year of follow-up assessments.

Preclinical *in vivo* efficacy studies in mice with MPS IIIB showed that a single dose of ABO-101 significantly restored normal cell and organ function, corrected neurological deficits, increased neuromuscular control, and normalized lifespan compared with untreated control animals. In addition, safety studies conducted in MPS IIIB and wildtype mice and in non-human primates have demonstrated that systemic delivery of ABO-101 is well tolerated with minimal side effects.

The first patient in the Phase I/II clinical trial for ABO-101 (“AAV-NAGLU”) was enrolled in 2017 at Nationwide Children’s Hospital (“NCH”) in Columbus, Ohio. Preliminary results observed in the initial U.S. trial enrollment to date demonstrate a clinically relevant biomarker response and a favorable safety profile. A second patient was enrolled in this study in January 2019, also at NCH.

In September 2018, we received authorization to move forward with a Phase I/II clinical trial in Spain. The clinical study was approved by the Agencia Espanola de Medicamentos y Productos Sanitarios and is being conducted at Hospital Clinico Universitario of Santiago de Compostela, Spain. This will be our second clinical trial conducted in Europe, alongside the ongoing Phase I/II clinical trial for patients with MPS IIIA (Sanfilippo syndrome type A). Abeona first initiated this trial in the United States. We have plans to activate additional clinical sites in the U.S., and abroad to facilitate patient enrollment.

Preclinical Programs

ABO-202 for the treatment of CLN1 disease, also known as infantile Batten disease (or Neuronal Ceroid Lipofuscinosis) (“NCL”)

Disease Overview

CLN1 disease (also known as infantile neuronal ceroid lipofuscinosis or Batten disease type 1) is a severe neurodegenerative lysosomal storage disease, currently with no approved treatment. It is caused by mutations in the CLN1 gene, encoding the soluble lysosomal enzyme palmitoyl-protein thioesterase-1 (“PPT1”).

In the classic form, rapidly progressive clinical features appear between the ages of 6 and 24 months, including speech and motor deterioration, refractory epilepsy, ataxia, myoclonus, and visual loss. By five years of age, CLN1 disease patients with the classic infantile form are typically poorly responsive and are no longer communicative. Death follows a few years after disease onset. Patients with low level of residual PPT1 activity develop a later onset form of CLN1 disease characterized by similar symptoms, but with slower progression. Patients with later onset of CLN1 generally succumb to the disease in the second decade.

There is no approved treatment for patients with CLN1 and the current care option is supportive and palliative. Gene therapy is proposed as a potential treatment for CLN1.

ABO-202 is designed to replace the faulty gene in affected cells and restore functionality of the protein. This therapy's viral construct, utilizing the AAV9 serotype, is able to cross the blood brain barrier ("BBB") to deliver the CLN1 gene to the CNS. The AAV9 trans-BBB neurotropism is advantageous because of involvement of the CNS in disease progression. This potential therapy is designed to allow the transformed cells to properly express the functional protein, and target PPT1 protein to the correct site of action, which in this case is the lysosomal matrix. The corrected enzyme can be secreted by transduced CNS cells and be taken up by neighboring cells via mannose 6-phosphate-mediated endocytosis and trafficked to the lysosome, cross-correcting substrate storing cells. This enables a potential therapeutic effect that goes beyond the initial transduction efficiency of the drug product. This in vivo gene therapy offers the possibility of a one-time treatment by inserting a healthy copy of the CLN1 gene and allowing the body to start making the missing enzyme, therefore slowing or halting CLN1 disease progression.

Program Status

On February 12, 2018, we announced that the FDA granted Orphan Drug Designation ("ODD") to our ABO-202 program. We have also secured an exclusive, worldwide license for AAV9 for treatment of CLN1. This therapeutic approach is a scAAV9 vector with codon-optimized CLN1 transgene ("AAV-CLN1"), an AAV-based gene therapy for the treatment of infantile Batten disease.

An investigational new drug ("IND") application is expected to be filed in this first quarter of 2019, with the clinical study initiation expected later in 2019.

The preclinical data for ABO-202 were presented at our Research & Development Day on December 6, 2018 and a slightly updated version of the presentation was delivered at the WORLD Congress of Lysosomal Storage Diseases in Orlando, FL on February 6, 2019. Key findings included:

- CLN1 mice recapitulate the major features of the human disease manifestations;
- The study data showed that a single intrathecal ("IT") injection of self-complementary adeno-associated virus 9 (scAAV9) encoding the human CLN1 gene administered to CLN1 mice at 1 week and 1 month (pre-symptomatic) and 12 weeks significantly increased their survival, improved behavior and reduced motor deficits; higher IT doses further improved longevity and function, suggesting that methods increasing CNS exposure may be beneficial and provided some survival and behavioral benefit to symptomatic CLN1 mice;
- A combination delivery approach administering ABO-202 by both intravenous and intrathecal routes to symptomatic animals (at 20 weeks) increased survival efficacy by >50% over intrathecal alone, and thus indicate a potential for treatment of patients with more advanced disease manifestations; and
- Consistent with other AAV studies for lysosomal storage disease, early intervention (i.e., treatment at a younger age) yielded better results compared with animals treated later, which required higher doses for the same benefit.

ABO-201 for the treatment of CLN3 disease, also known as juvenile Batten disease (or Juvenile Neuronal Ceroid Lipofuscinoses) ("CLN3 Disease")

Disease Overview and Program Status

CLN3 disease is a rare, fatal, autosomal recessive (inherited) disorder of the nervous system that typically begins between 4 and 8 years of age. Often the first noticeable sign of CLN3 disease is vision

impairment, which tends to progress rapidly and eventually result in blindness. As the disease progresses, children experience loss of previously acquired skills (developmental regression). This regression usually begins with the loss of the ability to speak in complete sentences. Children then lose motor skills, such as the ability to walk or sit. They also develop movement abnormalities that include rigidity or stiffness, slow or diminished movements (hypokinesia), and stooped posture. Beginning in mid- to late childhood, affected children may have recurrent seizures (epilepsy), heart problems, behavioral problems, and difficulty sleeping. Normal life expectancy is greatly reduced. Most people with juvenile Batten disease live into their twenties or thirties. As of December 31, 2018, no specific treatment is known that can halt or reverse the symptoms of CLN3 disease.

CLN3 disease is the most common form of a group of disorders known as neuronal ceroid lipofuscinosis ("NCLs"). Collectively, all forms of NCL affect an estimated 2 to 4 in 100,000 live births in the United States. NCLs are more common in Finland, where approximately 1 in 12,500 individuals are affected, as well as Sweden, other parts of northern Europe, and Newfoundland, Canada.

CLN3 disease is the most common form of Batten disease. Mutations associated with CLN3 disrupt the function of cellular structures called lysosomes. Lysosomes are compartments in the cell that normally digest and recycle different types of molecules. Lysosome malfunction leads to a buildup of fatty substances called lipopigments and proteins within these cell structures. These accumulations occur in cells throughout the body, but neurons in the brain seem to be particularly vulnerable to damage. The progressive functional impairment and eventual death of cells, especially in the brain, leads to vision loss, seizures, and intellectual decline in children with CLN3 disease.

ABO-201 (scAAV9.CLN3) is being developed as an AAV9-based gene therapy that has shown preclinical efficacy following delivery of a normal copy of the CLN3 gene to a mouse model of CLN3 disease. Preclinical studies have previously demonstrated reduced lysosomal storage and decreased astrocyte/microglia activation in the CNS as well as improved motor function.

Next-Generation Gene Therapy Treatments anchored in AIM™ Vector Platform

In 2016, we licensed a library of first-generation novel AAV capsids from the University of North Carolina. In partnership with academic institutions, our scientific research teams have identified vectors within the AIM™ capsid library showing strong potential to successfully target and reach the central nervous system, lung, skin, muscle, liver and other tissues. Based on continuing research being conducted by Abeona and our research partners, we see demonstrated improvements in gene delivery to specific tissues compared to currently available AAV technology. Viral vectors, which have shown increased tissue tropisms with potential for delivering treatment of cystic fibrosis and eye disorders, are supporting Abeona product programs in cystic fibrosis and retinal disorders. AIM™ vectors also have the potential for redosing previously treated AAV subjects in various disease states.

ABO 401 for the Treatment of Cystic Fibrosis

Disease Overview

Cystic Fibrosis ("CF") is a progressive, genetic disorder caused by a mutation in the cystic fibrosis transmembrane conductance regulator ("CFTR") gene. Malfunction of this gene affects cells that produce mucus, sweat and digestive juices. In unaffected individuals, these secreted fluids are normally thin and slippery, but in cystic fibrosis, a defective gene causes the secretions to become sticky and thick. Instead of acting as a lubricant, the secretions plug up tubes, ducts and passageways, especially in the lungs and pancreas, and cause repeated lung infections and difficulty breathing, and impaired pancreas function and digestive abnormalities.

Cystic fibrosis affects at least 30,000 people in the United States; between 900 and 1,000 new cases are diagnosed every year.

Program Status

The preclinical ABO-401 program has demonstrated vector delivery to the lungs of unaffected mice. Study results also demonstrate CFTR transgene expression that has corrected the underlying chloride current deficit in CF animals. Pre-clinical data suggest ABO-401, based on the AAV204 vector, efficiently

targets lung cells and that ABO-401 corrects the underlying cystic fibrosis chloride channel deficit, regardless of underlying mutations of the CF transmembrane conductance regulators, including the most common CF mutation, delta-F508.

ABO-50X for the treatment of genetic eye disorders

Program Overview

Eighty percent of genetic eye disorders occur in the photoreceptors and a correction of mutations in the retina has been accomplished by several groups using AAV gene therapy delivered through subretinal injection. Intravitreal delivery of small volume gene therapies constitutes an attractive alternative to deliver gene therapy to the retina in an out-patient setting.

Program Status

We reported non-human primate data suggesting that next-generation AIM™ AAV vectors can be used to efficiently target the retinal epithelium after intravitreal injection, creating the potential for new pipeline candidates that can address multiple eye disorders.

Also presented were data showing that certain AIM™ capsids demonstrated high tropisms for central nervous system tissue can evade neutralizing antibodies against naturally occurring AAV serotype, and potentially enable redosing in patients that have previously received an AAV injection.

Polymer Hydrogel Technology (PHT™)

MuGard® (mucoadhesive oral wound rinse) for mucositis, stomatitis, aphthous ulcers, and traumatic ulcers

MuGard is our legacy marketed product for the management of oral mucositis, a frequent side-effect of cancer therapy for which there is no other established treatment. MuGard, a proprietary nanopolymer formulation, received marketing clearance from the FDA in the U.S. as well as the applicable regulatory authorities in Europe, China, Australia, New Zealand and Korea. We launched MuGard in the U.S. in 2010 and licensed MuGard for commercialization in the U.S. to AMAG Pharmaceuticals, Inc. (AMAG) in 2013. We licensed MuGard to RHEI Pharmaceuticals, N.V. for China and other Southeast Asian countries in 2010; Hanmi Pharmaceutical Co. Ltd. for South Korea in 2014; and Norgine B.V. for the European Union, Switzerland, Norway, Iceland, Lichtenstein, Australia and New Zealand in 2014.

Establishing Leadership Position in Commercial-Scale Gene and Cell-Therapy Manufacturing

We have established a cGMP manufacturing facility to increase quality control, enhance supply chain control, increase supply capacity and manufacturing efficiency for clinical trials related to our product candidates and ensure commercial demand is met in the event our therapies receive marketing approval. Our facility is led by a team of highly-skilled production, process/assay development and QA/QC scientists with expertise in cell and gene therapy, particularly in cell culture, formulation, upstream, downstream and purification manufacturing. We have completed the first 6,000 square feet stage of our planned 26,000 square feet manufacturing build-out of a state-of-the-art process cGMP production facility for the manufacturing of cell and gene therapies at our Cleveland, Ohio location. The facility is designed to initially manufacture clinical drug product with later intent of manufacturing commercial grade cGMP drug product. The second stage of the build-out is underway and includes an 8,000 square feet state-of-the-art lab space to support our expanding quality control and process/assay development teams.

Additionally, we are working to advance our in-house manufacturing capabilities for our LZRSE COL7A1 retroviral autologous cell replacement therapy (EB-101) for the treatment of RDEB. The product is manufactured as a multilayer cellular sheet containing corrected keratinocytes that is fastened to a petrolatum gauze backing with surgical hemoclips. It is applied over wound areas, where they are expected to produce keratinocytes with normal Type VII collagen, providing wound coverage and allowing for wound healing.

We are developing manufacturing capabilities that use a chemical method we refer to as transfection for our AAV-based vector product candidates. We insert many copies of DNA plasmids encoding the specific therapeutic gene sequence, or transgene, into human embryonic kidney cells (HEK) 293 using adherent and suspension vector production technologies. During an incubation period following transfection, each cell produces vectors through biosynthesis using the natural machinery available within the cell. At the end of the incubation period, the newly generated vectors are collected from the cells that have been broken apart or, alternatively, from the cell culture medium. We continue to maintain focus on cGMP compliance and ensuring adequate supply to support our future clinical activity.

We have established and maintained strong and collaborative contract manufacturing relationships with third-party companies specializing in the manufacturing of gene therapy material to complement our process and assay development needs.

We have made significant investments in developing optimized manufacturing processes and believe that our processes and methods provide a comprehensive manufacturing process developed to date for EB-101 and AAV-based vector therapies, including:

- sufficient scale to support commercial manufacturing requirements for our product candidates;
- processes related to biopsy, cell collection, storage and transportation as part of manufacturing for EB-101;
- processes related to product release testing for EB-101;
- stable manufactured AAV vectors with sufficient longevity so that a small number of initial batches will likely provide adequate supply;
- proprietary AAV vector manufacturing processes and techniques that produce a highly purified product candidate;
- multiple assays to accurately characterize our process and the AAV vectors we produce;
- a series of purification processes, which may be adapted and customized for multiple different AAV capsids, with a goal of higher concentrations of active vectors, and that are essentially free of empty capsids; and
- establishing transportation and packaging processes and materials for finished EB-101 product.

We believe these improvements, and our continued investment in our manufacturing platform, will enable us to develop best-in-class, next-generation gene therapy products. We are working towards receiving FDA cGMP validation of our facility in Cleveland to produce commercial supply of EB-101.

Maintain a Strong Intellectual Property Portfolio

We strive to protect the proprietary technology, inventions, and know-how to enhance 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. We also rely on trade secrets and know-how relating to our proprietary technology platform, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our position in the field of gene therapy that may be important for the development of our business. We additionally may rely on regulatory protection afforded through data exclusivity, market exclusivity and patent term extensions where available.

Our success may depend in part on our ability to: obtain and maintain patent and other protections 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 intellectual property rights of third parties. Our ability to stop third parties from making, having made, using, selling, offering to sell or importing our products may depend on the extent to which we have rights under valid and enforceable licenses, patents or trade secrets that cover these activities. In some cases, these rights may need to be enforced by third-party licensors. 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 products and methods of manufacturing the same.

We are actively seeking U.S. and international patent protection for a variety of technologies, including the following: research tools and methods, methods for transferring genetic material into cells, AAV-based biological products, methods of designing novel AAV constructs, methods for treating diseases of interest and methods for manufacturing our products. We also intend to seek patent protection or rely upon trade secret rights to protect other technologies that may be used to discover and validate targets and that may be used to identify and develop novel biological products. We seek protection, in part, through confidentiality and proprietary information agreements. We are a party to various license agreements that give us rights to use specific technologies in our research and development, and future commercialization.

Licensed Technology and Intellectual Property

We have secured an exclusive license through Nationwide Children's Hospital to a family of patent applications for AAV treatments for patients with MPS III A and B. The family includes three pending applications in the United States. Patent(s) that may grant from this family is/are expected to expire approximately in 2031 and 2032.

We licensed the exclusive rights to an international patent family from the Nebraska Medical Center and the Ohio State Innovation Foundation. The family is directed to AAV gene therapy for the treatment of CLN3 disease. The patent family includes pending applications in the United States, Canada, Europe, China, Japan, New Zealand, and Australia. Patent(s) that may grant from this family is/are expected to expire approximately in 2035.

To support our EB franchise we licensed a patent family from Stanford University. The patent family covers technology for the treatment of RDEB. Patent applications are pending in the United States, Canada, Europe, Israel, Japan, South Korea, China, New Zealand, Australia, Russia, Mexico, South Africa, and Brazil. Patent(s) that may grant from this portfolio is/are expected to expire approximately in 2037.

We licensed an international patent family of novel AAV vectors for use across diseases from the University of North Carolina at Chapel Hill. This portfolio has been filed nationally in multiple jurisdictions including the United States, Australia, Brazil, China, Hong Kong, Europe, Canada, Israel, India, Japan, South Korea, Mexico, New Zealand, Russia, and South Africa. Patent(s) that may grant from this patent family is/are generally expected to expire approximately in 2035.

Also from the University of North Carolina at Chapel Hill, we have rights to a patent portfolio to support our CLN1 gene therapy program. This agreement provides an exclusive field of use license for the treatment of CLN1 disease. Patent applications are pending in the United States, Canada, Europe, Israel, India, China, Japan, South Korea, Australia, New Zealand, Mexico, Brazil, Russia, and South Africa. Patent(s) that may grant from this portfolio are expected to expire approximately in 2037.

On November 5, 2018, we announced a license agreement with REGENXBIO Inc. Under the terms of the agreement, REGENXBIO has granted Abeona an exclusive worldwide license (subject to certain non-exclusive rights previously granted for MPS IIIA), with rights to sublicense, to REGENXBIO's NAV AAV9 vector for the development and commercialization of gene therapies for the treatment of MPS IIIA, MPS IIIB, CLN1 Disease and CLN3 Disease. In return for these rights, REGENXBIO will receive a guaranteed $20 million upfront payment, $10 million of which was paid upon signing and $10 million of which will be paid within 12 months of the effective date. In addition, REGENXBIO will receive a total of $100 million in annual fees, payable upon the second through sixth anniversaries of the agreement, $20 million of which is guaranteed. REGENXBIO is also eligible to receive potential commercial milestone payments of up to $60 million. REGENXBIO will also receive low double-digit royalties on net sales of products incorporating the licensed intellectual property.

We continue to explore strategies to support patent grant extensions for all of our licensed portfolios.

U.S. Biologic Products Development Process

In the United States, the FDA regulates biologic products including gene therapy products under the Federal Food, Drug, and Cosmetic Act ("FDCA"), the Public Health Service Act ("PHSA"), and

regulations implementing these laws. The FDCA, PHSA and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, advertising and promotion of biologic products. Applications to the FDA are required before conducting human clinical testing of biologic products. Additionally, each clinical trial protocol for a gene therapy product candidate is reviewed by the FDA and, in limited instances NIH, through its Recombinant DNA Advisory Committee ("RAC"). FDA approval also must be obtained before marketing of biologic products.

Within the FDA, the Center for Biologics Evaluation and Research ("CBER") regulates gene therapy products. Within CBER, the review of gene therapy and related products is consolidated in the Office of Cellular, Tissue and Gene Therapies ("OCTGT") and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee ("CTGTAC"), a panel of medical and scientific experts and consumer representatives, to advise CBER on its reviews. CBER works closely with NIH and the RAC, which makes recommendations to 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 has issued a growing body of guidance documents on chemistry, manufacturing and control ("CMC"), clinical investigations and other areas of gene therapy development, all of which are intended to facilitate the industry's development of gene therapy products.

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

- completion of preclinical laboratory tests and in vivo studies in accordance with the FDA's current Good Laboratory Practice ("GLP") regulations and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an application for an Investigational New Drug exemption ("IND"), which allows human clinical trials to begin unless the FDA objects within 30 days;
- approval by an independent institutional review board ("IRB"), reviewing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials according to the FDA's Good Clinical Practice ("GCP") regulations, and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biologic product candidate for its intended use;
- preparation and submission to the FDA of a Biologics License Application ("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 biologic product candidate is produced to assess compliance with cGMP and to assure that the facilities, methods and controls are adequate to preserve the biologic product candidate's identity, safety, strength, quality, potency and purity;
- potential FDA audit of the nonclinical and clinical trial sites that generated the data in support of the BLA; and
- payment of user fees and the FDA review and approval, or licensure, of the BLA. BLA or new drug application ("NDA"), application fees for products designated as orphan drugs by the FDA are waived.

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

If a gene therapy trial is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, prior to the submission of an IND to the FDA, a protocol and related documents must be submitted to, and the study registered with, the NIH Office of Biotechnology Activities ("OBA"), pursuant to the NIH Guidelines for Research Involving Recombinant DNA Molecules ("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. NIH is responsible for convening the RAC that discusses protocols that raise novel or particularly important scientific, safety or ethical considerations at one of its quarterly public meetings. The OBA will notify the FDA of the RAC's decision regarding the necessity for full public review of a gene therapy protocol. RAC proceedings and reports are posted to the OBA website 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, unless the FDA places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. With gene therapy protocols, if the FDA allows the IND to proceed, but the RAC decides that full public review of the protocol is warranted, the FDA will request at the completion of its IND review that sponsors delay initiation of the protocol until after completion of the RAC review process. The FDA also may impose clinical holds on a biologic product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA.

Human clinical trials under an IND

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

Further, each clinical trial must be reviewed and approved by an IRB at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers items such as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject, or his or her legal representative, and must monitor the clinical trial until completed. Clinical trials involving recombinant DNA also must be reviewed by an institutional biosafety committee ("IBC"), a local institutional committee that reviews and oversees basic and clinical research that utilizes recombinant DNA at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

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

- Phase I: The biologic product candidate initially is introduced into healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early understanding of its effectiveness. In the case of some product candidates for severe or life-threatening diseases, especially when the product candidate may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.

- Phase II: The biologic product candidate is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product candidate for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.

- Phase III: The biologic product candidate is administered to an expanded patient population at geographically dispersed clinical trial sites in adequate and well-controlled clinical trials to generate sufficient data to statistically confirm the efficacy and safety of the product for approval. These clinical trials are intended to establish the overall risk/benefit ratio of the product candidate and provide an adequate basis for product labeling.

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

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, NIH and the investigators for serious and unexpected adverse events; any findings from other trials, in vivo laboratory tests or in vitro testing that suggest a significant risk for human subjects; or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information.

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

Additional regulation for gene therapy clinical trials

In addition to the regulations discussed above, there are a number of additional standards that apply to clinical trials involving the use of gene therapy. The FDA has issued various guidance documents regarding gene therapies, which outline additional factors that the FDA will consider at each of the above stages of development and relate to, among other things: the proper preclinical assessment of gene therapies; the CMC information that should be included in an IND application; the proper design of tests to measure product efficacy in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, the FDA usually recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by 10 years of annual queries, either in person or by questionnaire.

NIH and the FDA have a publicly accessible database, the Genetic Modification Clinical Research Information System, which includes information on gene therapy trials and serves as an electronic tool to facilitate the reporting and analysis of adverse events on these trials.

Compliance with cGMP requirements

Manufacturers of biologics must comply with applicable cGMP regulations. Manufacturers and others involved in the manufacture and distribution of such products also must register their establishments with the FDA and certain state agencies. Both domestic and foreign manufacturing establishments must register and provide additional information to the FDA upon their initial participation in the manufacturing process. Establishments may be subject to periodic, unannounced inspections by government authorities to ensure compliance with cGMP requirements and other laws. Discovery of problems may result in a

government entity placing restrictions on a product, manufacturer or holder of an approved BLA, and may extend to requiring withdrawal of the product from the market. The FDA will not approve an application unless it determines that the manufacturing processes and facilities comply with cGMP requirements and are adequate to assure consistent production of the product within required specification.

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

U.S. review and approval processes

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

For gene therapies, selecting patients with applicable genetic defects is a necessary condition to effective treatment. For the therapies we are currently developing, we believe that diagnoses based on symptoms, in conjunction with existing genetic tests developed and administered by laboratories certified under the Clinical Laboratory Improvement Amendments ("CLIA"), are sufficient to select appropriate patients and will be permitted by the FDA. For future therapies, however, it may be necessary to use FDA-cleared or FDA-approved diagnostic tests to select patients or to assure the safe and effective use of therapies in appropriate patients. The FDA refers to such tests as in vitro companion diagnostic devices. On July 31, 2014, the FDA announced the publication of a final guidance document describing the agency's current thinking about the development and regulation of in vitro companion diagnostic devices. The final guidance articulates a policy position that, when safe and effective use of a therapeutic product depends on a diagnostic device, the FDA generally will require approval or clearance of the diagnostic device at the same time that the FDA approves the therapeutic product. The final guidance allows for two exceptions to the general rule of concurrent drug/device approval, namely, when the therapeutic product is intended to treat serious and life-threatening conditions for which no alternative exists, and when a serious safety issue arises for an already approved therapeutic agent, and no FDA-cleared or FDA-approved companion diagnostic test is yet available. At this point, it is unclear how the FDA will apply this policy to our future gene therapy candidates, or even to our current products. Should the FDA deem genetic tests used for selecting appropriate patients for our therapies to be in vitro companion diagnostics requiring FDA clearance or approval, we may face significant delays or obstacles in obtaining approval for a BLA.

In addition, under the Pediatric Research Equity Act ("PREA"), a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the biologic 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 biologic product candidate for an indication for which orphan designation has been granted.

Under the Prescription Drug User Fee Act ("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, effective for fiscal year 2019, the user fee for an application requiring clinical data, such as a BLA, is $2,588,478. PDUFA also imposes an annual program fee of $309,915. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on BLAs for product candidates designated as orphan drugs, unless the product candidate also includes a non-orphan indication.

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

The FDA reviews the BLA to determine, among other things, whether the proposed product candidate is safe and potent, or effective, for its intended use, has an acceptable purity profile and whether the product candidate is being manufactured in accordance with cGMP to assure and preserve the product candidate's identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biologic products or biologic products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the product approval process, the FDA also will determine whether a risk evaluation and mitigation strategy ("REMS") is necessary to assure the safe use of the product candidate. A REMS could include medication guides, physician communication plans and elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

Before approving a BLA, the FDA will inspect the facilities at which the product candidate is manufactured. The FDA will not approve the product candidate unless it determines that the manufacturing processes and facilities comply with cGMP requirements and are adequate to assure consistent production of the product candidate within required specifications. Additionally, before approving a BLA, the FDA typically will inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND trial requirements and GCP requirements.

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

If a product candidate receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing or dispensing in the form of a REMS, or otherwise limit the scope of any approval. In addition, the FDA may require post-marketing clinical trials, sometimes referred to as Phase IV clinical trials, designed to further assess a biologic product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

The FDA has agreed to specified performance goals in the review of BLAs under the PDUFA. One such goal is to review 90% of standard BLAs in 10 months after the FDA accepts the BLA for filing, 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 designate a biologic product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing

and making a biologic product available in the United States for treatment of the disease or condition will be recovered from sales of the product). Orphan product designation must be requested before submitting a BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, meaning that the FDA may not approve any other applications to market the same drug or biologic product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity or if the party holding the exclusivity fails to assure the availability of sufficient quantities of the drug to meet the needs of patients with the disease or condition for which the drug was designated. Competitors, however, may receive approval of different products for the same 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 has exclusivity. Orphan medicinal product status in the European Union has similar, but not identical, benefits.

Expedited development and review programs

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

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

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

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

Finally, with passage of the 21st Century Cures Act (the "Cures Act") in December 2016, Congress authorized the FDA to accelerate review and approval of products designated as regenerative advanced therapies. A product is eligible for this designation if it is a regenerative medicine therapy (which may include a cell therapy) that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such disease or condition. The benefits of a regenerative advanced therapy designation include early interactions with the FDA to expedite development and review, benefits available to breakthrough therapies, potential eligibility for priority review and accelerated approval based on surrogate or intermediate endpoints.

Post-approval requirements

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

A sponsor also must comply with the FDA's advertising and promotion requirements, such as those 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"), 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. 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.

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 actions and adverse publicity. These actions could include refusal to approve pending applications or supplemental applications, withdrawal of an approval, clinical hold, suspension or termination of clinical trial by an IRB, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines or other monetary penalties, refusals of government contracts, mandated corrective advertising or communications to healthcare professionals or patients, debarment, restitution, disgorgement of profits or other civil or criminal penalties.

U.S. patent term restoration and marketing exclusivity

Depending upon the timing, duration and specifics of FDA approval of product candidates, some of a sponsor's U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period generally is one-half the time between the effective date of an IND and the submission date of a BLA plus the time

between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved biologic product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The United State Patent and Trademark Office ("USPTO"), in consultation with the FDA, reviews and approves the application for any patent term extension or restoration.

Pediatric exclusivity

Pediatric exclusivity is a type of non-patent marketing exclusivity in the United States that, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan exclusivity. This six-month exclusivity may be granted if a BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to, and accepted by, the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection that cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot accept or approve a biosimilar application.

Biosimilars and exclusivity

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act ("PPACA"), created an abbreviated approval pathway for biologic products shown to be similar to, or interchangeable with, an FDA-licensed reference biologic product, referred to as biosimilars. For the FDA to approve a biosimilar product, it must find that there are no clinically meaningful differences between the reference product and proposed biosimilar product. 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.

A reference biologic is granted 12 years of exclusivity from the time of first licensure of the reference product. An application for a biosimilar product may not be submitted to the FDA until four years following approval of the reference product, and it may not be approved until 12 years thereafter. These exclusivity provisions only apply to biosimilars — companies that rely on their own data and file a full BLA may be approved earlier than 12 years.

The 21st Century Cures Act

On December 13, 2016, President Obama signed the Cures Act into law. The Cures Act is designed to modernize and personalize healthcare, spur innovation and research and streamline the discovery and development of new therapies through increased federal funding of particular programs. It authorizes increasing funding for the FDA to spend on innovation projects. The new law also amends the Public Health Service Act to reauthorize and expand funding for the NIH. The Cures Act establishes the NIH Innovation Fund to pay for the cost of development and implementation of a strategic plan, early stage investigators and research. It also charges NIH with leading and coordinating expanded pediatric research. Further, the Cures Act directs the Centers for Disease Control and Prevention to expand surveillance of neurological diseases.

With amendments to the FDCA and the Public Health Service Act ("PHSA"), Title III of the Cures Act seeks to accelerate the discovery, development and delivery of new medicines and medical technologies. To that end, and among other provisions, the Cures Act reauthorizes for four years the priority review voucher program for certain drugs intended to treat rare pediatric diseases; creates a new priority review voucher program for drug applications determined to be material threat medical countermeasure applications; revises the FDCA to streamline review of combination product applications; requires the FDA to evaluate the potential use of "real world evidence" to help support approval of new indications for approved drugs; provides a new "limited population" approval pathway for antibiotic and antifungal drugs intended to treat serious or life-threatening infections; and authorizes the FDA to designate a drug as a "regenerative advanced therapy," thereby making it eligible for certain expedited review and approval designations.

Government regulation outside of the United States

In addition to regulations in the United States, sponsors are subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of biologic products. Because biologically-sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not a sponsor obtains FDA approval for a product, a sponsor must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application, much like the IND, prior to the commencement of human clinical trials. In the European Union, for example, a request for a Clinical Trial Authorization ("CTA") must be submitted to the competent regulatory authorities and the competent Ethics Committees in the European Union Member States in which the clinical trial takes place, much like the FDA and the IRB, respectively. Once the CTA request is approved in accordance with the European Union and the European Union Member State's requirements, clinical trial development may proceed.

The requirements and processes governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical trials are conducted in accordance with GCPs and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

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

European Union regulation and exclusivity

To obtain regulatory approval of an investigational biologic product under European Union regulatory systems, applicants must submit a marketing authorization application ("MAA"). The grant of marketing authorization in the European Union for products containing viable human tissues or cells such as gene therapy medicinal products is governed by Regulation 1394/2007/EC on advanced therapy medicinal products, read in combination with Directive 2001/83/EC of the European Parliament and of the Council, commonly known as the Community code on medicinal products. Regulation 1394/2007/EC lays down specific rules concerning the authorization, supervision and pharmacovigilance of gene therapy medicinal products, somatic cell therapy medicinal products and tissue engineered products. Manufacturers of advanced therapy medicinal products must demonstrate the quality, safety and efficacy of their products to the European Medicines Agency ("EMA") which provides an opinion regarding the application for marketing authorization. The European Commission grants or refuses marketing authorization in light of the opinion delivered by EMA.

Innovative medicinal products are authorized in the European Union based on a full marketing authorization application (as opposed to an application for marketing authorization that relies on data in the marketing authorization dossier for another, previously approved medicinal product). Applications for marketing authorization for innovative medicinal products must contain the results of pharmaceutical tests, preclinical tests and clinical trials conducted with the medicinal product for which marketing authorization is sought. Innovative medicinal products for which marketing authorization is granted are entitled to eight years of data exclusivity. During this period, applicants for approval of generics or biosimilars of these innovative products cannot rely on data contained in the marketing authorization dossier submitted for the innovative medicinal product to support their application. Innovative medicinal products for which marketing authorization is granted are also entitled to 10 years of market exclusivity. During these 10 years of market exclusivity, no generic or biosimilar medicinal product may be placed on the European Union market even if a generic or biosimilar marketing authorization can be submitted to the competent regulatory authorities in the European Union Member States. The overall 10-year period will be extended to a maximum of 11 years if, during the first eight years of those 10 years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be a new chemical entity and the innovator is able

to gain the period of data exclusivity, another company, nevertheless, could also market another competing medicinal product for the same therapeutic indication if such company obtained marketing authorization based on an MAA with a complete independent data package of pharmaceutical tests, preclinical tests and clinical trials.

Products receiving orphan designation in the European Union can receive 10 years of market exclusivity. During this 10-year period, the competent authorities of the European Union Member States and European Commission may not accept applications or grant marketing authorization for other similar medicinal product for the same orphan indication. There are, however, three exceptions to this principle. Marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

- The second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;
- The holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- The holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product.

An orphan product can also obtain an additional two years of market exclusivity in the European Union for the conduct of pediatric trials. The 10-year market exclusivity may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation; for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity.

The criteria for designating an "orphan medicinal product" in the European Union are similar, in principle, to those in the United States. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers. The application for orphan medicinal product designation must be submitted before the application for marketing authorization. Orphan medicinal product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

In April 2014, the EU adopted a new Clinical Trials Regulation (EU) No 536/2014 (the "Clinical Trials Regulation"), which is set to replace the current Clinical Trials Directive 2001/20/EC (the "Clinical Trials Directive"). The new Clinical Trials Regulation is still pending. Until the Clinical Trials Regulation becomes applicable, all clinical trials performed in the European Union are required to be conducted in accordance with the Clinical Trials Directive, which will be repealed on the day of entry into application of the Clinical Trial Regulation. It will however still apply three years from that day to (i) clinical trials applications submitted before the entry into application and (ii) clinical trials applications submitted within one year after the entry into application if the sponsor opted for the previous system. The Clinical Trial Regulation will overhaul the current system of approvals for clinical trials in the EU. Specifically, the legislation, which will be directly applicable in all member states, aims at simplifying and streamlining the approval of clinical trials in the EU. For instance, the legislation provides for a streamlined application procedure via a single-entry point and strictly defined deadlines for the assessment of clinical trial applications.

Other Healthcare Laws and Regulations

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and use of pharmaceutical products that are granted marketing approval. Arrangements with third-party payors, existing or potential customers and referral sources are subject to broadly applicable fraud and abuse and other healthcare laws and regulations, and these laws and regulations may constrain the business or financial arrangements and relationships through which manufacturers market, sell and distribute the products for which they obtain marketing approval. Such restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in cash or kind, in exchange for, or to induce, either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made

under federal healthcare programs such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers, on the one hand, and prescribers, purchasers and formulary managers on the other. The PPACA amended the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to commit a violation;

- the federal false claims and civil monetary penalties laws, including the civil False Claims Act (the "FCA"), which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid or other third-party payors that are false or fraudulent, or making a false statement to avoid, decrease, or conceal an obligation to pay money to the federal government. Certain marketing practices, including off-label promotion, also may implicate the FCA. In addition, the PPACA codified case law 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 FCA;

- the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services ("CMS"), information related to payments and other transfers of value to physicians, certain other healthcare providers and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members;

- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services; and

- state and foreign 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 pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict 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.

Violation of the laws described above or any other governmental laws and regulations may result in penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of operations, the exclusion from participation in federal and state healthcare programs, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, and imprisonment. Furthermore, efforts to ensure that business activities and business arrangements comply with applicable healthcare laws and regulations can be costly.

Data Privacy and Security

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and its implementing regulations, which imposes obligations, including mandatory contractual terms, with respect to safeguarding the transmission, security and privacy of protected health information; and

- the General European Data Protection Regulation, which became applicable May 25, 2018, harmonizes data privacy laws across Europe. This Regulation lays down rules relating to the protection with regard to the processing of personal data and rules relating to the transfer of personal data, as well as an individual's right to the protection of personal data, including medical information and clinical trial related data.

Coverage and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any products for which we may obtain regulatory approval. In the United States, sales of any product candidates for which regulatory approval for commercial sale is obtained will depend in part on the availability of coverage and adequate reimbursement from third-party payors. Third-party payors include government authorities and health programs in the United States such as Medicare and Medicaid, managed care providers, private health insurers and other organizations. These third-party payors are increasingly reducing reimbursements for medical products and services. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all FDA-approved drugs for a particular indication. Additionally, the containment of healthcare costs has become a priority of federal and state governments, and the prices of drugs have been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In the EU, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies. EU member states may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other member states allow companies to fix their own prices for products, but monitor and control company profits. The downward pressure on health care costs has become intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert competitive pressure that may reduce pricing within a country. Any country that has price controls or reimbursement limitations may not allow favorable reimbursement and pricing arrangements.

Health Reform

The United States and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality or expanding access. In the United States, for example, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected and continues to face major uncertainty due to the status of major legislative initiatives surrounding healthcare reform.

Additional Regulation

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservation and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern the use, handling and disposal of various biologic, chemical and radioactive substances used in, and wastes generated by, operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. Equivalent laws have been adopted in other countries that impose similar obligations.

U.S. Foreign Corrupt Practices Act

The U.S. Foreign Corrupt Practices Act ("FCPA"), prohibits U.S. corporations and individuals from engaging in certain activities to obtain or retain business abroad or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. The scope of the FCPA includes interactions with certain healthcare professionals in many countries. Equivalent laws have been adopted in other foreign countries that impose similar obligations.

Competition

Companies that are currently engaged in gene therapy or companies not yet focused on developing gene and cell therapies could at any time decide to develop therapies relevant to our business. Many of our competitors, either 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 product candidates and commercializing those product candidates. Accordingly, our competitors may be more successful than us in obtaining approval for product candidates and achieving widespread market acceptance. Our competitors' product candidates may be more effective, or more effectively marketed and sold, than any product candidate we may commercialize and may render our treatments obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our product candidates.

Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and subject registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We anticipate facing intense and increasing competition as new product candidates enter the market and advanced technologies become available. We expect any product candidates that we develop and commercialize to compete on the basis of, among other things, efficacy, safety, convenience of administration and delivery, price, and the availability of reimbursement from government and other third-party payors.

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 product candidates 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.

Corporate Information

Our principal executive office is located at 1330 Avenue of the Americas, 33 rd Floor, New York, NY 10019. Our telephone number in New York is (646) 813-4708. We also have manufacturing and laboratory facilities and administrative offices in Cleveland, Ohio and laboratory facilities in Madrid, Spain.

We were incorporated in Wyoming in 1974 as Chemex Corporation, and in 1983 we changed our name to Chemex Pharmaceuticals, Inc. We changed our state of incorporation from Wyoming to Delaware on June 30, 1989. In 1996 we merged with Access Pharmaceuticals, Inc., a private Texas corporation, and changed our name to Access Pharmaceuticals, Inc. On October 24, 2014 we changed our name to PlasmaTech Biopharmaceuticals, Inc. On May 15, 2015 we acquired Abeona Therapeutics LLC and on June 19, 2015 we changed our name to Abeona Therapeutics Inc.

Suppliers

Some materials used by us are specialized. We obtain materials from several suppliers based in different countries around the world. If materials are unavailable from one supplier, we generally have alternate suppliers available.

Employees

As of March 13, 2019, we had 83 full-time employees. We have never experienced employment-related work stoppages and consider that we maintain good relations with our personnel. In addition, to complement our internal expertise, we have contracts with scientific consultants, contract research organizations and university research laboratories that specialize in various aspects of drug development including clinical development, regulatory affairs, toxicology, process scale-up and preclinical testing.

Web Availability

We make available free of charge through our website, www.abeonatherapeutics.com, our annual reports on Form 10-K and other reports that we file with the Securities and Exchange Commission as well as certain of our corporate governance policies, including the charters for the audit, compensation and nominating and corporate governance committees of the Board of Directors (the "Board") and our code of ethics, corporate governance guidelines and whistleblower policy. We will also provide to any person without charge, upon request, a copy of any of the foregoing materials. Any such request must be made in writing to us at: Abeona Therapeutics Inc. c/o Investor Relations, 1330 Avenue of the Americas, 33rd Floor, New York, NY 10019. The SEC's website, www.sec.gov, contains reports, proxy statements, and other information that we file electronically with the SEC. The content on any website referred to in this Form 10-K is not incorporated by reference in this Form 10-K unless expressly noted.

ITEM 1A. RISK FACTORS

An investment in shares of our common stock involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this Annual Report on Form 10-K, including our financial statements and related notes hereto, before deciding to invest in our common stock. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.

Risks related to the discovery and development of our product candidates

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. Only a few gene therapy products have been approved in the U.S. and the EU.

We have concentrated our therapeutic product research and development efforts on our gene therapy platform, and our future success depends on the successful development of this therapeutic 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 delays in developing a sustainable, reproducible and commercial-scale manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical studies or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical study 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 the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Currently, only a few gene therapy products have been approved in the Western world, including Spark's gene therapy product, which received approval from the FDA in 2018. Additionally, GlaxoSmithKline's Strimvelis and Novartis's and Gilead's CAR-T therapies received approval from the FDA in 2017. Given the few precedents of approved gene therapy products, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the EU or other jurisdictions. Approvals by the EMA and the European Commission may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene and cell therapy products have evolved and may continue to change in the future. For example, the FDA has established the Office of Cellular, Tissue and Gene Therapies within 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 studies conducted at institutions that receive funding for recombinant DNA research from NIH are also subject to review by the NIH Office of Biotechnology Activities' RAC. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC review process can impede the initiation of a clinical study, even if the FDA has reviewed the study and approved its initiation. Clinical trial sites in the United States that receive NIH funding for research involving recombinant or synthetic nucleic acid molecules are required to follow RAC recommendations, or risk losing NIH funding for such research or needing NIH pre-approval before conducting such research. In addition, the FDA can put an investigational new drug application, or IND, on clinical hold if the information in an IND is not sufficient to assess the risks in pediatric patients. Before a clinical study can begin at any institution, that institution's institutional review board, or IRB, and its Institutional Biosafety Committee will have to review the proposed clinical study to assess the safety of the study. Moreover, serious adverse events or developments in clinical trials of gene therapy product candidates conducted by others may cause the FDA or other regulatory bodies to initiate a clinical hold on our clinical trials or otherwise change the requirements for approval of any of our product candidates.

These regulatory review agencies, committees and advisory groups and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or

larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval studies, 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 requirements and guidelines. If we fail to do so, we may be required to delay or discontinue development of our 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.

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

Identifying and qualifying patients to participate in clinical studies of our product candidates is critical to our success. The timing of our clinical studies depends on the speed at which we can recruit eligible patients to participate in testing our product candidates. We have experienced delays in some of our clinical studies, and we may experience similar delays in the future. 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 studies for similar patient populations, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of potential products may be delayed. These delays could result in increased costs, delays in advancing our product development, delays in testing the effectiveness of our technology or termination of the clinical studies altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a study, to complete our clinical studies in a timely manner. Patient enrollment is affected by factors including:

- severity of the disease under investigation;
- design of the study protocol;
- size of the patient population;
- eligibility criteria for the study in question;
- perceived risks and benefits of the product candidate under study, including as a result of adverse effects observed in similar or competing therapies;
- proximity and availability of clinical study sites for prospective patients;
- availability of competing therapies and clinical studies;
- efforts to facilitate timely enrollment in clinical studies;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

In particular, each of the conditions for which we plan to evaluate our current AAV product candidates are rare genetic disorders with limited patient pools from which to draw for clinical studies. Further, because newborn screening for MPSIIIA and B, other diseases we plan to address through gene and cell therapy (e.g., CLN1, retinal disease) is generally not performed, and it can be difficult to diagnose in the absence of a genetic screen, we may have difficulty finding patients who are eligible to participate in our study. The eligibility criteria of our clinical studies will further limit the pool of available study participants. Additionally, the process of finding and diagnosing patients may prove costly.

Enrollment into our upcoming Phase III VITAL™ study for EB-101 may encounter some challenges related to identification and enrollment of patients with RDEB. Whereas RDEB is a progressive condition diagnosed in early childhood, not all patients may qualify to participate in our study based on their ability to meet our inclusion criteria including related to overall medical condition, type of wounds (recurrent vs. chronic, location, size, etc.), presence of antibodies against Collagen VII, or restrictions on the ability to travel to study centers. The process for manufacturing EB-101 requires at least two biopsies from an area of

intact skin that must then be shipped to Abeona's manufacturing facility, posing possible risks of transportation, and ultimately viability of the specimens. The clinical trial will also require enrolled patients to travel to the clinical trial site for treatment and follow up. For individuals with RDEB, traveling can be challenging and pose health risks.

Our current product candidates are being developed to treat severe genetic diseases. We plan to seek initial marketing approval in the United States and the European Union. We may not be able to initiate or continue clinical studies if we cannot enroll a sufficient number of eligible patients to participate in the clinical studies required by the FDA or the EMA or other regulatory agencies. Our ability to successfully initiate, enroll and complete a clinical study in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with treatment centers, contract research organizations and physicians;
- different standards for the conduct of clinical studies;
- our inability to locate qualified local consultants, physicians and partners;
- 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 treatment; and
- Ability to transport product to the sites/maintain product viability in order to treat patients.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical studies as planned, we may need to delay, limit or terminate ongoing or planned clinical studies, any of which would have an adverse effect on our business.

We may encounter substantial delays in our clinical studies 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 studies to demonstrate the safety, purity and potency, or efficacy, of the product candidates in humans. Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical studies will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical studies can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory agencies on study design;
- delays in obtaining required IRB or Institutional Ethics Committee approval at each clinical study site; and
- delays in recruiting suitable patients to participate in our clinical studies.

Our product or product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or commercialization.

Undesirable side effects caused by our product or product candidates, including adverse events associated with our products, could interrupt, delay or halt clinical trials and could result in the denial of regulatory approval by the FDA, EMA or other regulatory authorities for any or all targeted indications, and in turn prevent us from commercializing our product or product candidates and generating revenues from their sale. In addition, if we or others identify undesirable side effects caused by our products or product candidates after receipt of marketing approval regulatory authorities may require the addition of restrictive labeling statements. Regulatory authorities may withdraw their approval of the product. We also may be required to change the way the product is administered or additional clinical trials are conducted. Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or product candidate or could substantially increase the costs and expenses of commercializing the product or product candidate, which in turn could delay or prevent us from generating significant revenues from its sale or adversely affect our reputation.

Even if we complete the necessary preclinical and clinical studies, 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 until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates demonstrate safety and efficacy in clinical studies, 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 advisory group or authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based on additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical studies and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our treatment candidates. For example, the development of our product candidates for pediatric use is an important part of our current business strategy, and if we are unable to obtain regulatory approval for the desired age ranges, our business may suffer.

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, regulatory authorities may still impose significant restrictions on the indicated uses or marketing of our product candidates or impose ongoing requirements for potentially costly post-approval studies, post-market surveillance or patient or drug restrictions. For example, the FDA typically advises that patients treated with gene therapy undergo follow-up observations for potential adverse events for a 15-year period. Additionally, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. 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 and adherence to commitments made in the BLA. If we or a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions relative to that product or the manufacturing facility, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

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 studies;
- refuse to approve a pending marketing application, such as a BLA or supplements to a BLA submitted by us;
- seize product; 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.

Risks related to Manufacturing

We could experience production problems in our manufacturing facilities that result in delays in our development or commercialization programs or otherwise adversely affect our business.

Gene therapies are novel, complex and difficult to manufacture. We commenced construction of our own manufacturing facility in 2018, and we may encounter difficulties in operating this facility. The manufacturing process we use to produce our product candidates including EB-101 is complex, novel and has not yet been validated for commercial use in the United States or in Europe.

Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, disruption in utility services, human error or disruptions in the operations of our suppliers. Our product and product candidates require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner.

There are several risks specific to the manufacturing process for EB-101 which require close attention. As an autologous product there are challenges associated with viability of biopsies as an incoming material. Due to the fragility of RDEB skin and site of the biopsy, initiation of autologous keratinocyte growth and expansion can be challenging or may be extended out of the scheduled timing. The other concern during manufacturing is slowing of cell proliferation rate resulting in extended manufacturing time. If pre-release criteria are not met the production process should be stopped for this batch of cells and a new batch will be initiated from the frozen culture or a new biopsy must be obtained. If release criteria are out of range, epidermal sheets should be discarded and repeated manufacturing must take place.

Accordingly, we employ multiple steps to control our manufacturing process to assure that the product or product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical grade materials that meet FDA, EU or other applicable standards or specifications with consistent and acceptable production yields and costs. In addition, FDA, EMA and other foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, FDA, EMA or other foreign regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls for approved and marketed products.

Lot failures or product recalls could cause us to delay product launches or clinical trials, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects. We also may encounter problems hiring and retaining the experienced specialist scientific, quality control and manufacturing personnel needed to operate our manufacturing process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements. Any problems in our manufacturing process or facilities could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in our manufacturing process including in internal and external facilities providing supply necessary for manufacturing, also could restrict our ability to meet clinical trial supply demand, and eventually market demand for any product candidates for which we may receive marketing approval. Disruptions in our manufacturing process may delay or disrupt our commercialization efforts.

In order to obtain regulatory approval for commercial manufacturing, we will need to continue to ensure that all of our processes, methods and equipment are compliant with cGMP and perform extensive audits of vendors, contract laboratories and suppliers. If any of our vendors, contract laboratories or suppliers is found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we work with these third parties to remedy the violation or while we work to identify suitable replacement vendors, contract laboratories or suppliers. The cGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with cGMP, we will be obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we would be subject to possible regulatory action and may not be permitted to sell any products that we may develop. We may rely on third parties to conduct aspects of our product manufacturing, and these third parties may not perform satisfactorily. We may rely on third parties for the production of certain materials for our product candidates and, therefore, we can control only certain aspects of their activities. We have manufacturing agreements with third parties that provide for, among other things, production of product candidates for our current and future early stage clinical trials. Under certain circumstances, the other party is entitled to terminate its arrangement with us. If we need to enter into alternative arrangements, it could delay our product development activities. Our reliance on third parties for certain manufacturing activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations. If a third party does not successfully carry out its contractual duties, meet expected deadlines or manufacture our product candidates in accordance with regulatory requirements, or if there are disagreements between us and any such third party, we will not be able to complete, or may be delayed in completing, the preclinical studies required to support future IND submissions and the clinical trials required for approval of our product candidates. In such instances, we may need to enter into an appropriate replacement third-party relationship, which may not be readily available or on acceptable terms, which would cause additional delay or increased expense prior to the approval of our product candidates and would thereby have a material adverse effect on our business, financial condition, results of operations and prospects. Our reliance on these third parties entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- reduced control for certain aspects of manufacturing activities;
- termination or nonrenewal of manufacturing and service agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers and service providers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or service provider.

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 or action of equivalent competent authorities in foreign jurisdictions, including injunction, recall, seizure or total or partial suspension of product manufacture. Failure to comply with ongoing regulatory requirements could cause us to suspend production or put in place costly or time-consuming remedial measures. The regulatory authorities may, at any time following approval of a product for sale, audit the manufacturing facilities for such product. If any such inspection or audit identifies a failure to comply with applicable regulations, or if a violation of product specifications or applicable regulations occurs independent of such an inspection or audit, the relevant regulatory authority may require remedial measures that may be costly or time-consuming 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 could materially harm our business, financial condition, results of operations and prospects. If we fail to comply with applicable cGMP regulations, FDA and foreign regulatory authorities can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new product candidate or suspension or revocation of a pre-existing approval. Such an occurrence may cause our business, financial condition, results of operations and prospects to be materially harmed. Additionally, if supply from our facility is interrupted, there could be a significant disruption in commercial supply of any of our product candidates for which we obtain marketing approval, and in clinical supply for our product candidates.

We currently do not have a backup manufacturer clinical trial supply for EB-101. An alternative manufacturer would need to be qualified, through regulatory filings, which could result in further delay. The regulatory authorities also may require additional clinical trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and could result in a delay in our desired clinical and commercial timelines.

If we, our collaborators, or any third-party manufacturers we engage fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We, our collaborators, and any third-party manufacturers we engage are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biologic and radioactive materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain general liability insurance and workers' compensation insurance for certain costs and expenses, we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biologic, hazardous or radioactive materials.

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

Risks related to our reliance on third-parties

We expect to rely on third parties to conduct some or all aspects of our viral vector production, drug product manufacturing, research and preclinical and clinical testing, and these third parties may not perform satisfactorily.

We do not expect to independently conduct all aspects of our viral vector production, drug product manufacturing and distribution, research and preclinical and clinical testing. We currently rely, and expect to continue to rely, on third parties with respect to these items. In some cases, these third parties are academic, research or similar institutions that may not apply the same quality control protocols utilized in certain commercial settings.

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 protocols. For example, for product candidates that we develop and commercialize on our own, we will remain responsible for ensuring that each of our IND-enabling studies and clinical studies are conducted in accordance with the study plan and protocols, and that our viral vectors and drug products are manufactured in accordance with GMP as applied in the relevant jurisdictions.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines, conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, or manufacture our viral vectors and drug products in accordance with GMP, we will not be able to complete, or may be delayed in completing, the preclinical and clinical studies and manufacturing process validation activities required to support future IND, MAA and BLA submissions and approval of our product candidates.

Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it could delay our product development activities.

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

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 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 associated with commercializing our product candidates

Our drug candidates are subject to the risks of failure inherent in the development of pharmaceutical products based on new technologies, and our failure to develop safe commercially viable drugs would severely limit our ability to become profitable or to achieve significant revenues.

We may be unable to successfully commercialize our product candidates if some or all of our product candidates are found to be unsafe or ineffective or otherwise fail to meet applicable regulatory standards or receive necessary regulatory clearances. Additionally, our product candidates may be deemed too difficult to develop into commercially viable drugs. We may encounter difficulty in manufacturing or marketing our product candidates on a large scale, and proprietary rights of third parties preclude us from marketing our drug candidates. Moreover, third parties may be able to market superior or equivalent drugs successfully. Failure to successfully commercialize our product candidates would have a material adverse effect on our business.

We may be unable to successfully develop, market, or commercialize our products or our product candidates without establishing new relationships and maintaining current relationships and our ability to successfully commercialize, and market our product candidates could be limited if a number of these existing relationships are terminated.

Our strategy for the research, development and commercialization of our potential pharmaceutical products may require us to enter into various arrangements with corporate and academic collaborators,

licensors, licensees and others, in addition to our existing relationships with other parties. Specifically, we may seek to joint venture, sublicense or enter other marketing arrangements with parties that have an established marketing capability or we may choose to pursue the commercialization of such products on our own. We may, however, be unable to establish such additional collaborative arrangements, license agreements, or marketing agreements as we may deem necessary to develop, commercialize and market our potential pharmaceutical products on acceptable terms. Furthermore, if we maintain and establish arrangements or relationships with third parties, our business may depend upon the successful performance by these third parties of their responsibilities under those arrangements and relationships.

We are subject to extensive governmental regulation, which increases our cost of doing business and may affect our ability to commercialize any new products that we may develop.

The FDA and comparable agencies in foreign countries impose substantial requirements upon the introduction of pharmaceutical products through lengthy and detailed laboratory, preclinical and clinical testing procedures and other costly and time-consuming procedures to establish safety and efficacy. All of our drugs and drug candidates require receipt and maintenance of governmental approvals for commercialization. Preclinical and clinical trials and manufacturing of our drug candidates will be subject to the rigorous testing and approval processes of the FDA and corresponding foreign regulatory authorities. Satisfaction of these requirements typically takes a significant number of years and can vary substantially based upon the type, complexity and novelty of the product.

Due to the time-consuming and uncertain nature of the drug candidate development process and the governmental approval process described above, we cannot assure you when we, independently or with our collaborative partners, might submit a NDA for FDA or other regulatory review. Further, our ability to commence and/or complete development projects will be subject to our ability to raise enough funds to pay for the development costs of these projects. Government regulation also affects the manufacturing and marketing of pharmaceutical products. Government regulations may delay marketing of our potential drugs for a considerable or indefinite period of time, impose costly procedural requirements upon our activities and furnish a competitive advantage to larger companies or companies more experienced in regulatory affairs. Delays in obtaining governmental regulatory approval could adversely affect our marketing as well as our ability to generate significant revenues from commercial sales.

Our drug candidates may not receive FDA or other regulatory approvals on a timely basis or at all. Moreover, if regulatory approval of a drug candidate is granted, such approval may impose limitations on the indicated use for which such drug may be marketed. Even if we obtain initial regulatory approvals for our drug candidates, our drugs and our manufacturing facilities would be subject to continual review and periodic inspection, and later discovery of previously unknown problems with a drug, manufacturer or facility may result in restrictions on the marketing or manufacture of such drug, including withdrawal of the drug from the market. The FDA and other regulatory authorities stringently apply regulatory standards and failure to comply with regulatory standards can, among other things, result in fines, denial or withdrawal of regulatory approvals, product recalls or seizures, operating restrictions and criminal prosecution.

We may incur substantial product liability expenses due to the use or misuse of our products for which we may be unable to obtain insurance coverage.

Our business exposes us to potential liability risks that are inherent in the testing, manufacturing and marketing of pharmaceutical products. These risks will expand with respect to our drug candidates, if any, that receive regulatory approval for commercial sale and we may face substantial liability for damages in the event of adverse side effects, including injury or death, or product defects identified with any of our products that are used in clinical tests or marketed to the public. Product liability insurance for the biotechnology industry is generally expensive, if available at all, and as a result, we may be unable to obtain insurance coverage at acceptable costs or in a sufficient amount in the future, if at all. We may be unable to satisfy any claims for which we may be held liable as a result of the use or misuse of products which we developed, manufactured or sold and any such product liability claim could adversely affect our business, operating results or financial condition.

Intense competition may limit our ability to successfully develop and market commercial products.

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. Our competitors in the U.S. and elsewhere are numerous and include,

among others, major multinational pharmaceutical and chemical companies, specialized biotechnology firms and universities and other research institutions. Many of our competitors have and employ greater financial and other resources, including larger research and development, marketing and manufacturing organizations. As a result, our competitors may successfully develop technologies and drugs that are more effective or less costly than any that we are developing or which would render our technology and future products obsolete and noncompetitive.

In addition, some of our competitors have greater experience than we do in conducting preclinical and clinical trials and obtaining FDA and other regulatory approvals. Accordingly, our competitors may succeed in obtaining FDA or other regulatory approvals for drug candidates more rapidly than we can. Companies that complete clinical trials, obtain required regulatory agency approvals and commence commercial sale of their drugs before their competitors may achieve a significant competitive advantage. Drugs resulting from our research and development efforts or from our joint efforts with collaborative partners therefore may not be commercially competitive with our competitors' existing products or products under development.

Our ability to successfully develop and commercialize our drug candidates will substantially depend upon the availability of reimbursement funds for the costs of the resulting drugs and related treatments.

Market acceptance and sales of our product candidates may depend on coverage and reimbursement policies and health care reform measures. Decisions about formulary coverage as well as levels at which government authorities and third-party payers, such as private health insurers and health maintenance organizations, reimburse patients for the price they pay for our products as well as levels at which these payors pay directly for our products, where applicable, could affect whether we are able to commercialize these products. We cannot be sure that reimbursement will be available for any of these products. Also, we cannot be sure that coverage or reimbursement amounts will not reduce the demand for, or the price of, our products. We have not commenced efforts to have our product candidates reimbursed by government or third-party payors. If coverage and reimbursement are not available or are available only at limited levels, we may not be able to commercialize our products. In recent years, officials have made numerous proposals to change the health care system in the U.S. These proposals include measures that would limit or prohibit payments for certain medical treatments or subject the pricing of drugs to government control. In addition, in many foreign countries, particularly the countries of the European Union, the pricing of prescription drugs is subject to government control. If our products are or become subject to government regulation that limits or prohibits payment for our products, or that subjects the price of our products to governmental control, we may not be able to generate revenue, attain profitability or commercialize our products.

As a result of legislative proposals and the trend towards managed health care in the U.S., third-party payors are increasingly attempting to contain health care costs by limiting both coverage and the level of reimbursement of new drugs. They may also impose strict prior authorization requirements and/or refuse to provide any coverage of uses of approved products for medical indications other than those for which the FDA has granted market approvals. As a result, significant uncertainty exists as to whether and how much third-party payors will reimburse patients for their use of newly-approved drugs, which in turn will put pressure on the pricing of drugs.

The market may not accept any pharmaceutical products that we develop.

The drugs that we are attempting to develop may compete with a number of well-established drugs manufactured and marketed by major pharmaceutical companies. The degree of market acceptance of any drugs developed by us will depend on a number of factors, including the establishment and demonstration of the clinical efficacy and safety of our drug candidates, the potential advantage of our drug candidates over existing therapies and the reimbursement policies of government and third-party payers. Physicians, patients or the medical community in general may not accept or use any drugs that we may develop independently or with our collaborative partners and if they do not, our business could suffer.

Healthcare reform measures could hinder or prevent our product candidates' commercial success.

The U.S. government and other governments have shown significant interest in pursuing healthcare reform. Any government-adopted reform measures could adversely impact the pricing of healthcare products and services in the U.S. or internationally and the amount of reimbursement available from

governmental agencies or other third-party payors. The continuing efforts of the U.S. and foreign governments, insurance companies, managed care organizations and other payors of health care services to contain or reduce health care costs may adversely affect our ability to set prices for our products which we believe are fair, and our ability to generate revenues and achieve and maintain profitability.

New laws, regulations and judicial decisions, or new interpretations of existing laws, regulations and decisions, that relate to healthcare availability, methods of delivery or payment for products and services, or sales, marketing or pricing, may limit our potential revenue, and we may need to revise our research and development programs. The pricing and reimbursement environment may change in the future and become more challenging due to several reasons, including policies advanced by the current executive administration in the U.S., new healthcare legislation or fiscal challenges faced by government health administration authorities. Specifically, in both the U.S. and some foreign jurisdictions, there have been a number of legislative and regulatory proposals to change the health care system in ways that could affect our ability to sell our products profitably.

For example, in March 2010, President Obama signed the PPACA. This law will substantially change the way healthcare is financed by both government health plans and private insurers, and significantly impact the pharmaceutical industry. The PPACA contains a number of provisions that are expected to impact our business and operations in ways that may negatively affect our potential revenues in the future. For example, the PPACA imposes a non-deductible excise tax on pharmaceutical manufacturers or importers that sell branded prescription drugs to U.S. government programs which we believe will increase the cost of our products. In addition, as part of the PPACA's provisions closing a funding gap that currently exists in the Medicare Part D prescription drug program (commonly known as the ''donut hole''), we will be required to provide a discount on branded prescription drugs equal to 50% of the government-negotiated price, for drugs provided to certain beneficiaries who fall within the donut hole. Similarly, PPACA increases the level of Medicaid rebates payable by manufacturers of brand-name drugs from 15.1% to 23.1% and requires collection of rebates for drugs paid by Medicaid managed care organizations. The PPACA also includes significant changes to the 340B drug discount program including expansion of the list of eligible covered entities that may purchase drugs under the program. At the same time, the expansion in eligibility for health insurance benefits created under PPACA is expected to increase the number of patients with insurance coverage who may receive our products. While it is too early to predict all the specific effects the PPACA or any future healthcare reform legislation will have on our business, they could have a material adverse effect on our business and financial condition.

Congress periodically adopts legislation like the PPACA and the Medicare Prescription Drug, Improvement and Modernization Act of 2003, that modifies Medicare reimbursement and coverage policies pertaining to prescription drugs. Implementation of these laws is subject to ongoing revision through regulatory and sub regulatory policies. Congress also may consider additional changes to Medicare policies, potentially including Medicare prescription drug policies, as part of ongoing budget negotiations. While the scope of any such legislation is uncertain at this time, there can be no assurances that future legislation or regulations will not decrease the coverage and price that we may receive for our proposed products. Other third-party payors are increasingly challenging the prices charged for medical products and services. It will be time consuming and expensive for us to go through the process of seeking coverage and reimbursement from Medicare and private payors. Our proposed products may not be considered cost-effective, and coverage and reimbursement may not be available or sufficient to allow us to sell our proposed products on a profitable basis. Further federal and state proposals and health care reforms are likely which could limit the prices that can be charged for the product candidates that we develop and may further limit our commercial opportunities. Our results of operations could be materially adversely affected by proposed healthcare reforms, by the Medicare prescription drug coverage legislation, by the possible effect of such current or future legislation on amounts that private insurers will pay and by other health care reforms that may be enacted or adopted in the future. In September 2007, the Food and Drug Administration Amendments Act of 2007 was enacted, giving the FDA enhanced post-marketing authority, including the authority to require post-marketing studies and clinical trials, labeling changes based on new safety information, and compliance with risk evaluations and mitigation strategies approved by the FDA. The FDA's exercise of this authority could result in delays or increased costs during product development, clinical trials and regulatory review, increased costs to assure compliance with post-approval regulatory requirements, and potential restrictions on the sale and/or distribution of approved products.

In December 2016, the Cures Act was signed into law. The Cures Act, among other things, is intended to modernize the regulation of drugs and spur innovation, but its ultimate implementation is unclear. 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.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the Trump administration may impact our business and industry. Namely, the Trump administration has taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. An under-staffed FDA could result in delays in the FDA's responsiveness or in its ability to review submissions or applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion or at all. Moreover, on January 30, 2017, President Trump issued an Executive Order, applicable to all executive agencies, including the FDA, which requires that for each notice of proposed rulemaking or final regulation to be issued in fiscal year 2017, the agency shall identify at least two existing regulations to be repealed, unless prohibited by law. These requirements are referred to as the "two-for-one" provisions. This Executive Order includes a budget neutrality provision that requires the total incremental cost of all new regulations in the 2017 fiscal year, including repealed regulations, to be no greater than zero, except in limited circumstances. For fiscal years 2018 and beyond, the Executive Order requires agencies to identify regulations to offset any incremental cost of a new regulation and approximate the total costs or savings associated with each new regulation or repealed regulation. In interim guidance issued by the Office of Information and Regulatory Affairs within OMB on February 2, 2017, the administration indicates that the "two-for-one" provisions may apply not only to agency regulations, but also to significant agency guidance documents. In addition, on February 24, 2017, President Trump issued an executive order directing each affected agency to designate an agency official as a "Regulatory Reform Officer" and establish a "Regulatory Reform Task Force" to implement the two-for-one provisions and other previously issued executive orders relating to the review of federal regulations, however it is difficult to predict how these requirements will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

We may be subject, directly or indirectly, to federal, state, and foreign healthcare laws and regulations, including 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.

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations will be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state laws and regulations, including, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal false claims act, and the Physician Payments Sunshine Act and regulations. These laws will impact, among other things, our proposed sales, marketing and educational programs. In addition, we may be subject to data privacy laws by both the federal government and the states in which we conduct our business. Such laws that may constrain the business or financial arrangements and relationships through which we conduct our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and 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, to induce or in return for either the referral of an individual for, or the purchase, recommendation, leasing or furnishing of, an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and

formulary managers on the other. Further, the Affordable Care Act amended the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it;

- the federal civil and criminal false claims laws and civil monetary penalty laws, including the civil False Claims Act, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent, or making a false statement to avoid, decrease, or conceal an obligation to pay money to the federal government. The Affordable Care Act provided and recent government cases against pharmaceutical and medical device manufacturers support the view that federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the civil False Claims Act;
- the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), which created additional federal criminal statutes that prohibit a person from knowingly and willfully executing or attempting to execute 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 ("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 Payments Sunshine Act, which is part of the Affordable Care Act, that requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to CMS information related to payments and other transfers of value provided to physicians and teaching hospitals, and ownership and investment interests held by physicians and their immediate family members, by the 90th day of each subsequent calendar year, and disclosure of such information is made by CMS on a publicly available website; and
- state and/or foreign law equivalents of each of the above federal laws, such as state anti-kickback and false claims laws that may apply to arrangements and claims involving health care items or services reimbursed by non-governmental third-party payors; 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; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; and state and foreign 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.

Our business could suffer if we lose the services of, or fail to attract, key personnel.

We are highly dependent upon the efforts of our senior management. The loss of the services of these individuals could delay or prevent the achievement of our research, development, marketing, or product commercialization objectives. We do not have employment contracts with our other key personnel. We do not maintain any 'key-man' insurance policies on any of our key employees and we do not intend to obtain such insurance. In addition, due to the specialized scientific nature of our business, we are highly dependent upon our ability to attract and retain qualified scientific and technical personnel and consultants. In view of the stage of our development and our research and development programs, we have restricted our hiring to research scientists, consultants and a small administrative staff and we have made only limited investments

in manufacturing, production, sales or regulatory compliance resources. There is intense competition among major pharmaceutical and chemical companies, specialized biotechnology firms and universities and other research institutions for qualified personnel in the areas of our activities, however, and we may be unsuccessful in attracting and retaining these personnel.

Trends toward managed health care and downward price pressures on medical products and services may limit our ability to profitably sell any drugs that we may develop.

Lower prices for pharmaceutical products may result from:

- third-party-payers' increasing challenges to the prices charged for medical products and services;
- the trend toward managed health care in the U.S. and the concurrent growth of HMOs and similar organizations that can control or significantly influence the purchase of healthcare services and products; and
- legislative proposals to reform healthcare or reduce government insurance programs.

The cost containment measures that healthcare providers are instituting, including practice protocols and guidelines and clinical pathways, and the effect of any healthcare reform, could limit our ability to profitably sell any drugs that we may successfully develop. Moreover, any future legislation or regulation, if any, relating to the healthcare industry or third-party coverage and reimbursement, may cause our business to suffer.

Security breaches and other disruptions could compromise our information and expose us to liability, which would cause our business and reputation to suffer.

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, our proprietary business information and that of our suppliers and business partners, as well as personally identifiable information of clinical trial participants and employees. Similarly, our business partners and third-party providers possess certain of our sensitive data. The secure maintenance of this information is critical to our operations and business strategy. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. Any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. If such an event were to occur and cause interruptions in our operations, it could result in a disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Although we maintain cyber risk insurance for certain costs, we may incur due to a cyber related event, this insurance may not provide adequate coverage against potential liabilities. Additionally, to the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed, and the further development and commercialization of our product candidates could be delayed.

Risks Related to Our Intellectual Property

Our rights to develop and commercialize our product candidates are subject to, in part, the terms and conditions of licenses granted to us by others.

We are reliant upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of our technology and products, including technology related to our manufacturing process and our product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories included in all of our licenses. These licenses may also require us to grant back certain rights to licensors and to pay certain amounts relating to sublicensing patent and other rights under the agreement.

In some circumstances, particularly in-licenses with academic institutions, we may 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, we cannot be certain that these patents and applications will be prosecuted, maintained and enforced in a manner consistent with the best interests of our business. If our licensors fail to maintain such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our products that are the subject of such licensed rights could be adversely affected. In certain circumstances, we have or may license technology from third parties on a non-exclusive basis. In such instances, other licensees may have the right to enforce our licensed patents in their respective fields, without our oversight or control. Those other licensees may choose to enforce our licensed patents in a way that harms our interest, for example, by advocating for claim interpretations or agreeing on invalidity positions that conflict with our positions or our interest. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we may own in the future.

Further, in many of our license agreements we are responsible for bringing any actions against any third party for infringing on the patents we have licensed. Certain of our license agreements also require us to meet development thresholds to maintain the license, including establishing a set timeline for developing and commercializing products and minimum yearly diligence obligations in developing and commercializing the product. 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 inventorship or ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of 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.

If we fail to comply with our obligations under these license 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 develop, manufacture, or market products covered by the license or may face other penalties under the agreements. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

Furthermore, the research resulting in certain of our licensed patent rights and technology was funded by the U.S. government. As a result, the government may have certain rights, or march-in rights, to such patent rights and technology. When new technologies are developed with U.S. government funding, the U.S. government generally obtains certain rights in any resulting patents, including a non-exclusive, royalty-free license authorizing the U.S. government, or a third party on its behalf, to use the invention for non-commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. The U.S. government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations or to give preference to U.S.

industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government, or a third party on its behalf, of such rights could harm our competitive position, business, financial condition, results of operations and prospects.

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

Our success depends, in large part, on our and our licensors' ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary product candidates and manufacturing technology. We and our licensors have sought and we intend to seek in the future, to protect our proprietary position by filing patent applications in the United States and abroad related to many of our novel technologies and product candidates that are important to our business.

The patent prosecution process is expensive, time-consuming and complex, and we may not have and may not in the future be able to file, prosecute, maintain, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. For example, in some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which may compromise our ability to obtain patent protection for certain inventions related to or building upon such prior work. Consequently, we will not be able to obtain any such patents to prevent others from using our technology for, and developing and marketing competing products to treat, these indications. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our and our licensors' patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates. In particular, during prosecution of any patent application, the issuance of any patents based on the application may depend upon our ability to generate additional preclinical or clinical data that support the patentability of our proposed claims. We may not be able to generate sufficient additional data on a timely basis, or at all. 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 and our licensors' patent protection.

We may not be aware of all third-party intellectual property rights potentially relating to our product candidates. Publications of discoveries in the scientific literature often lag 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 be certain that we were the first to make the inventions claimed in any owned or any licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. Databases for patents and publications, and methods for searching them, are inherently limited, so it is not practical to review and know the full scope of all issued and pending patent applications. As a result, the issuance, scope, validity, enforceability, and commercial value of our and our licensed patent rights are uncertain.

Even if the patent applications we license or may own in the future do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our 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 in patent claims being narrowed, invalidated or held

unenforceable, 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 product candidates. 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 intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could harm our business, financial condition, results of operations and prospects.

We may not be successful in obtaining necessary rights to our product candidates through acquisitions and in-licenses.

We currently have rights to certain intellectual property, through licenses from third parties, to develop our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire, in-license or use these proprietary rights. We may be unable to acquire or in-license any compositions, methods of use, processes or other intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign 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.

We sometimes collaborate with non-profit and 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 option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to develop our program.

If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may be required to expend significant time and resources to redesign our product candidates or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly.

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

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our licensed patents and/or applications and any patent rights we may own in the future. We rely on our outside counsel or our licensing partners to pay these fees due to non-U.S. patent agencies. The USPTO and various non-U.S. government patent agencies require compliance with

several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could harm our business.

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

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States could 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. For example, an April 2014 report from the Office of the United States Trade Representative identified a number of countries, including India and China, where challenges to the procurement and enforcement of patent rights have been reported. Several countries, including India and China, have been listed in the report every year since 1989. 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.

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court. We may not be able to protect our trade secrets in court.

If one of our licensing partners or we initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid 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, lack of written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may 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, *inter partes* review and equivalent proceedings in foreign jurisdictions. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover our product candidates. 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 the patent examiner and we or our licensing partners 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. Such a loss of patent protection could harm our business.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. Some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, collaborators, contractors, and other third-parties. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. 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.

Third-parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could harm our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may become party to, or threatened with, infringement litigation claims regarding our products and technology, including claims from competitors or from non-practicing entities that have no relevant product revenue and against whom our own patent portfolio may have no deterrent effect. Moreover, we may become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates and technology, including interference proceedings, post grant review and *inter partes* review before the USPTO or foreign patent offices. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could adversely affect our ability to commercialize our product candidates or any other of our product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. Similar challenges exist in other jurisdictions. If we are found to infringe a third-party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third-party to continue developing, manufacturing and marketing our product candidates and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could harm our business. In addition, we may be forced to redesign our product candidates, seek new regulatory approvals, and

indemnify third parties pursuant to contractual agreements. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, reputation, financial condition, results of operations and prospects.

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

Competitors may infringe our intellectual property rights or the intellectual property rights of our licensing partners, or we may be required to defend against claims of infringement. To counter infringement or unauthorized use claims or to defend against claims of infringement can be expensive and time consuming. 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 and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could adversely affect our ability to compete in the marketplace.

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

Many of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

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

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

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

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. On September 16, 2011, the Leahy-Smith America Invents Act (the "Leahy-Smith Act") was signed into law. The Leahy-Smith Act includes several significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted and also may affect patent litigation. These also include provisions that switched

the United States from a "first-to-invent" system to a "first-to-file" system, allow third-party submission of prior art to the USPTO during patent prosecution and set forth additional procedures to attack the validity of a patent by the USPTO administered post grant proceedings. Under a first-to-file system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to the patent on an invention regardless of whether another inventor had made the invention earlier. The USPTO 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 harm our business, financial condition, results of operations and prospects.

For example, the Leahy-Smith Act provides a new administrative tribunal known as the Patent Trial and Appeals Board ("PTAB"), that provides a venue for companies to challenge the validity of competitor patents at a cost that is much lower than district court litigation and on timelines that are much faster. Although it is not clear what, if any, long-term impacts the PTAB proceedings will have on the operation of our business, the initial results of patent challenge proceedings before the PTAB since its inception in 2013 have resulted in the invalidation of many U.S. patent claims. The availability of the PTAB as a lower-cost, faster and potentially more potent tribunal for challenging patents could therefore increase the likelihood that our own licensed patents will be challenged, thereby increasing the uncertainties and costs of maintaining and enforcing them. Moreover, if such challenges occur, we may not have the right to control the defense. In certain situations, we may be required to rely on our licensor to consider our suggestions and to defend such challenges, with the possibility that it may not do so in a way that best protects our interests.

We also may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in other contested proceedings such as 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 products.

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

On March 4, 2014, the USPTO issued a guidance memorandum to patent examiners titled 2014 Procedure for Subject Matter Eligibility Analysis of Claims Reciting or Involving Laws of Nature/Natural Principles, Natural Phenomena, and/or Natural Products. These guidelines and more recent guidelines

superseding them instruct USPTO examiners on the ramifications of the Prometheus and Myriad rulings and apply the Myriad ruling to natural products and principles including all naturally occurring nucleic acids. Patents for certain of our product candidates contain claims related to specific DNA sequences that are naturally occurring and, therefore, could be the subject of future challenges made by third parties. In addition, the recent USPTO guidance could make it impossible for us to pursue similar patent claims in patent applications we may prosecute in the future.

We cannot assure you that our efforts to seek patent protection for our technology and products will not be negatively impacted by the decisions described above, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what impact the Supreme Court's decisions in Prometheus and Myriad may have on the ability of life science companies to obtain or enforce patents relating to their products and technologies in the future. These decisions, the guidance issued by the USPTO and rulings in other cases or changes in USPTO guidance or procedures could have an adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

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

Outside the United States, other courts have also begun to address the patenting of genetic material. In August 2015, the Australian High Court ruled that isolated genes cannot be patented in Australia. The decision did not address methods of using genetic material. Any ruling of a similar scope in other countries could affect the scope of our intellectual property rights.

If we do not obtain patent term extension and data exclusivity for our product candidates, our business may be harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of our product candidates, one or more 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 (the "Hatch-Waxman Amendments"). The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended per FDA-approved product, and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. Further, certain of our licenses currently or in the future may not provide us with the right to control decisions the licensor or its other licensees on Orange Book listings or patent term extension decisions under the Hatch-Waxman Act. Thus, if one of our important licensed patents is eligible for a patent term extension under the Hatch-Waxman Act, and it covers a product of another licensee in addition to our own product candidate, we may not be able to obtain that extension if the other licensee seeks and obtains that extension first. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time-period or the scope of patent protection afforded during any such extension could be less than we request. If we are unable to obtain patent term extension or the scope of term of any such extension is less than we request, the period during which we will have the right to exclusively market our product may be shortened and our competitors may obtain approval of competing products following our patent expiration, and our revenue could be materially reduced.

Intellectual property rights do not necessarily address all potential threats.

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

- others may be able to make gene therapy products that are similar to our product candidates but that are not covered by the claims of the patents that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending patent applications or those that we may own in the future will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may have an adverse effect on our business; and
- we may choose not to file a patent for certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could significantly harm our business, financial condition, results of operations and prospects.

Risks Relating to our Financial Condition and Capital Requirements

We have experienced a history of losses, we expect to incur future losses and we may be unable to obtain necessary additional capital to fund operations in the future.

We have recorded minimal revenue to date and have incurred an accumulated deficit of approximately $410.2 million through December 31, 2018. The net loss for the year ended December 31, 2018 was $56.7 million. Our losses have resulted principally from costs incurred in research and development activities related to our efforts to develop clinical drug candidates, from losses due to derivatives and from the associated administrative costs.

We require substantial capital for our development programs and operating expenses, to pursue regulatory clearances and to prosecute and defend our intellectual property rights. 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 studies for our product candidates;
- further develop the manufacturing process for our vectors or our product candidates;
- change or add additional manufacturers or suppliers;
- seek regulatory and marketing approvals for our product candidates that successfully complete clinical studies;

- seek to identify and validate additional product candidates;
- acquire or in-license other product candidates and technologies;
- make milestone or other payments under any license agreements;
- maintain, protect and expand our intellectual property portfolio;
- establish a sales, marketing and distribution infrastructure in the United States and Europe to commercialize any products for which we may obtain marketing approval;
- attract and retain skilled personnel;
- build additional infrastructure to support our operations as a larger public company and our product development and planned future commercialization efforts, including manufacturing capacity; 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.

As of December 31, 2018, our cash, cash equivalents and short-term investments were $85.0 million. We expect that our existing cash, cash equivalents, and marketable securities will be sufficient to fund our business operations for the foreseeable future. However, our 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, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. In any event, we will require additional capital to obtain regulatory approval for, and to commercialize, our product candidates. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic objectives.

Any additional 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, we cannot guarantee that future financing will be available 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 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 and we may be required to relinquish 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, operating results and prospects.

If we are unable to obtain 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 and results of operations.

We do not have significant operating revenue and may never attain profitability.

To date, we have funded our operations primarily through public offerings of our common stock. Our ability to achieve significant revenue or profitability depends upon our ability to complete the development of our drug candidates, to develop and obtain patent protection and regulatory approvals for our drug

candidates and to manufacture and commercialize the resulting drugs. We are not expecting any significant revenues in the short-term from our products or product candidates. Furthermore, we may not be able to ever successfully identify, develop, commercialize, patent, manufacture, obtain required regulatory approvals and market any additional products. Moreover, even if we do identify, develop, commercialize, patent, manufacture, and obtain required regulatory approvals to market additional products, we may not generate revenues or royalties from commercial sales of these products for a significant number of years, if at all. Therefore, our proposed operations are subject to all the risks inherent in the establishment of a new business enterprise. In the next few years, our revenues may be limited to minimal product sales, and any amounts that we receive under strategic partnerships and research or drug development collaborations that we may establish and, as a result, we may be unable to achieve or maintain profitability in the future or to achieve significant revenues in order to fund our operations.

Risks Related to our Common Stock

The market price of our common stock may be volatile and adversely affected by several factors.

The market price of our common stock could fluctuate significantly in response to various factors and events, including:

- our ability to integrate operations, technology, products and services;
- our ability to execute our business plan;
- operating results below expectations;
- announcements concerning product development results, including clinical trial results, or intellectual property rights of others;
- litigation or public concern about the safety of our potential products;
- our issuance of additional securities, including debt or equity or a combination thereof, which will be necessary to fund our operating expenses;
- announcements of technological innovations or new products by us or our competitors;
- loss of any strategic relationship;
- industry developments, including, without limitation, changes in healthcare policies or practices or third-party reimbursement policies; economic and other external factors;
- period-to-period fluctuations in our financial results; and
- whether an active trading market in our common stock develops and is maintained.

In addition, the securities markets have from time to time experienced significant price and volume fluctuations that are unrelated to the operating performance of particular companies. These market fluctuations may also materially and adversely affect the market price of our common stock.

We have not paid cash dividends in the past and do not expect to pay cash dividends in the foreseeable future. Any return on investment may be limited to the value of our common stock.

We have never paid cash dividends on our common stock and do not anticipate paying cash dividends on our common stock in the foreseeable future. The payment of dividends on our capital stock will depend on our earnings, financial condition and other business and economic factors affecting us at such time as the board of directors may consider relevant. If we do not pay dividends, our common stock may be less valuable because a return on your investment will only occur if the common stock price appreciates.

Our quarterly operating results may fluctuate significantly.

We expect our operating results to be subject to quarterly fluctuations. Our net loss and other operating results will be affected by numerous factors, including:

- variations in the level of expenses related to our development programs;

- addition or termination of clinical trials;
- any intellectual property infringement lawsuit in which we may become involved;
- regulatory developments affecting our product candidates; and
- our execution of any collaborative, licensing or similar arrangements, and the timing of payments we may make or receive under these arrangements.

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

Provisions of our charter documents could discourage an acquisition of our company that would benefit our stockholders and may have the effect of entrenching, and making it difficult to remove, management.

Provisions of our Certificate of Incorporation and By-laws may make it more difficult for a third party to acquire control of us, even if a change in control would benefit our stockholders. In particular, shares of our preferred stock may be issued in the future without further stockholder approval and upon such terms and conditions, and having such rights, privileges and preferences, as our Board of Directors may determine, including, for example, rights to convert into our common stock. The rights of the holders of our common stock will be subject to, and may be adversely affected by, the rights of the holders of any of our preferred stock that may be issued in the future. The issuance of our preferred stock, while providing desirable flexibility in connection with possible acquisitions and other corporate purposes, could have the effect of making it more difficult for a third party to acquire control of us. This could limit the price that certain investors might be willing to pay in the future for shares of our common stock and discourage these investors from acquiring a majority of our common stock. Further, the existence of these corporate governance provisions could have the effect of entrenching management and making it more difficult to change our management.

Failure to achieve and maintain effective internal controls could have a material adverse effect on our business.

Effective internal controls are necessary for us to provide reliable financial reports. If we cannot provide reliable financial reports, our operating results could be harmed. All internal control systems, no matter how well designed, have inherent limitations. Therefore, even those systems determined to be effective can provide only reasonable assurance with respect to financial statement preparation and presentation.

Based on our evaluation, our management concluded that there is no material weakness in our internal control over financial reporting for the year ended December 31, 2018 based on the criteria established in Internal Control — Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (“COSO”).

While we continue to evaluate and improve our internal controls, we cannot be certain that these measures will ensure adequate controls over our financial processes and reporting in the future. Any failure to implement required new or improved controls, or difficulties encountered in their implementation, could harm our operating results or cause us to fail to meet our reporting obligations. Failure to achieve and maintain an effective internal control environment could cause investors to lose confidence in our reported financial information, which could have a material adverse effect on our stock price. Failure to comply with Section 404 could also potentially subject us to sanctions or investigations by the Securities and Exchange Commission (“SEC”) or other regulatory authorities.

There can be no assurance that we will be able to comply with continued listing standards of the Nasdaq Capital Market.

The Nasdaq Capital Market’s continued listing standards for our common stock require, among other things, that (i) we maintain a closing bid price for our common stock of at least $1.00, and (ii) we maintain: (A) stockholders’ equity of $2.5 million; (B) market value of listed securities of $35 million; or (C) net income from continuing operations of $500,000 in the most recently completed fiscal year or in two of the last three most recently completed fiscal years. Any failures to satisfy any continued listing requirements

could lead to the receipt of a deficiency notice from Nasdaq and ultimately to a delisting from trading of our common stock. We cannot assure you that we will be able to continue to comply with the minimum bid price and the other standards that we are required to meet in order to maintain a listing of our common stock on the Nasdaq Capital Market. Our failure to continue to meet these requirements may result in our common stock being delisted from the Nasdaq Capital Market. If our common stock were delisted from the Nasdaq Capital Market, among other things, this could result in a number of negative implications, including reduced liquidity in our common stock as a result of the loss of market efficiencies associated with Nasdaq and the loss of federal preemption of state securities laws as well as the potential loss of confidence by suppliers, customers and employees, institutional investor interest, fewer business development opportunities, greater difficulty in obtaining financing and breaches of certain contractual obligations.

Our ability to use our net operating loss carry forwards may be subject to limitation.

Generally, a change of more than 50% in the ownership of a company's stock, by value, over a three-year period constitutes an ownership change for U.S. federal income tax purposes. An ownership change may limit our ability to use our net operating loss carryforwards attributable to the period prior to the change. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may become subject to limitations, which could potentially result in increased future tax liability for us. At December 31, 2018, we had net operating loss carryforwards aggregating approximately $184.6 million.

Ownership of our shares is concentrated in the hands of a few investors which could limit the ability of our other stockholders to influence the direction of the Company.

As calculated by SEC rules of beneficial ownership, SCO Capital Partners LLC and affiliates beneficially owned approximately 28% of our common stock as of March 13, 2019. Accordingly, they collectively have the ability to significantly influence or determine the election of all of our directors or the outcome of most corporate actions requiring stockholder approval. They may exercise this ability in a manner that advances their best interests and not necessarily those of our other stockholders.

ITEM 1B. UNRESOLVED STAFF COMMENTS

Not Applicable.

ITEM 2. PROPERTIES

Our corporate headquarters are located in New York, New York, where we currently lease 10,400 square feet of office space that expires in January 2026. We also lease 43,700 square feet of manufacturing, laboratory and office space in Cleveland, Ohio that expires in December 2025. Finally, we lease 1,700 square feet of office space in Madrid, Spain that expires in September 2019; we expect to renew this lease before it expires. We believe that our facilities are sufficient to meet our current needs and that suitable space will be available as and when needed.

ITEM 3. LEGAL PROCEEDINGS

On January 18, 2018, William Mahon, a Company stockholder, served a demand upon the Company's board of directors (the "Board") pursuant to Section 220 of the Delaware General Corporation Law (the "Demand") seeking to inspect certain of the Company's books and records. Generally, the Demand's stated purpose was to investigate allegedly excessive compensation awarded to non-employee Board members for the fiscal years 2015–2017. The Board denied the allegations in the Demand, and agreed to provide limited books and records to Mahon. On September 17, 2018, another Company stockholder, Francisco Dos Ramos, filed a stockholder derivative complaint in the Delaware Chancery Court (the "Dos Ramos Action") against Steven Rouhandeh, Frank Carsten Thiel, Mark Alvino, Stefano Buono, Stephen Howell, Richard Van Duyne, and Todd Wider as defendants, and the Company as nominal defendant (the "Dos Ramos Defendants"). Dos Ramos generally alleges that the Board breached its fiduciary duties, were unjustly enriched, and committed corporate waste by approving allegedly excessive compensation to non-employee Board members for the fiscal years 2015–2017. Dos Ramos generally seeks disgorgement of

the allegedly improper payments to the Board, money damages, an order requiring corporate governance reforms, costs and attorneys' fees. On November 28, 2018, while the motion to dismiss was pending, Mahon filed a stockholder derivative complaint (the "Mahon Action") in the United States District Court for the District of Delaware against Mark Ahn, Mark Alvino, Jeffrey Davis, Stephen Howell, Todd Wider, and Steven Rouhandeh, as defendants, and the Company as a nominal defendant ("Mahon Defendants"). The allegations in the Mahon Action are substantially similar to those set forth in his Demand, as well as those in the Dos Ramos Action. Mahon generally seeks the disgorgement of the allegedly improper payments to the Board, a constructive trust, money damages, costs and attorneys' fees. On December 6, 2018, Mahon and Defendants filed a joint motion for preliminary approval of settlement, along with a stipulation of settlement (the "Stipulation") intending to settle all claims asserted in the Mahon Action. Pursuant to the Stipulation, the Company shall adopt and implement certain governance reforms for a period of three years, and at its discretion thereafter. In exchange, the Company agrees not to oppose a petition for attorneys' fees by Mahon's counsel up to $240,000. On December 10, 2018, the Dos Ramos Defendants filed a motion to dismiss. On January 8, 2019, the court approved the parties' notice of settlement, enjoining all Company stockholders from commencing or further prosecuting any claims asserted in the Mahon Action, and scheduled a settlement approval hearing for May 1, 2019. On January 25, 2019, the Chancery Court entered an order staying the Dos Ramos Action pending the settlement hearing in the Mahon Action.

On October 22, 2018, EB Research Partnership, Inc. ("EBRP") served upon the Company a Request for Arbitration (the "Request"), alleging that the Company is in breach of an Agreement executed in July 2016 (the "Agreement") between and among the Company, EBRP, and Epidermolysis Bullosa Medical Research Foundation ("EBMRF"). EBRP alleges that Abeona has refused to provide to EBRP 350,000 unrestricted shares of Abeona common stock, purportedly in breach of the Agreement. On November 21, 2018, the Company filed an action in the United States District Court for the Southern District of New York seeking a declaration that it is not required to arbitrate its dispute with EBRP on the basis that the Agreement is void for lack of consideration. On February 4, 2019, the court granted EBRP and EBMRF's motion to compel arbitration. The parties' initial submissions to the arbitrator are due March 18, 2019. The Company intends to defend against the claims vigorously.

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

Price Range of Common Stock and Dividend Policy

Our common stock has traded on the Nasdaq Capital Market ("Nasdaq") under the symbol "ABEO" since June 22, 2015.

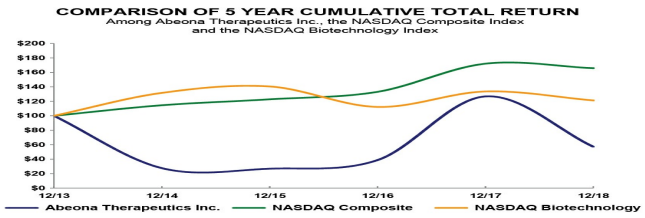
We have never declared or paid any cash dividends on our common stock and we do not anticipate paying any cash dividends on our common stock in the foreseeable future. The payment of dividends, if any, in the future is within the discretion of our Board of Directors and will depend on our earnings, capital requirements and financial condition and other relevant facts. We currently intend to retain all future earnings, if any, to finance the development and growth of our business.

The number of record holders of our common stock at March 13, 2019 was approximately 167. On March 13, 2019, the closing price for the common stock as quoted on Nasdaq was $7.43. There were 47,949,694 shares of common stock outstanding at March 13, 2019.

Performance Graph

The performance information below relates to sales prices of our predecessor Access Pharmaceuticals, Inc. for the period from December 31, 2013 to October 23, 2014, our predecessor PlasmaTech Biopharmaceuticals, Inc. for the period from October 24, 2014 to June 18, 2015 and Abeona Therapeutics Inc. from June 19, 2015 to December 31, 2018.

The stock performance graph below shows how an initial investment of $100 in our shares would have compared over a five-year period with an equal investment in (1) the Nasdaq Composite Index and (2) the Nasdaq Biotechnology Index as defined below.



	December 31, 2013	December 31, 2014	December 31, 2015	December 31, 2016	December 31, 2017	December 31, 2018
Abeona Therapeutics Inc.	$ 100.00	$ 27.60	$ 26.88	$ 38.80	$ 126.80	$ 57.12
Nasdaq Composite Index	100.00	114.62	122.81	133.19	172.11	165.84
Nasdaq Biotechnology Index	100.00	131.71	140.56	112.25	133.67	121.24

Equity Compensation Plan Information

The following table sets forth, as of December 31, 2018, information about shares of common stock outstanding and available for issuance under our existing equity compensation plans.

Plan Category	Number of securities to be issued upon exercise of outstanding options, warrants and rights	Weighted-average exercise price of outstanding options, warrants and rights	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a))
	(a)	(b)	(c)
Equity compensation plans approved by security holders:			
2015 Equity Incentive Plan	5,525,405	$ 8.08	2,333,346
2005 Equity Incentive Plan	316,400	14.15	—
Equity compensation plans not approved by security holders	—	—	—
Total	5,841,805	$ 8.41	2,333,346

Issuer Repurchases of Equity Securities

None.

Recent Sales of Unregistered Securities

None.

ITEM 6. SELECTED FINANCIAL DATA

The following discussion should be read in conjunction with our consolidated financial statements and related notes included in this Form 10-K. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period. The financial information included in the tables below are derived from audited financial statements.

	For the years ended December 31,				
	2018	2017	2016	2015	2014
Statement of Operations Data:					
Foundation revenues[1]	$ 2,796,000	$ —	$ —	$ —	$ —
License revenues[1]	—	602,000	602,000	602,000	598,000
Royalties[1]	202,000	235,000	287,000	438,000	327,000
Research and development	38,698,000	16,989,000	10,655,000	4,715,000	333,000
General and administrative	20,106,000	10,943,000	13,290,000	14,320,000	3,712,000
Loss from operations	(58,166,000)	(27,836,000)	(23,881,000)	(18,546,000)	(3,131,000)
Net loss	(56,671,000)	(27,319,000)	(21,873,000)	(14,526,000)	(26,778,000)
Basic and diluted loss per common share	(1.19)	(0.66)	(0.64)	(0.53)	(15.26)
Weighted average number of common shares outstanding – basic and diluted	47,528,248	41,636,752	34,180,253	27,597,434	1,942,905

	As of December 31,				
	2018	2017	2016	2015	2014
Balance Sheet Data:					
Current assets	$ 88,851,000	$140,592,000	$ 69,421,000	$40,568,000	$11,555,000
Current liabilities[1]	20,354,000	5,607,000	8,296,000	1,477,000	2,898,000
Working capital[1]	68,497,000	134,985,000	61,125,000	39,091,000	8,657,000
Property and equipment, net	9,443,000	1,374,000	721,000	350,000	4,000
Licensed technology, net	43,042,000	3,977,000	8,384,000	6,609,000	4,991,000
Total assets	174,399,000	178,766,000	111,058,000	80,055,000	16,582,000
Total stockholders' equity[1]	134,045,000	170,098,000	99,098,000	67,721,000	4,816,000

(1) Effective January 1, 2018, we adopted ASU 2014-09, *Revenue from Contracts with Customers*, as amended (ASC 606), using the modified retrospective transition method. The ASC 606 revenue recognition standard replaced the prior revenue recognition standard ASC 605, *Revenue Recognition*. The cumulative effect of applying the ASC 606 standard was an increase of $6,275,000 to stockholders' equity as of January 1, 2018. The balance sheet and the statement of operations as of and for the year ended December 31, 2018 are presented under ASC 606. Our balance sheets prior to December 31, 2018 and statement of operations prior to 2018 are presented under ASC 605. Please refer to Note 2, *New Accounting Standards Implemented-Revenue Recognition*, to the consolidated financial statements for more information.

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

The following discussion should be read in conjunction with our consolidated financial statements and related notes included in this Form 10-K.

Abeona is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Our lead programs include EB-101, an autologous, gene-corrected cell therapy for RDEB, ABO-102, an AAV-based gene therapy for MPS IIIA, and ABO-101 an AAV-based gene therapy for MPS IIIB. We are also developing ABO-201 and ABO-202, which are AAV-based gene therapies for the CLN1 and CLN3 forms of Batten Disease, ABO-401 for the treatment of cystic fibrosis, and ABO-5OX for the treatment of retinal diseases. In addition, we are developing a novel vector platform, AIM™ for gene therapy product candidates.

Results of Operations

Effective January 1, 2018, we adopted ASU 2014-09, *Revenue from Contracts with Customers*, as amended (ASC 606), using the modified retrospective transition method. The ASC 606 revenue recognition standard replaced the prior revenue recognition standard ASC 605, *Revenue Recognition*. The cumulative effect of applying the ASC 606 standard was an increase of $6.3 million to stockholders' equity as of January 1, 2018. The statement of operations for the year ended December 31, 2018 are presented under ASC 606, while our statement of operations for the years ended December 31, 2017 and 2016 are presented under ASC 605.

Comparison of Years Ended December 31, 2018 and December 31, 2017

Foundation revenues relate to a collaborative agreement between us and nine Sanfilippo foundations to provide up to approximately $13.9 million of grants to us in installments for the advancement of our clinical stage gene therapies for MPS IIIA and MPS IIIB, subject to the achievement of certain milestones. Our foundation revenue was $2.8 million and $0 for the years ended December 31, 2018 and 2017, respectively. As of December 31, 2017, we had received $2.6 million of these grants and recorded them as deferred revenue under ASC 605 on our consolidated balance sheet. There was no foundation revenue recorded in 2017 under ASC 605 since we had not achieved the milestones outlined in each installment. In conjunction with our adoption of ASC 606 on January 1, 2018, we concluded that the cash received upfront from the foundations should be deferred on the consolidated balance sheet until the costs of the activities as outlined in the manufacturing and clinical work plan are incurred by installment as outlined in the agreement with the foundations. As a result, we recorded foundation revenues of $2.8 million in 2018 to match the costs of the activities performed under the collaborative agreement.

Our licensing revenue was $0 and $0.6 million for the years ended December 31, 2018 and 2017, respectively. In 2017, we recognized licensing revenue over the period of the performance obligation under our licensing agreements under the guidance in ASC 605. The adoption of ASC 606 in 2018 resulted in recognizing licensing revenue at the point of sale to the licensee and no longer amortizing revenue over time. As a result, deferred licensing revenue on the date of adoption of ASC 606 was recorded as an adjustment to accumulated deficit on January 1, 2018.

We recorded royalty revenue for MuGard of $0.2 million for each of the years ended December 31, 2018 and 2017. We licensed MuGard to AMAG Pharmaceuticals, Inc. ("AMAG") and Norgine B.V. ("Norgine") and receive quarterly royalties under our agreements.

Total research and development spending for the year ended December 31, 2018 was $38.7 million, as compared to $17.0 million for the same period of 2017, an increase of $21.7 million. The increase in expenses was primarily due to:

- increased clinical and development work for the manufactured product for EB-101, ABO-102 and ABO-101 and other gene therapy products ($13.7 million);
- increased salary and related costs ($4.2 million) due to hiring additional scientific staff;
- increased stock-based compensation expense ($2.1 million); and

- increased scientific consulting expense ($1.4 million).

Total general and administrative expenses were $20.1 million for the year ended December 31, 2018, as compared to $10.9 million for the same period of 2017, an increase of $9.2 million. The increase in expenses was due primarily to the following:

- increased stock-based compensation expense ($1.4 million);
- increased salary and related costs ($3.4 million);
- increased professional fees ($2.8 million); and
- increased recruiting expenses ($0.9 million).

Depreciation and amortization expenses were $2.4 million for the year ended December 31, 2018, as compared to $0.7 million for the same period in 2017, an increase of $1.7 million. The increase was partially driven by increased depreciation expense resulting from the build-out of the manufacturing facility in Cleveland and additional administrative office space in Cleveland and New York as well as increased amortization expense resulting from the REGENXBIO license.

Net loss for the year ended December 31, 2018 was $56.7 million, or a $1.19 basic and diluted loss per common share as compared to a net loss of $27.3 million, or a $0.66 basic and diluted loss per common share, for the same period in 2017.

Comparison of Years Ended December 31, 2017 and December 31, 2016

We did not generate foundation revenue in 2017 or 2016. Our licensing revenue was $0.6 million for each of the years ended December 31, 2017 and 2016. During both years, we recognized licensing revenue over the period of the performance obligation under our licensing agreements in accordance with ASC 605.

We recorded royalty revenue for MuGard of $0.2 million and $0.3 million for the years ended December 31, 2017 and 2016, respectively.

Total research and development spending for the year ended December 31, 2017 was $17.0 million, as compared to $10.7 million for the same period of 2016, an increase of $6.3 million. The increase in expenses was primarily due to:

- increased clinical and development work for the manufactured product for EB-101, ABO-102 and ABO-101 and other gene therapy products ($4.4 million);
- increased salary and related costs ($0.6 million) due to hiring additional scientific staff;
- increased stock option compensation expense ($0.4 million);
- increased travel and entertainment expense ($0.3 million); and
- increased scientific consulting expense ($0.2 million).

Total general and administrative expenses were $10.9 million for the year ended December 31, 2017, as compared to $13.3 million for the same period of 2016, a decrease of $2.4 million. The decrease in expenses was due primarily to the following:

- decreased restricted common stock-based compensation expense ($2.0 million) and decreased stock option compensation expense ($0.6 million);
- decreased salary and related costs ($0.3 million); and
- decreases in net other general and administrative expenses ($0.1 million); offset by
- increased patent expenses ($0.4 million); and
- investor relation expenses ($0.2 million).

Depreciation and amortization expenses were $0.7 million for the year ended December 31, 2017 as compared to $0.8 million for the same period in 2016.

Net loss for the year ended December 31, 2017 was $27.3 million, or a $0.66 basic and diluted loss per common share, as compared to a net loss of $21.9 million, or a $0.64 basic and diluted loss per common share, for the same period in 2016.

Liquidity and Capital Resources

We have historically funded our operations primarily through sales of common stock and, to a significantly lesser extent, foundation grants and licensing agreements. Our principal source of liquidity is cash, cash equivalents and short-term investments. As of December 31, 2018 and 2017, our cash, cash equivalents and short-term investments were $85.0 million and $138.8 million, respectively.

As of December 31, 2018 and 2017, our working capital was $68.5 million and $135.0 million, respectively. The decrease in working capital at December 31, 2018 results primarily from $39.1 million of cash needed for operating activities, $9.2 million for capital expenditures and $10.0 million for the acquisition of the REGENXBIO license during 2018.

On August 17, 2018, we entered into an open market sale agreement with Jefferies LLC. Pursuant to the terms of this agreement, we may sell from time to time, through Jeffries LLC, shares of our common stock for an aggregate sales price of up to $150 million. Any sales of shares pursuant to this agreement will be made under our effective "shelf" registration statement on Form S-3 that is on file and has been declared effective by the SEC Staff.

On October 19, 2017, we closed an underwritten public offering of 5,750,000 shares of common stock, at a public offering price of $16.00 per share. The gross proceeds to the Company were approximately $92.0 million, before deducting the underwriting discounts and commissions and estimated offering expenses payable by the Company.

On October 16, 2017, we announced a collaborative agreement between us and nine Sanfilippo foundations to provide up to approximately $13.9 million of grants to us in installments for the advancement of our clinical stage gene therapies for MPS IIIA and MPS IIIB, subject to the achievement of certain milestones. As of December 31, 2018, we had received $5.7 million of such grants.

We have incurred negative cash flows from operations since inception, and have expended, and expect to continue to expend, substantial funds to complete our planned product development efforts. Since inception, our expenses have significantly exceeded revenues, resulting in an accumulated deficit as of December 31, 2018 of $410.2 million. We cannot provide assurance that we will ever be able to generate sufficient product sales or royalty revenue to achieve profitability on a sustained basis, or at all.

Since our inception, we have devoted our resources primarily to fund our research and development programs. We have not been profitable since inception and to date have received limited revenues from the sale of products. We expect to incur losses for the next several years as we continue to invest in product research and development, preclinical studies, clinical trials and regulatory compliance.

If we raise additional funds by selling additional equity securities, the relative equity ownership of our existing investors will be diluted and the new investors could obtain terms more favorable than previous investors. If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs, or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit or terminate our product development programs or any future commercialization efforts or grant rights to develop and market product candidates to third parties that we would otherwise prefer to develop and market ourselves.

We plan to expend substantial funds to conduct research and development programs, expand our manufacturing capabilities, preclinical studies and clinical trials of potential products, including research and development with respect to our acquired and developed technology. Our future capital requirements and adequacy of available funds will depend on many factors, including:

- the successful development and commercialization of our cell and gene therapy and other product candidates;

- the ability to establish and maintain collaborative arrangements with corporate partners for the research, development and commercialization of products;
- continued scientific progress in our research and development programs;
- the magnitude, scope and results of preclinical testing and clinical trials;
- the costs involved in filing, prosecuting and enforcing patent claims;
- the costs involved in conducting clinical trials;
- competing technological developments;
- the cost of manufacturing and scale-up;
- the ability to establish and maintain effective commercialization arrangements and activities; and
- successful regulatory filings.

We have devoted substantially all of our efforts and resources to research and development conducted on our own behalf. The following table summarizes research and development spending by project category, which spending includes, but is not limited to, payroll and personnel expense, lab supplies, preclinical expense, development cost, clinical trial expense, outside manufacturing expense and consulting expense:

Project	Years ended December 31,			Inception Date[1]
	2018	2017	2016	
Gene therapy	$38,593,000	$15,789,000	$ 8,846,000	$ 65,560,000
Plasma therapy	105,000	546,000	1,714,000	4,697,000
MuGard	—	22,000	45,000	5,434,000
Others[2]	—	632,000	50,000	40,702,000
Total	$38,698,000	$16,989,000	$10,655,000	$116,393,000

(1) Cumulative spending from inception of the Company or project through December 31, 2018.

(2) Includes other projects which the Company is no longer focused on pursuing.

Due to uncertainties and certain of the risk factors described above, including those relating to our ability to successfully commercialize our drug candidates, our ability to obtain necessary additional capital to fund operations in the future, our ability to successfully manufacture our products and our product candidates in clinical quantities or for commercial purposes, government regulation to which we are subject, the uncertainty associated with preclinical and clinical testing, intense competition that we face, market acceptance of our products, the potential necessity of licensing technology from third parties and protection of our intellectual property, it is not possible to reliably predict future spending or time to completion by project or product category or the period in which material net cash inflows from significant projects are expected to commence. If we are unable to timely complete a particular project, our research and development efforts could be delayed or reduced, our business could suffer depending on the significance of the project and we might need to raise additional capital to fund operations, as discussed in the risk factors above, including without limitation those relating to the uncertainty of the success of our research and development activities and our ability to obtain necessary additional capital to fund operations in the future. As discussed in such risk factors, delays in our research and development efforts and any inability to raise additional funds could cause us to eliminate one or more of our research and development programs.

We plan to continue our policy of investing any available funds in certificates of deposit, money market funds, government securities and investment-grade interest-bearing securities. We do not invest in derivative financial instruments.

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 under applicable SEC rules.

Contractual Obligations

The following table summarizes our significant contractual obligations as of the payment due date by period at December 31, 2018:

	Payments Due by Period				
	Less than 1 year	1 to 3 years	4 to 5 years	After 5 years	Total
Operating leases	$ 1,735,000	$ 3,430,000	$3,487,000	$3,546,000	$12,198,000
Payable to licensor	10,000,000	20,000,000	—	—	30,000,000
Purchase and other commitments	10,100,000	4,900,000	—	—	15,000,000

We enter into agreements in the normal course of business with clinical research organizations for clinical trials and clinical manufacturing organizations for supply manufacturing and with vendors for preclinical research studies and other services and products for operating purposes. These contractual obligations are cancelable at any time by us, generally upon prior written notice to the vendor, and are thus not included in the contractual obligations table.

Operating lease amounts represent future minimum lease payments under our non-cancelable operating lease agreements. The minimum lease payments above do not include any related common area maintenance charges or real estate taxes.

Under the terms of the license agreement with REGENXBIO, REGENXBIO has granted us an exclusive worldwide license (subject to certain non-exclusive rights previously granted for MPS IIIA), with rights to sublicense, to REGENXBIO's NAV AAV9 vector for the development and commercialization of gene therapies for the treatment of MPS IIIA, MPS IIIB, CLN1 Disease and CLN3 Disease. In return for these rights, REGENXBIO will receive a guaranteed $20 million upfront payment, $10 million of which was paid on signing of the agreement on November 4, 2018 and $10 million of which will be paid by November 4, 2019 and is included in the contractual obligations table above. In addition, REGENXBIO will receive a total of $100 million in annual fees, payable upon the second through sixth anniversaries of the agreement, $20 million of which is guaranteed and is included in the contractual obligations table above. REGENXBIO is also eligible to receive potential commercial milestone payments of up to $60 million as well as low double-digit royalties on net sales of products incorporating the licensed intellectual property; however, these amounts are not included since the payment is uncertain as of December 31, 2018.

In addition, we are also party to other license agreements, which include contingent payments. However, contingent payments related to these license agreements are not disclosed as the satisfaction of these contingent payments is uncertain at December 31, 2018 and, if satisfied, the timing of payment for these amounts was not reasonably estimable at December 31, 2018. Commitments related to the license agreements include contingent payments that will become payable if and when certain development, regulatory and commercial milestones are achieved. During the next 12 months, we do not expect to make milestone payments related to such license agreements.

Critical Accounting Policies and Estimates

The preparation of our consolidated financial statements in conformity with accounting principles generally accepted in the U.S. requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities at the date of the financial statements and the reported amount of revenues and expenses during the reported period. In applying our accounting principles, we must often make individual estimates and assumptions regarding expected outcomes or uncertainties. As you might expect, the actual results or outcomes are often different than the estimated or assumed amounts. These differences are usually minor and are included in our consolidated

financial statements as soon as they are known. Our estimates, judgments and assumptions are continually evaluated based on available information and experience. Because of the use of estimates inherent in the financial reporting process, actual results could differ from those estimates.

Receivables

Receivables are reported in the balance sheets at the outstanding amount net of an allowance for doubtful accounts. We continually evaluate the creditworthiness of our customers and their financial condition and generally do not require collateral. The allowance for doubtful accounts is based upon reviews of specific customer balances, historic losses, and general economic conditions. As of December 31, 2018 and 2017, no allowance was recorded as all accounts were considered collectible.

Licensed Technology

We maintain licensed technology on our consolidated balance sheet until either the licensed technology agreement underlying it is completed or the asset becomes impaired. When we determine that an asset has become impaired or we abandon a project, we write down the carrying value of the related intangible asset to its fair value and take an impairment charge in the period in which the impairment occurs.

Generally, licensed technology is amortized over the life of the patent or the agreement. We test our intangible assets for impairment on an annual basis, or more frequently if indicators are present or changes in circumstance suggest that impairment may exist. Events that could result in an impairment, or trigger an interim impairment assessment, include the receipt of additional clinical or nonclinical data regarding our drug candidate or a potentially competitive drug candidate, changes in the clinical development program for a drug candidate or new information regarding potential sales for the drug. In connection with each annual impairment assessment and any interim impairment assessment, we compare the fair value of the asset as of the date of the assessment with the carrying value of the asset on our consolidated balance sheet.

In 2018, 2017 and 2016, we did not impair any licensed technology.

Goodwill

As of December 31, 2018 and 2017, we recorded goodwill of $32.5 million on our consolidated balance sheet. In accordance with ASC 350 — *Intangibles — Goodwill and Other,* goodwill is tested annually for impairment and whenever changes in circumstances occur that would indicate impairment.

In 2018, 2017 and 2016, we did not impair any goodwill.

Revenue Recognition

Effective January 1, 2018, we adopted ASU 2014-09, *Revenue from Contracts with Customers*, as amended (ASC 606)), using the modified retrospective transition method. The ASC 606 revenue recognition standard replaced the prior revenue recognition standard ASC 605, *Revenue Recognition*. The cumulative effect of applying the ASC 606 standard was an increase of $6,275,000 to stockholders' equity as of January 1, 2018. The balance sheet and the statement of operations as of and for the year ended December 31, 2018 are presented under ASC 606, while our balance sheet as of December 31, 2017 and statement of operations for each of the two years ended December 31, 2017 are presented under ASC 605.

Foundation revenues related to a collaborative agreement between nine Sanfilippo foundations to provide up to approximately $13,850,000 of grants to Abeona in installments for the advancement of our clinical stage gene therapies for MPS IIIA and MPS IIIB, subject to the achievement of certain milestones. As of December 31, 2017, we had received $2,611,000 of these grants and recorded them as deferred revenue under ASC 605. There was no foundation revenue recorded in 2017 under ASC 605 since we had not achieved the milestones outlined in each installment. In conjunction with our adoption of ASC 606, we assessed the ASC 606-10-25-27 criteria used to determine whether the foundation revenue should be recognized over time and determined that our performance does not create an asset with an alternative use to the foundations and we have an enforceable right to payment for performance completed to date. We determined that the input method based on costs incurred in accordance with ASC 606-10-55-20 would be the most appropriate method for measuring progress. As a result, we have concluded that cash received

upfront from the foundations should be deferred on the balance sheet until the costs of the activities as outlined in the manufacturing and clinical work plan are incurred by installment as outlined in the agreement with the foundations. Effectively, this matches the revenue up to the costs incurred by installment. Should the aggregate cash received exceed the costs incurred by installment, the excess of aggregate cash over costs will be deferred.

License revenues relate to sales of certain of our technology in years 2008 through 2014. Under ASC 605, the initial upfront cash payments for license sales were recorded as deferred revenue on our consolidated balance sheet and then recognized as revenue on a straight-line basis over the life of the patent. In conjunction with our adoption of ASC 606, we assessed the nature of the promised license to determine whether the license had significant stand-alone functionality and evaluated whether such functionality can be retained without ongoing activities. We determined that the licenses have significant stand-alone functionality (i.e., functional intellectual property). Furthermore, we have no ongoing activities associated with the license to support or maintain the license's utility to the customer. Consistent with the functional IP guidance in ASU 2016-10, we determined that the pattern of transfer of control to the customer was at a point in time (since the customer was granted a right to use the IP). Given the above, we concluded that the license revenue would have been recognized at the point of the various sales during the period 2008 through 2014. So, upon adoption of ASC 606, the deferred revenue was written-off to accumulated deficit and there will be no further amortization of deferred revenue.

Royalty revenues result from the license of our MuGard product. In conjunction with our adoption of ASC 606, we assessed the nature of the promised license to determine whether the license had significant stand-alone functionality and evaluated whether such functionality can be retained without ongoing activities. We determined that the licenses have significant stand-alone functionality (i.e., functional intellectual property). Furthermore, we have no ongoing activities associated with the license to support or maintain the license's utility to the customer. We determined that the pattern of transfer of control to the customer was at a point in time (since the customer was granted a right to use the IP). Although the ASC 606 standard requires an entity to estimate and constrain variable consideration in a contract with a customer, there is an exception to the general model for consideration of sales-based royalty related to licenses of IP. As such, revenue is not recognized until (1) the underlying sale or usage has occurred and (2) the related performance obligation has been satisfied (or partially satisfied). That is, we are not required to estimate the amount of a sales-based royalty at contract inception; rather, revenue is recognized as the subsequent sale occurs. As a result, we have concluded that the royalty revenues will continue to be recognized as the subsequent sales of MuGard occur. There is effectively no change in the recognition of royalty revenue upon the adoption of ASC 606.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advanced payments. We make estimates of our accrued expenses as of each balance sheet date in our consolidated financial statements based on facts and circumstances known to us at that time. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the clinical expense. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or amount of prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in us reporting amounts that are too high or too low in any particular period. To date, we have not made any material adjustments to our prior estimates of accrued research and development expenses.

Stock-Based Compensation Expense

We account for stock-based compensation expense in accordance with FASB ASC 718, *Stock Based Compensation*. We have two stock-based compensation plans under which incentive and qualified stock

options and restricted shares may be granted to employees, directors and consultants. We measure the cost of the employee/director/consultant services received in exchange for an award of equity instruments based on the grant date fair value for employees and directors and vesting date fair value of the award for consultants. We use the Black-Scholes option pricing model to value our options which includes expected volatility, risk-free interest rate, dividend yield and estimated expected term.

Stock-based compensation expense recognized for the years ended December 31, 2018, 2017 and 2016 was approximately $8,178,000, $4,644,000 and $4,829,000, respectively.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Our business and financial results are not materially affected by fluctuations in currency exchange rates or interest rates. We do not use derivative financial instruments for trading or speculative purposes.

Interest Rate Risk

Our exposure to market risk for changes in interest rates relates to our investment portfolio. Our investment strategy is focused on preserving capital and supporting our liquidity requirements, while earning a reasonable market return. We invest only in U.S. government, U.S. agency and U.S. treasury securities. The market value of our investments would not materially decline if current market interest rates rise given the short duration of our investments.

Concentrations of Risk

We invest excess cash in short-term, fixed-rate debt securities, and diversify the investments between financial institutions.

Foreign Currency Fluctuation Risk

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we have contracted with and may continue to contract with foreign vendors that are located in Europe and Australia.

Inflation Fluctuation Risk

Inflation can affect us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the years ended December 31, 2018, 2017 and 2016.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

Financial statements required by this Item are incorporated in this Annual Report Form 10-K on pages F-1 through F-22 hereto. Reference is made to Item 15 of this Form 10-K.

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

None.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and principal financial officer, has evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act")), as of the end of the period covered by this Annual Report on Form 10-K. Based on such evaluation, our principal executive officer and principal financial officer have concluded that as of such date, our disclosure controls and procedures were effective.

Management's Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is defined in Rules 13a-15(f) and 15d-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, our principal executive and principal financial officers and effected by our board of directors, management and other personnel, 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 and includes those policies and procedures that:

- Pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of our assets;
- Provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and
- Provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on the financial statements. Under the supervision and with the participation of management, including our principal executive and financial officers, we assessed our internal control over financial reporting as of December 31, 2018, based on criteria for effective internal control over financial reporting established in Internal Control — Integrated Framework (2013), issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). Our management's assessment of the effectiveness of our internal control over financial reporting included testing and evaluating the design and operating effectiveness of our internal controls. In our management's opinion, we have maintained effective internal control over financial reporting as of December 31, 2018, based on criteria established in the COSO 2013 framework.

The effectiveness of our internal control over financial reporting as of December 31, 2018 has been audited by Whitley Penn LLP, an independent registered public accounting firm, as stated in their report, which is included herein.

Inherent Limitations of Internal Controls

Our management, including our principal executive officer and principal financial officer, does not expect that our disclosure controls and procedures or our internal controls will prevent all errors and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within the Company have been detected. These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of a simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by management override of the control. The design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions. Over time, controls may become inadequate because of changes in conditions, or the degree of

compliance with the policies or procedures may deteriorate. 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. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15(d)-15(f) promulgated under the Securities Exchange Act of 1934, during the fourth quarter of 2018 that have materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders of
Abeona Therapeutics Inc. and Subsidiaries

Opinion on Internal Control Over Financial Reporting

We have audited Abeona Therapeutics Inc. and subsidiaries' (the "Company") internal control over financial reporting as of December 31, 2018, based on criteria established in *2013 Internal Control — Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2018, based on criteria established in *2013 Internal Control — Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO).

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) ("PCAOB"), the consolidated balance sheets as of December 31, 2018 and 2017, and the related consolidated statements of operations, stockholders' equity, and cash flows for each of the three years ended December 31, 2018, of the Company, and our report dated March 18, 2019, expressed an unqualified opinion on those consolidated financial statements.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting, and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying *Management's Report on Internal Control Over Financial Reporting*. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit of internal control over financial reporting included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audit also included performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control Over Financial Reporting

The entity's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with accounting principles generally accepted in the United States of America. An entity's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the entity; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the entity are being made only in accordance with authorizations of management and directors of the entity; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the entity's assets that could have a material effect on the financial statements.

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

/s/ WHITLEY PENN LLP

Plano, Texas
March 18, 2019

ITEM 9B. OTHER INFORMATION

None.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

Directors and Reports of Beneficial Ownership. The information required by this Item is incorporated herein by reference from the information to be contained in our 2019 Proxy Statement to be filed with the SEC within 120 days after December 31, 2018 in connection with the solicitation of proxies for our 2019 Annual Meeting of Stockholders (the "2019 Proxy Statement").

Code of Ethics. We have adopted a Code of Business Conduct and Ethics (the "Code") that applies to all of our employees (including executive officers) and directors. The Code is available on our website at www.abeonatherapeutics.com under the heading "Investors & Media-Corporate Governance-Governance Documents." We intend to satisfy the disclosure requirement regarding any waiver of a provision of the Code applicable to any executive officer or director, by posting such information on such website. We shall provide to any person without charge, upon request, a copy of the Code. Any such request must be made in writing to Abeona Therapeutics Inc., c/o Investor Relations, 1330 Avenue of the Americas, 33rd Floor, New York, NY 10019.

Our corporate governance guidelines and the charters of the Audit Committee, Compensation Committee and Nominating and Corporate Governance Committee of the Board of Directors are available on our website at www.abeonatherapeutics.com under the heading "Investors & Media-Corporate Governance-Governance Documents." We shall provide to any person without charge, upon request, a copy of any of the foregoing materials. Any such request must be made in writing to Abeona Therapeutics Inc., c/o Investor Relations, 1330 Avenue of the Americas, 33rd Floor, New York, NY 10019.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item is contained in the 2019 Proxy Statement and is incorporated herein by reference.

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

The information required by this Item is contained in the 2019 Proxy Statement and is incorporated herein by reference.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS AND DIRECTOR INDEPENDENCE

The information required by this Item is contained in the 2019 Proxy Statement and is incorporated herein by reference.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

The information required by this Item is contained in the 2019 Proxy Statement and is incorporated herein by reference.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES

b. Exhibits

Exhibit Number	Description of Document
2.1	Amended and Restated Agreement of Merger and Plan of Reorganization between the Registrant and Chemex Pharmaceuticals, Inc., dated as of October 31, 1995 (Incorporated by reference to Exhibit A of our Registration Statement on Form S-4 dated December 20, 1995, Commission File No. 33-64031)
2.2	Agreement and Plan of Merger, by and among the Registrant, MACM Acquisition Corporation and MacroChem Corporation, dated July 9, 2008 (Incorporated by reference to Exhibit 2.3 of our Form 10-Q for the quarter ended June 30, 2008)
3.1	Restated Certificate of Incorporation of Abeona Therapeutics Inc. (Incorporated by reference to Exhibit 3(a) of our Form 8-K dated July 12, 1989, Commission File Number 9-9134)
3.2	Amended and Restated Bylaws of Abeona Therapeutics Inc.
4.1*	2015 Equity Incentive Plan (Incorporated by reference to Exhibit 4.1 to our Form S-8 filed May 11, 2015)
4.2*	2015 Equity Incentive Plan amendment (Incorporated by reference to our Definitive Proxy Statement on Schedule 14A filed on April 4, 2016)
4.3*	2015 Equity Incentive Plan, as amended and in effect (Incorporated by reference to Exhibit 4.1 of our Registration Statement on Form S-8 dated May 11, 2015, Commission File No. 333-204055)
10.1*	401(k) Plan (Incorporated by reference to Exhibit 10.20 of our Form 10-K for the year ended December 31, 1999)
10.2*	2005 Equity Incentive Plan (Incorporated by reference to Exhibit 1 of our Proxy Statement filed on April 18, 2005)
10.3	Board Designation Agreement dated November 15, 2007, between the Registrant and SCO Capital Partners LLC (Incorporated by reference to Exhibit 10.26 of our Form S-1 filed on March 11, 2008)
10.4+	License Agreement, dated June 6, 2013, by and between us and AMAG Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 10.16 to our Form 10-Q for the quarter ended June 30, 2013 filed on August 14, 2013)
10.5	Agreement and Plan of Merger, dated May 5, 2015, by and among the Company, PlasmaTech Merger Sub Inc., Abeona Therapeutics LLC and Paul A. Hawkins, in his capacity as Member Representative (Incorporated by reference to Exhibit 10.1 to our Form 10-Q for the quarter ended June 30, 2015 filed on August 14, 2015)
10.6	Form of Common Stock Purchase Agreement, dated April 1, 2015 (Incorporated by reference to Exhibit 10.4 to our Form 10-Q for the quarter ended June 30, 2015 filed on August 14, 2015)
10.7	Form of Securities Purchase Agreement dated May 6, 2015 (Incorporated by reference to Exhibit 10.5 to our Form 10-Q for the quarter ended June 30, 2015 filed on August 14, 2015)

Exhibit Number	Description of Document
10.8*	Employment Agreement dated May 6, 2015 between the Company and Timothy J. Miller (Incorporated by reference to Exhibit 10.1 to our Form 10-Q for the quarter ended September 30, 2015 filed on November 16, 2015)
10.9	Form of Indemnification Agreement, between us and directors and officers of the Company (Incorporated by reference to Exhibit 10.1 to our Form 10-Q for the quarter ended June 30, 2016 filed on August 15, 2016)
10.10*	Employment Agreement dated March 29, 2018 between the Company and F. Carsten Thiel (Incorporated by reference to Exhibit 10.1 to our Form 10-Q for the quarter ended March 31, 2018 filed on May 10, 2018)
10.11*	Letter of Agreement and General Release dated October 1, 2018, by and between the Company and Jeffrey Davis (incorporated by reference to Exhibit 10.1 of Form 8-K filed on October 5, 2018 (File No. 001-15771))
10.12*	Letter of Agreement dated October 5, 2018, by and between the Company and Stephen B. Thompson (incorporated by reference to Exhibit 10.1 of Form 8-K/A filed on October 9, 2018 amending the Form 8-K filed on October 5, 2018 (File No. 001-15771))
10.13*	Offer Letter, effective October 19, 2018, by and between the Company and Edward Carr (incorporated by reference to Exhibit 10.1 of Form 8-K filed on November 9, 2018)
10.14*	Letter Agreement between the Company and Joao Siffert, M.D., dated September 28, 2018 (incorporated by reference to Exhibit 10.1 of Form 8-K filed on November 30, 2018)
10.15*	Letter Agreement between the Company and Joao Siffert, M.D., dated November 29, 2018 (incorporated by reference to Exhibit 10.2 of Form 8-K filed on November 30, 2018)
10.16*	Separation Agreement between the Company and F. Carsten Thiel, Ph.D., dated November 29, 2018 (incorporated by reference to Exhibit 10.3 of Form 8-K filed on November 30, 2018)
10.17	Open Market Sale Agreement, dated August 17, 2018, by and between the Company and Jefferies LLC (incorporated by reference to Exhibit 1.1 of Form 8-K filed on August 20, 2018 (File No. 001-15771))
10.18+	License Agreement, dated November 4, 2018, between the Company and REGENXBIO Inc.
21	Subsidiaries of the Registrant
23.1	Consent of Whitley Penn LLP
31.1	Principal Executive Officer Certification Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Principal Financial Officer Certification Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32	Principal Executive Officer Certification and Principal Financial Officer Certification Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101	The following materials from the Company's Annual Report on Form 10-K, formatted in XBRL (Extensible Business Reporting Language): (i) Consolidated Balance Sheets, (ii) Consolidated Statements of Operations, (iii) Consolidated Statements of Stockholders' Deficit, (iv) Consolidated Statements of Cash Flows, and (v) Notes to Consolidated Financial Statements, tagged as blocks of text.

* Management contract or compensatory plan required to be filed as an Exhibit to this Form pursuant to Item 15c of the report.

\+ Portions of this exhibit were omitted and filed separately with the U.S. Securities and Exchange Commission pursuant to a request for confidential treatment.

ITEM 16. FORM 10-K SUMMARY

None.

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.

ABEONA THERAPEUTICS INC.

Date: March 18, 2019 By: /s/ Steven H. Rouhandeh
Steven H. Rouhandeh
Executive Chairman
Principal Executive Officer

Date: March 18, 2019 By: /s/ Christine Silverstein
Christine Silverstein
Chief Financial Officer
Principal Financial Officer

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

Date: March 18, 2019 By: /s/ Steven H. Rouhandeh
Steven H. Rouhandeh
Executive Chairman
Principal Executive Officer
Chairman of the Board

Date: March 18, 2019 By: /s/ Christine Silverstein
Christine Silverstein
Chief Financial Officer
Principal Financial Officer

Date: March 18, 2019 By: /s/ Edward G. Carr
Edward G. Carr
Chief Accounting Officer
Principal Accounting Officer

Date: March 18, 2019 By: /s/ Mark J. Alvino
Mark J. Alvino, Director

Date: March 18, 2019 By: /s/ Stefano Buono
Stefano Buono, Director

Date: March 18, 2019 By: /s/ Stephen B. Howell
Stephen B. Howell, Director

Date: March 18, 2019 By: /s/ João Siffert
João Siffert, Director

Date: March 18, 2019 By: /s/ Richard Van Duyne
Richard Van Duyne, Director

Date: March 18, 2019 By: /s/ Todd Wider
Todd Wider, Director

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

Board of Directors and Stockholders of
Abeona Therapeutics Inc. and Subsidiaries

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Abeona Therapeutics Inc. and subsidiaries (the "Company"), as of December 31, 2018 and 2017, and the related consolidated statements of operations, stockholders' equity and cash flows for each of the three years ended December 31, 2018, and the related notes (collectively referred to as the "financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2018 and 2017, and the results of their operations and their cash flows for each of the three years ended December 31, 2018, in conformity with accounting principles generally accepted in the United States of America.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) ("PCAOB"), the Company's internal control over financial reporting as of December 31, 2018, based on criteria established in *2013 Internal Control — Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO), and our report dated March 18, 2019, expressed an unqualified opinion.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ WHITLEY PENN LLP

We have served as the Company's auditor since 2006.

Plano, Texas
March 18, 2019

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

CONSOLIDATED BALANCE SHEETS

	December 31, 2018	December 31, 2017
ASSETS		
Current assets:		
Cash and cash equivalents	$ 18,750,000	$ 137,750,000
Short-term investments	66,218,000	—
Receivables	81,000	107,000
Prepaid expenses and other current assets	3,802,000	2,735,000
Total current assets	88,851,000	140,592,000
Property and equipment, net	9,443,000	1,374,000
Licensed technology, net	43,042,000	3,977,000
Goodwill	32,466,000	32,466,000
Other assets and restricted cash	597,000	357,000
Total assets	$ 174,399,000	$ 178,766,000
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 6,122,000	$ 1,881,000
Accrued expenses	3,936,000	512,000
Current portion of payable to licensor	10,000,000	—
Current portion of deferred revenue	296,000	3,214,000
Total current liabilities	20,354,000	5,607,000
Payable to licensor, net of current portion	20,000,000	—
Deferred revenue, net of current portion	—	3,061,000
Total liabilities	40,354,000	8,668,000
Commitments and contingencies		
Stockholders' equity:		
Common stock – $0.01 par value; authorized 200,000,000 shares; issued and outstanding 47,944,486 at December 31, 2018; issued and outstanding 46,888,108 at December 31, 2017	479,000	469,000
Additional paid-in capital	543,754,000	529,421,000
Accumulated deficit	(410,188,000)	(359,792,000)
Total stockholders' equity	134,045,000	170,098,000
Total liabilities and stockholders' equity	$ 174,399,000	$ 178,766,000

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

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

CONSOLIDATED STATEMENTS OF OPERATIONS

	For the years ended December 31,		
	2018	**2017**	**2016**
Revenues:			
Foundation revenues	$ 2,796,000	$ —	$ —
License revenues	—	602,000	602,000
Royalties	202,000	235,000	287,000
Total revenues	2,998,000	837,000	889,000
Expenses:			
Research and development	38,698,000	16,989,000	10,655,000
General and administrative	20,106,000	10,943,000	13,290,000
Depreciation and amortization	2,360,000	741,000	825,000
Total expenses	61,164,000	28,673,000	24,770,000
Loss from operations	(58,166,000)	(27,836,000)	(23,881,000)
Interest and miscellaneous income	1,506,000	525,000	2,014,000
Interest and other expense	(11,000)	(8,000)	(6,000)
Net loss	$(56,671,000)	$(27,319,000)	$(21,873,000)
Basic and diluted loss per common share	$ (1.19)	$ (0.66)	$ (0.64)
Weighted average number of common shares outstanding – basic and diluted	47,528,248	41,636,752	34,180,253

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

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount			
Balance, December 31, 2015	32,743,013	$328,000	$377,993,000	$(310,600,000)	$ 67,721,000
Stock-based compensation expense	—	—	4,829,000	—	4,829,000
Restricted stock-based compensation expense	100,000	1,000	3,431,000	—	3,432,000
Restricted common stock issued for $2.85 share	52,690	—	150,000	—	150,000
Common stock issued for:					
– $3.27 per share for licenses	750,000	8,000	2,444,000	—	2,452,000
– $6.44 per share net of costs	158,029	2,000	967,000	—	969,000
– $7.00 per share net of costs	6,293,889	63,000	41,005,000	—	41,068,000
– cash exercise of options	151,000	1,000	349,000	—	350,000
– exercise of $5.00 warrants	5,836	—	—	—	—
Net loss	—	—	—	(21,873,000)	(21,873,000)
Balance, December 31, 2016	40,254,457	$403,000	$431,168,000	$(332,473,000)	$ 99,098,000
Stock-based compensation expense	—	—	4,644,000	—	4,644,000
Restricted stock-based compensation expense	—	—	1,272,000	—	1,272,000
Common stock issued for:					
– $16.00 per share net of costs	5,750,000	58,000	86,116,000	—	86,174,000
– cash exercise of options	81,719	—	344,000	—	344,000
– exercise of $8.00 warrants	625,000	6,000	4,994,000	—	5,000,000
– exercise of $5.00 warrants	176,932	2,000	883,000	—	885,000
Net loss	—	—	—	(27,319,000)	(27,319,000)
Balance, December 31, 2017	46,888,108	$469,000	$529,421,000	$(359,792,000)	$170,098,000
Cumulative effect adjustment of ASC 606 on January 1, 2018	—	—	—	6,275,000	6,275,000
Stock-based compensation expense	—	—	8,178,000	—	8,178,000
Restricted stock-based compensation expense	—	—	688,000	—	688,000
Common stock issued for:					
– cash exercise of options	360,853	4,000	2,240,000	—	2,244,000
– exercise of $5.00 warrants	646,763	6,000	3,227,000	—	3,233,000
– cashless warrant exercises	48,762	—	—	—	—
Net loss	—	—	—	(56,671,000)	(56,671,000)
Balance, December 31, 2018	47,944,486	$479,000	$543,754,000	$(410,188,000)	$134,045,000

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

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

CONSOLIDATED STATEMENTS OF CASH FLOWS

	For the years ended December 31,		
	2018	2017	2016
Cash flows from operating activities:			
Net loss	$ (56,671,000)	$ (27,319,000)	$(21,873,000)
Adjustments to reconcile net loss to cash used in operating activities:			
Depreciation and amortization	2,360,000	741,000	825,000
Stock-based compensation expense	8,178,000	4,644,000	4,829,000
Restricted stock-based compensation expense	688,000	1,272,000	3,432,000
Non-cash earnings on investments	(626,000)	—	—
Net gain on write-off of licensed technology	—	(127,000)	—
Change in operating assets and liabilities:			
Receivables	26,000	17,000	(9,000)
Prepaid expenses and other current assets	(1,067,000)	(2,580,000)	160,000
Other assets	40,000	(11,000)	(4,000)
Accounts payable and accrued expenses	7,665,000	(1,301,000)	2,819,000
Contingent consideration milestone	—	—	(2,591,000)
Deferred revenue	296,000	2,009,000	(602,000)
Net cash used in operating activities	(39,111,000)	(22,655,000)	(13,014,000)
Cash flows from investing activities:			
Capital expenditures	(9,243,000)	(860,000)	(519,000)
Acquisition of licensed technology	(10,251,000)	—	—
Purchases of short-term investments	(136,092,000)	—	—
Proceeds from maturities of short-term investments	70,500,000	—	—
Net cash used in investing activities	(85,086,000)	(860,000)	(519,000)
Cash flows from financing activities:			
Proceeds from exercise of $5.00 warrants	3,233,000	885,000	—
Proceeds from exercise of stock options	2,244,000	344,000	350,000
Proceeds from $16.00 common stock offering, net of costs	—	86,174,000	—
Proceeds from exercise of $8.00 warrants	—	5,000,000	—
Proceeds from $7.00 common stock offering, net of costs	—	—	41,068,000
Proceeds from $6.44 per share common stock offering, net of costs	—	—	969,000
Proceeds from $2.85 restricted common stock issuance	—	—	150,000
Net cash provided by financing activities	5,477,000	92,403,000	42,537,000
Net (decrease)/increase in cash, cash equivalents and restricted cash	(118,720,000)	68,888,000	29,004,000
Cash, cash equivalents and restricted cash at beginning of year	138,030,000	69,142,000	40,138,000
Cash, cash equivalents and restricted cash at end of year	$ 19,310,000	$138,030,000	$ 69,142,000
Supplemental cash flow information:			
Cash and cash equivalents	$ 18,750,000	$137,750,000	$ 69,142,000
Restricted cash	560,000	280,000	—
Total cash, cash equivalents and restricted cash	$ 19,310,000	$138,030,000	$ 69,142,000
Payable for acquistion of licensed technology	$ 30,000,000	$ —	$ —
Write off of licensed asset and corresponding liability	$ —	$ 4,000,000	$ —
Shares issued to EB Research Partnership and Epidermolysis Bullosa Medical Research Foundation for licenses	$ —	$ —	$ 2,452,000
Cash paid for interest	$ 11,000	$ 8,000	$ 6,000

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

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

NOTE 1 — NATURE OF OPERATIONS AND SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Nature of Operations

Abeona Therapeutics Inc. (together with our subsidiaries, "we," "our," "Abeona" or the "Company"), a Delaware corporation, is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Our lead programs include EB-101, an autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa ("RDEB"), ABO-102, an adeno-associated (AAV)-based gene therapy for Sanfilippo syndrome type A ("MPS IIIA"), and ABO-101 an AAV based gene therapy for Sanfilippo syndrome type B ("MPS IIIB"). We are also developing ABO-201 and ABO-202, which are AAV based gene therapies for the CLN1 and CLN3 forms of Batten Disease, ABO-401 for the treatment of cystic fibrosis, and ABO-5OX for the treatment of retinal diseases. In addition, we are developing a novel vector platform, AIM™ for gene therapy product candidates.

A summary of the significant accounting policies applied in the preparation of the accompanying consolidated financial statements follows:

Principles of Consolidation

The consolidated financial statements include the financial statements of Abeona Therapeutics Inc. and our wholly-owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

Uses and Sources of Liquidity

The financial statements have been prepared on the going concern basis, which assumes the Company will have sufficient cash to pay its operating expenses, as and when they become payable, for a period of at least 12 months from the date the financial report was issued.

As of December 31, 2018, we had cash, cash equivalents and short-term investments of $85.0 million and net assets of $134.0 million. For the year ended December 31, 2018, we had cash outflows from operations of $39.1 million. We believe we have sufficient resources to fund our business operations for the foreseeable future. However, we have implemented a multi-faceted program to secure sufficient liquidity through at least the end of 2020.

This program considers the possibility of accessing additional equity funding from current or new stockholders, out-licensing technology and/or other assets, deferring and/or eliminating planned expenditures, restructuring operations and/or reducing headcount and sales of assets. We believe that we will be able to complete our liquidity program and will have sufficient funding to support planned expenditure commitments and our planned level of growth, and therefore we believe it is appropriate to prepare the financial statements on the going concern basis.

Use of Estimates

The preparation of consolidated financial statements in conformity with accounting principles generally accepted in the United States of America ("U.S. GAAP") requires management to make estimates and assumptions that affect the reported amount of assets and disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of revenue and expenses during the reported period. Actual results could differ from these estimates and assumptions.

Segments

The Company operates in a single segment. The Company's chief operating decision maker, its Executive Chairman, manages the Company's operations on a consolidated basis for the purpose of allocating resources.

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

Cash and Cash Equivalents

We consider all highly liquid investments with a maturity of three months or less when purchased to be cash equivalents. We maintain deposits primarily in financial institutions, which may at times exceed amounts covered by insurance provided by the U.S. Federal Deposit Insurance Corporation ("FDIC"). We have not experienced any losses related to amounts in excess of FDIC limits.

Short-term Investments

Short-term investments consist of investments in U.S. government, U.S. agency and U.S. treasury securities. We determine the appropriate classification of the securities at the time they are acquired and evaluate the appropriateness of such classifications at each balance sheet date. We classify our short-term investments as available-for-sale pursuant to Accounting Standards Codification, or ASC, 320, *Investments — Debt and Equity Securities*. Investments classified as current have maturities of less than one year.

We review our short-term investments for other-than-temporary impairment whenever the fair value of a marketable security is less than the amortized cost and evidence indicates that a short-term investment's carrying amount is not recoverable within a reasonable period of time.

Receivables

Receivables are reported in the consolidated balance sheets at net realizable value. We continually evaluate the creditworthiness of our customers and their financial condition and generally do not require collateral. The allowance for doubtful accounts is based upon reviews of specific customer balances, historic losses, and general economic conditions. As of December 31, 2018 and 2017, no allowance was recorded as all accounts are considered collectible.

Property and Equipment

Property and equipment are recorded at cost. Depreciation is provided using the straight-line method over estimated useful lives ranging from three to seven years for equipment and five to ten years for leasehold improvements. Expenditures for major renewals and betterments that extend the useful lives are capitalized. Expenditures for normal maintenance and repairs are expensed as incurred. The cost of assets sold or abandoned and the related accumulated depreciation are eliminated from the accounts and any gains or losses are recognized in the accompanying consolidated statements of operations of the respective period.

Licensed Technology

We maintain licensed technology on our consolidated balance sheet until either the licensed technology agreement underlying it is completed or the asset becomes impaired. When we determine that an asset has become impaired or we abandon a project, we write down the carrying value of the related intangible asset to its fair value and take an impairment charge in the period in which the impairment occurs.

Licensed technology is amortized over the life of the patent or the agreement and periodically reviewed for impairment.

We test our intangible assets for impairment on an annual basis, or more frequently if indicators are present or changes in circumstance suggest that impairment may exist. Events that could result in an impairment, or trigger an interim impairment assessment, include the receipt of additional clinical or nonclinical data regarding our drug candidate or a potentially competitive drug candidate, changes in the clinical development program for a drug candidate or new information regarding potential sales for the drug. In connection with each annual impairment assessment and any interim impairment assessment, we compare the fair value of the asset as of the date of the assessment with the carrying value of the asset on our consolidated balance sheet.

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

In 2018, 2017 and 2016, we did not impair any licensed technology.

Goodwill

As of December 31, 2018 and 2017, goodwill of $32,466,000 was recorded on the Company's consolidated balance sheet. In accordance with ASC 350, *Intangibles — Goodwill and Other,* goodwill is tested annually for impairment and whenever changes in circumstances occur that would indicate impairment. The Company did not recognize any impairment charges related to goodwill in 2018, 2017 or 2016.

Research and Development Expenses

Research and development costs are expensed as incurred. Research and development expenses include, but are not limited to, payroll and personnel expense, lab supplies, preclinical, development cost, clinical trial expense, outside manufacturing and consulting. The cost of materials and equipment or facilities that are acquired for research and development activities and that have alternative future uses are capitalized when acquired.

General and Administrative Expenses

General and administrative expenses primarily consist of personnel, contract personnel, personnel-related expenses to support our administrative and operating activities, facility costs and professional expenses (i.e., legal expenses) and investor relations fees.

Income Taxes

Income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the consolidated financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date. A valuation allowance is provided for deferred tax assets to the extent their realization is in doubt.

We account for uncertain income tax positions in accordance with ASC 740, *Income Taxes*. Interest costs and penalties related to income taxes are classified as interest expense and general and administrative costs, respectively, in our consolidated financial statements. For 2018, 2017 and 2016, we did not recognize any uncertain tax positions or interest or penalty expense related to income taxes. It is determined not to be reasonably likely for the amounts of unrecognized tax benefits to significantly increase or decrease within the next 12 months. We file U.S. federal and state income tax returns as necessary. The federal return generally has a three-year statute of limitations and most states have a four-year statute of limitations; however, the taxing authorities are allowed to review the tax year in which the net operating loss was generated when the loss is utilized on a tax return. We currently do not have any open income tax audits.

Loss Per Share

We have presented basic loss per share, computed on the basis of the weighted average number of common shares outstanding during the year, and diluted loss per share, computed on the basis of the weighted average number of common shares and all dilutive potential common shares outstanding during the year. Potential common shares result from outstanding stock options and warrants. Common equivalent shares have not been included in the net loss per share calculations for 2018, 2017 or 2016 because the effect of including them would have been anti-dilutive.

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

We did not include the following potentially dilutive securities in the computation of diluted net loss per common share because the securities were anti-dilutive during the periods presented:

	For the years ended December 31,		
	2018	2017	2016
Warrants	1,820,686	2,934,685	3,736,617
Stock options	5,841,805	5,429,727	4,771,560
Total	7,662,491	8,364,412	8,508,177

Stock-Based Compensation

We account for stock-based compensation expense in accordance with ASC 718, *Stock Based Compensation*. We have two stock-based compensation plans under which incentive and qualified stock options and restricted shares could be granted to employees, directors and consultants. Our 2015 Equity Incentive Plan was approved by stockholders on May 7, 2015. As of January 20, 2015, no further grants can be made under our prior plan, the 2005 Equity Incentive Plan. We measure the cost of the employee/director/consultant services received in exchange for an award of equity instruments based on the grant date fair value for the employees and directors and vesting date fair value for consultants of the award. We use the Black-Scholes option pricing model to value our options. We account for forfeitures as they occur.

The following table summarizes stock-based option compensation for 2018, 2017 and 2016, which was allocated as follows:

	For the years ended December 31,		
	2018	2017	2016
Research and development	$3,913,000	$1,668,000	$1,219,000
General and administrative	4,265,000	2,976,000	3,610,000
Stock-based compensation expense included in operating expense	8,178,000	4,644,000	4,829,000
Total stock-based compensation expense	8,178,000	4,644,000	4,829,000
Tax benefit	—	—	—
Stock-based compensation expense, net of tax	$8,178,000	$4,644,000	$4,829,000

The following table summarizes restricted stock-based compensation for 2018, 2017 and 2016, which was allocated as follows:

	For the years ended December 31,		
	2018	2017	2016
Research and development	$ —	$ —	$ 200,000
General and administrative	688,000	1,272,000	3,232,000
Stock-based compensation expense included in operating expense	688,000	1,272,000	3,432,000
Total stock-based compensation expense	688,000	1,272,000	3,432,000
Tax benefit	—	—	—
Stock-based compensation expense, net of tax	$688,000	$1,272,000	$3,432,000

Recent Accounting Pronouncements

In February 2016, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2016-02, *Leases* (ASC 842), which sets out the principles for the recognition, measurement, presentation and disclosure of leases for both parties to a contract (i.e., lessees and lessors).

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. Leases with a term of 12 months or less will be accounted for similar to existing guidance for operating leases today. ASC 842 supersedes the previous leases standard, ASC 840, *Leases*. The standard is effective for public entities for annual and interim periods beginning after December 15, 2018, with early adoption permitted. The Company is in the process of evaluating the impact of this new guidance.

NOTE 2 — NEW ACCOUNTING STANDARDS IMPLMEMENTED

Revenue Recognition

Effective January 1, 2018, we adopted ASU 2014-09, *Revenue from Contracts with Customers*, as amended (ASC 606), using the modified retrospective transition method. The ASC 606 revenue recognition standard replaced the prior revenue recognition standard ASC 605, *Revenue Recognition*. The cumulative effect of applying the ASC 606 standard was an increase of $6,275,000 to stockholders' equity as of January 1, 2018. The balance sheet and the statement of operations as of and for the year ended December 31, 2018 are presented under ASC 606, while our balance sheet as of December 31, 2017 and statement of operations for each of the two years ended December 31, 2017 are presented under ASC 605. See below for disclosure of the effect of the ASC 606 adoption on our consolidated balance sheet as of January 1, 2018 and the impact of the adoption of ASC 606 on our statement of operations for the year ended December 31, 2018.

The table below presents the cumulative effect of the changes made to the consolidated January 1, 2018 balance sheet due to the adoption of ASC 606.

	December 31, 2017, As Reported Under ASC 605	ASC 606 Adjustments	January 1, 2018, As Adjusted Under ASC 606
Liabilities:			
Current liabilities:			
Current portion of deferred revenue	$ 3,214,000	$(3,214,000)	$ —
Total current liabilities	5,607,000	(3,214,000)	2,393,000
Deferred revenue, net of current portion	3,061,000	(3,061,000)	—
Total liabilities	8,668,000	(6,275,000)	2,393,000
Stockholders' equity:			
Accumulated deficit	(359,792,000)	6,275,000	(353,517,000)
Total stockholders' equity	170,098,000	6,275,000	176,373,000

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

The table below presents the impact of the adoption of ASC 606 on our statement of operations.

	For the year ended December 31, 2018		
	Under ASC 605	Effect of ASC 606	As Reported Under ASC 606
Revenues			
Foundation revenues	$ —	$2,796,000	$ 2,796,000
License revenues	602,000	(602,000)	—
Total revenues	804,000	2,194,000	2,998,000
Loss from operations	(60,360,000)	2,194,000	(58,166,000)
Net loss	(58,865,000)	2,194,000	(56,671,000)
Basic and diluted loss per common share	(1.24)	0.05	(1.19)

The table below presents the impact of the adoption of ASC 606 on our balance sheet.

	December 31, 2018		
	Under ASC 605	Effect of ASC 606	As Reported Under ASC 606
Liabilities:			
Current liabilities:			
Current portion of deferred revenue	$ 6,308,000	$(6,012,000)	$ 296,000
Total current liabilities	26,366,000	(6,012,000)	20,354,000
Deferred revenue, net of current portion	2,460,000	(2,460,000)	—
Total liabilities	48,826,000	(8,472,000)	40,354,000
Stockholders' equity:			
Accumulated deficit	(412,382,000)	2,194,000	(410,188,000)
Total stockholders' equity	131,851,000	2,194,000	134,045,000

Foundation revenues: On October 16, 2017, we announced a collaborative agreement with nine Sanfilippo foundations to provide up to approximately $13,850,000 of grants to Abeona in installments for the advancement of the Company's clinical stage gene therapies for MPS IIIA and MPS IIIB, subject to the achievement of certain milestones. As of December 31, 2017, we had received $2,611,000 of these grants and recorded them as deferred revenue on our consolidated balance sheet under ASC 605. There was no foundation revenue recorded in 2017 under ASC 605.

As a result of the adoption of ASC 606, we decreased deferred revenue and the accumulated deficit by $2,611,000 as of January 1, 2018. As of December 31, 2018, we received $5,706,000 of the grants. In accordance with ASC 606, we record revenue to match expenses for the advancement of the Company's clinical stage gene therapies for MPS IIIA and MPS IIIB, the production of which is the primary performance obligation. We recorded foundation revenues of $2,796,000 in 2018. The Company allocated the transaction price based on the expected costs plus a margin approach. The aggregate amount of the transaction price allocated to the performance obligations that are unsatisfied as of December 31, 2018 is $296,000. The Company expects the unsatisfied performance obligations to be recognized in the next 12 months.

License revenues: We received upfront cash payments for licenses of our technology in years 2008 through 2014. Under ASC 605, the initial upfront cash payments were recorded as deferred revenue on our consolidated balance sheet and then recognized as revenue on a straight-line basis over the life of the

patent. As of December 31, 2017, deferred revenues from the licenses were $3,664,000. Under ASC 606 and in accordance with ASU 2016-10, *Revenue from Contracts with Customers (Topic 606): Identifying Performance Obligations and Licensing*, the licenses qualify as functional intellectual property that requires no ongoing performance obligations of the Company after the point the licenses were granted. As a result of the adoption of ASC 606, we decreased deferred revenue and the accumulated deficit by $3,664,000 as of January 1, 2018. Under ASC 605, we would have recorded license revenues of $602,000 in 2018.

Royalty revenues: Royalty revenues are recognized in the period of sales under both ASC 605 and ASC 606. Under ASC 606 and in accordance with ASU 2016-10, this license qualifies as functional intellectual property that requires no ongoing performance obligations of the Company after the point the license was granted. ASC 606 specifies an entity should recognize revenue for a sales-based or usage-based royalty promised in exchange for a license of intellectual property at the subsequent sale or as usage occurs, unless the performance obligation has not been satisfied.

Restricted cash disclosure

In November 2016, the FASB issued ASU 2016-18, *Statement of Cash Flows (Topic 230): Restricted Cash*, requiring restricted cash and restricted cash equivalents to be included with cash and cash equivalents on the statement of cash flows when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. The guidance was effective for interim and annual periods beginning after December 15, 2017, with early adoption permitted. We adopted this standard during the first quarter of 2018. Restricted cash is now included as a component of cash, cash equivalents and restricted cash on our consolidated statements of cash flows. Restricted cash is recorded within other assets and restricted cash in the accompanying consolidated balance sheets. The inclusion of restricted cash increased the ending balance of the consolidated statements of cash flows by $280,000 for the year ended December 31, 2017 and the corresponding beginning balance of the consolidated statements of cash flows by $280,000 for the year ended December 31, 2018. The inclusion of restricted cash increased the ending balance of the consolidated statements of cash flows by $560,000 for the year ended December 31, 2018.

NOTE 3 — SHORT-TERM INVESTMENTS

The following table summarizes the available-for-sale investments held as of December 31, 2018. There were no available-for-sale investments in prior periods.

Description	Fair value
U.S. government and agency securities and treasuries	$66,218,000

The amortized cost of the available-for-sale investments is adjusted for amortization of premiums and accretion of discounts to maturity. There were no material realized gains or losses recognized on the sale or maturity of available-for-sale investments during the year ended December 31, 2018.

NOTE 4 — PROPERTY AND EQUIPMENT

Property and equipment consist of the following:

	December 31, 2018	December 31, 2017
Laboratory equipment	$ 4,366,000	$ 627,000
Furniture and office equipment	1,454,000	449,000
Leasehold improvements	4,774,000	229,000
Construction work-in-progress	409,000	455,000
	11,003,000	1,760,000
Less: accumulated depreciation and amortization	1,560,000	386,000
Property and equipment, net	$ 9,443,000	$ 1,374,000

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

Depreciation and amortization on property and equipment was $1,174,000, $207,000 and $148,000 for 2018, 2017 and 2016, respectively.

NOTE 5 — LICENSED TECHNOLOGY

On November 4, 2018, we entered into a license agreement with REGENXBIO to obtain rights to an exclusive worldwide license (subject to certain non-exclusive rights previously granted for MPS IIIA), with rights to sublicense, to REGENXBIO's NAV AAV9 vector for the development and commercialization of gene therapies for the treatment of MPS IIIA, MPS IIIB, CLN1 Disease and CLN3 Disease. In return for these rights, REGENXBIO will receive a guaranteed $20 million upfront payment, $10 million of which was paid on signing of the agreement on November 4, 2018 and $10 million of which will be paid by November 4, 2019. In addition, REGENXBIO will receive a total of $100 million in annual fees, payable upon the second through sixth anniversaries of the agreement, $20 million of which is guaranteed. REGENXBIO is also eligible to receive potential commercial milestone payments of up to $60 million as well as low double-digit royalties on net sales of products incorporating the licensed intellectual property. The license is amortized over the life of the patent of 8 years.

On August 3, 2016, we announced that we entered into an agreement (the "EB Agreement") with EB Research Partnership ("EBRP") and Epidermolysis Bullosa Medical Research Foundation ("EBMRF") to collaborate on gene therapy treatments for EB. The EB Agreement became effective August 3, 2016, on the execution of two licensing agreements with The Board of Trustees of Leland Stanford Junior University ("Stanford"). We also entered into a license with Stanford for the AAV-based gene therapy EB-201 (AAV DJ COL7A1) technology, and are performing preclinical development and clinical trials of a gene therapy treatment for EB based upon such in-licensed technology. EB-201 (AAV DJ COL7A1) is a preclinical candidate targeting a novel, AAV-mediated gene editing and delivery approach (known as homologous recombination) to correct gene mutations in skin cells (keratinocytes) for patients with RDEB. The licenses are amortized over the life of the license of 20 years.

On May 15, 2015, we acquired Abeona Therapeutics LLC, which had an exclusive license through Nationwide Children's Hospital to the AB-101 and AB-102 patent portfolios for developing treatments for patients with Sanfilippo Syndrome Type A and Type B. The license is amortized over the life of the license of 20 years.

On September 22, 2014, we entered into an exclusive, worldwide, licensing agreement with Plasma Technologies LLC ("PlasmaTech") to obtain rights to utilize and to sub-license to other pharmaceuticals firms its patented methods for the extraction of therapeutic biologics from human plasma. The license was to be amortized over the life of the patent of 11 years. Under the terms of the licensing agreement, as amended on January 23, 2015, we paid a license fee of $1 million in cash. In December 2017, the agreement was terminated and the technology was returned to PlasmaTech.

Licensed technology consists of the following:

	December 31, 2018	December 31, 2017
Licensed technology	$44,859,000	$ 4,608,000
Less accumulated amortization	1,817,000	631,000
Licensed technology, net	$43,042,000	$ 3,977,000

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

The aggregate estimated amortization expense for intangible assets remaining as of December 31, 2018 is as follows (in thousands):

2019	$ 5,378,000
2020	5,378,000
2021	5,378,000
2022	5,378,000
2023	5,378,000
Thereafter	16,152,000
Total	$43,042,000

Amortization on licensed technology was $1,186,000, $534,000 and $677,000 for the years ended December 31, 2018, 2017 and 2016, respectively.

NOTE 6 — PAYABLE TO LICENSOR

Under the terms of the license agreement with REGENXBIO, we are obligated to pay $10 million to REGENXBIO by November 4, 2019. This amount is recorded as current portion of payable to licensor on the consolidated balance sheet. In addition, REGENXBIO will receive a total of $100 million in annual fees, payable upon the second through sixth anniversaries of the agreement, $20 million of which is guaranteed. The guaranteed amount of $20 million is recorded as payable to licensor on the consolidated balance sheet. Refer to Note 5 for further information about the license agreement with REGENXBIO.

NOTE 7 — 401(k) PLAN

We have a tax-qualified employee savings and retirement plan (the "401(k) Plan") covering all our employees. Pursuant to the 401(k) Plan, employees may elect to reduce their current compensation by up to the statutorily prescribed annual limit ($18,500 in 2018 and $18,000 in both 2017 and 2016) and to have the amount of such reduction contributed to the 401(k) Plan. The 401(k) Plan is intended to qualify under Section 401 of the Internal Revenue Code so that contributions by employees or by us to the 401(k) Plan, and income earned on 401(k) Plan contributions, are not taxable to employees until withdrawn from the 401(k) Plan, and so that contributions by us, if any, will be deductible by us when made. At the direction of each participant, we invest the assets of the 401(k) Plan in any of over 50 investment options. Company contributions under the 401(k) Plan were $317,000, $0 and $0 in 2018, 2017 and 2016, respectively.

NOTE 8 — COMMITMENTS AND CONTINGENCIES

Operating Leases

At December 31, 2018, we had operating leases for our manufacturing and laboratory facilities and administrative offices in Cleveland that expire on December 31, 2025 and total $5,012,000. We have the option to extend the leases for an additional five years. We can also terminate the lease early at December 31, 2020, at the end of year five, and pay for unamortized tenant improvements. Our total lease costs and unamortized tenant improvements would total $2,205,000 by exercising the early termination provision.

At December 31, 2018, we also had an operating lease for our New York office until January 31, 2026 totaling $7,145,000, an operating lease for our Spain office until September 31, 2019 totaling $21,000 and an operating lease for our Dallas office until August 31, 2019 totaling $19,000.

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

Future operating lease payments are as follows:

2019	$ 1,735,000
2020	1,708,000
2021	1,722,000
2022	1,736,000
2023	1,751,000
Thereafter	3,546,000
Total	$12,198,000

Rent expense for the years ended December 31, 2018, 2017 and 2016 was $1,297,000, $742,000 and $548,000, respectively.

Manufacturing

We have engaged a contract manufacturer to assist us with developing and defining the processes necessary to manufacture our MPS IIIA and RDEB products. We had a remaining commitment of $15 million at December 31, 2018. The amounts are payable based on the completion of specific activities outlined in the contracted project plan; we expect to spend $10.1 million in 2019 and $4.9 million in 2020.

Legal

We are not currently subject to any material pending legal proceedings as of December 31, 2018.

NOTE 9 — FAIR VALUE MEASUREMENTS

We calculate the fair value of our assets and liabilities that qualify as financial instruments and include additional information in the notes to the consolidated financial statements when the fair value is different than the carrying value of these financial instruments. The estimated fair value of receivables, prepaid expenses, other assets, accounts payable, accrued expenses, payable to licensor and deferred revenue approximate their carrying amounts due to the relatively short maturity of these instruments.

U.S. GAAP defines fair value as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants at the measurement date. This guidance establishes a three-level fair value hierarchy that prioritizes the inputs used to measure fair value. The hierarchy requires entities to maximize the use of observable inputs and minimize the use of unobservable inputs. The three levels of inputs used to measure fair value are as follows:

- Level 1 — Quoted prices in active markets for identical assets or liabilities.
- Level 2 — Observable inputs other than quoted prices included in Level 1, such as quoted prices for similar assets and liabilities in active markets; quoted prices for identical or similar assets and liabilities in markets that are not active; or other inputs that are observable or can be corroborated by observable market data.
- Level 3 — Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets and liabilities. This includes certain pricing models, discounted cash flow methodologies and similar valuation techniques that use significant unobservable inputs.

The guidance requires an entity to maximize the use of observable inputs and minimize the use of unobservable inputs when measuring fair value.

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

We have segregated all financial assets and liabilities that are measured at fair value on a recurring basis (at least annually) into the most appropriate level within the fair value hierarchy based on the inputs used to determine the fair value at the measurement date in the table below.

Financial assets and liabilities measured at fair value on a recurring and non-recurring basis as of December 31, 2018 and December 31, 2017 are summarized below:

Description	December 31, 2018	Level 1	Level 2	Level 3	Total Gains/(Losses)
Recurring					
Assets:					
Short-term investments	$66,218,000	$—	$66,218,000	$ —	$—
Non-recurring					
Assets:					
Licensed technology, net	$43,042,000	$—	$ —	$43,042,000	$—
Goodwill	32,466,000	—	—	32,466,000	—

Description	December 31, 2017	Level 1	Level 2	Level 3	Total Gains/(Losses)
Non-recurring					
Assets:					
Licensed technology, net	$ 3,977,000	$—	$—	$ 3,977,000	$127,000
Goodwill	32,466,000	—	—	32,466,000	—

NOTE 10 — STOCKHOLDERS' EQUITY

2017 Public Offering

On October 19, 2017, we closed an underwritten public offering of 5,750,000 shares of common stock, at a public offering price of $16.00 per share. The gross proceeds to the Company were approximately $92,000,000 before deducting the underwriting discounts and commissions and estimated offering expenses payable by the Company.

2016 Public Offering

On November 1, 2016, we closed an underwritten public offering of 6,000,000 shares of common stock, at a public offering price of $7.00 per share. On November 23, 2016, we closed a follow-on offering of 293,889 shares as permitted by the underwriting agreement at the same offering price of $7.00 per share. The gross proceeds to the Company were $44,057,000 before deducting the underwriting discounts and commissions and estimated offering expenses payable by the Company.

Warrants

There were warrants to purchase a total of 1,820,686 and 2,934,685 shares of common stock outstanding at December 31, 2018 and 2017, respectively. All warrants are exercisable at December 31, 2018.

We received cash from the exercise of warrants of $3,233,000, $5,885,000 and $0 during 2018, 2017 and 2016, respectively.

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

The warrants had various exercise prices and terms as follows as of December 31, 2018:

Summary of Warrants	Warrants Outstanding	Exercise Price	Expiration Date
2015 Financing[a]	20,000	$ 6.05	July 31, 2020
2015 Financing[b]	50,000	11.00	May 11, 2020
2014 Financing[c]	1,749,186	5.00	December 24, 2019
2014 Financing[c]	1,500	5.00	December 18, 2019
2012 Series B private placement[d]	—	25.00	October 24, 2018
Total	1,820,686		

a) In connection with the offering on July 31, 2015, the placement agent received warrants to purchase 20,000 shares of common stock at $6.05 per share. The warrants are exercisable and expire on July 31, 2020.

b) In connection with the offering on May 11, 2015, the placement agent received warrants to purchase 50,000 shares of common stock at $11.00 per share. The warrants are exercisable and expire on May 11, 2020.

c) In connection with an offering on December 24, 2014, warrants to purchase 3,500,000 shares of common stock at $5.00 per share were purchased and issued for $0.01 per warrant. The warrants are exercisable and expire on December 24, 2019. Also, in connection with the offering on December 24, 2014, the underwriter received warrants to purchase 68,735 shares of common stock at $5.00 per share. The warrants are exercisable and expire on December 18, 2019.

d) In connection with a private placement offering on October 25, 2012, warrants to purchase 400,001 shares of common stock at $25.00 per share were issued. The warrants expired on October 24, 2018.

NOTE 11 — STOCK OPTION PLANS

Our stock-based employee compensation plans are described below:

2015 Equity Incentive Plan

Under our 2015 Equity Incentive Plan, as amended, up to 10,000,000 shares of our authorized but unissued common stock are reserved for issuance to employees, consultants, or to non-employee members of the Board or to any member of the board of directors (or similar governing authority) of any affiliate of the Company. The 2015 Equity Incentive Plan, approved by our stockholders on May 7, 2015, replaced the previously approved stock option plan (the "2005 Equity Incentive Plan"). The maximum contractual term of awards is generally 10 years.

We estimate the fair value of each option award on the date of grant using the Black-Scholes option valuation model. We then recognize the grant date fair value of each option as compensation expense ratably using the straight-line attribution method over the service period (generally the vesting period). The Black-Scholes model incorporates the following assumptions:

- Expected volatility — we estimate the volatility of our share price at the date of grant using a "look-back" period which coincided with the expected term, defined below. We believe using a "look-back" period which coincides with the expected term is the most appropriate measure for determining expected volatility.
- Expected term — we estimate the expected term using the "simplified" method, as outlined in Staff Accounting Bulletin No. 107, "Share-Based Payment."

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

- Risk-free interest rate — we estimate the risk-free interest rate using the U.S. Treasury yield curve for periods equal to the expected term of the options in effect at the time of grant.
- Dividends — we use an expected dividend yield of zero because we have not declared or paid a cash dividend, nor do we have any plans to declare a dividend.

We used the following weighted-average assumptions to estimate the fair value of the options granted for the years indicated:

	2018	2017	2016
Expected volatility	109%	111%	109%
Expected term	5 years	5 years	5 years
Risk-free interest rate	2.62%	1.81%	1.10%
Expected dividend yield	0.00	0.00%	0.00%

Summarized information for the 2015 Equity Incentive Plan is as follows:

	Options	Weighted-average exercise price
Outstanding options at January 1, 2016	1,994,000	$ 6.90
Granted, fair value of $2.59 per share	2,622,500	3.29
Exercised	(151,000)	2.32
Expired/forfeited	(22,500)	3.23
Outstanding options at December 31, 2016	4,443,000	$ 4.94
Granted, fair value of $11.35 per share	1,077,000	$ 14.31
Exercised	(81,719)	4.22
Expired/forfeited	(325,314)	5.52
Outstanding options at December 31, 2017	5,112,967	$ 9.07
Granted, fair value of $10.23 per share	1,497,300	$ 12.95
Exercised	(360,853)	6.22
Expired/forfeited	(724,009)	10.65
Outstanding options at December 31, 2018	5,525,405	$ 8.08
Non-vested options at December 31, 2016	3,115,313	$ 4.24
Non-vested options at December 31, 2017	2,641,832	8.21
Non-vested options at December 31, 2018	2,355,719	10.41

The intrinsic value related to the outstanding options under this plan was $8,398,000, $45,988,000 and $3,806,000 at December 31, 2018, 2017 and 2016, respectively. The intrinsic value related to the exercisable options under this plan was $6,026,000, $25,614,000 and $417,000 at December 31, 2018, 2017 and 2016, respectively.

The total intrinsic value of the options exercised was $3,182,000, $641,000 and $450,000 during 2018, 2017 and 2016, respectively.

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

Further information regarding options outstanding under the 2015 Equity Incentive Plan at December 31, 2018 is summarized below:

Range of exercise prices		Number of options outstanding	Weighted-average		Number of options exercisable	Weighted-average	
			Remaining life in years	Exercise price		Remaining life in years	Exercise price
$ 2.31	$ 4.45	2,252,855	6.7	$ 3.51	1,593,792	6.3	$ 3.42
5.90	9.23	1,645,800	6.5	7.46	1,191,561	5.4	7.25
11.43	14.45	591,700	9.5	13.20	50,000	9.2	13.65
15.25	17.30	1,035,050	9.0	16.09	334,333	8.9	16.04
		5,525,405			3,169,686		

At December 31, 2018, the total compensation cost related to non-vested options not recognized is $17,261,000. The expected weighted average period over which the total compensation costs related to non-vested options is 2.1 years.

2005 Equity Incentive Plan

Under the 2005 Equity Incentive Plan, as amended, shares of our authorized but unissued common stock were reserved for issuance to employees, consultants, or to non-employee members of the Board or to any member of the board of directors (or similar governing authority) of any affiliate of the Company. As of January 20, 2015, no additional shares were available for grant under the 2005 Equity Incentive Plan. A total of 316,400 options were outstanding and exercisable under this plan at December 31, 2018.

Summarized information for the 2005 Equity Incentive Plan is as follows:

	Options	Weighted-average exercise price
Outstanding options at January 1, 2016	330,084	$ 13.49
Expired/forfeited	(1,524)	36.93
Outstanding options at December 31, 2016	328,560	$ 14.57
Expired/forfeited	(11,800)	21.53
Outstanding options at December 31, 2017	316,760	$ 14.31
Expired/forfeited	(360)	150.00
Outstanding options at December 31, 2018	316,400	$ 14.15

The intrinsic value related to the outstanding or exercisable options under this plan was $466,000, $1,529,000 and $192,000 at December 31, 2018, 2017 and 2016, respectively.

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

Further information regarding options outstanding under the 2005 Equity Incentive Plan at December 31, 2018 is summarized below:

Range of exercise prices		Number of options outstanding	Weighted-average Remaining life in years	Weighted-average Exercise price	Number of options exercisable	Weighted-average Remaining life in years	Weighted-average Exercise price
$ 3.25	$ 3.25	120,000	1.1	$ 3.25	120,000	1.1	$ 3.25
11.50	18.50	189,000	3.9	18.35	189,000	3.9	18.35
30.50	122.50	7,400	2.1	83.72	7,400	2.1	83.72
		316,400			316,400		

NOTE 12 — INCOME TAXES

Income tax expense differs from the statutory amounts for each of the following years:

	2018	2017	2016
Income taxes at U.S. statutory rate	$(11,901,000)	$ (9,289,000)	$(7,437,000)
Current year reserve	11,877,000	(25,175,000)	7,423,000
Expenses not deductible	24,000	46,000	14,000
Rate change	—	34,418,000	—
Total tax expense	$ —	$ —	$ —

Deferred taxes are provided for the temporary differences between the financial reporting bases and the tax bases of our assets and liabilities. The temporary differences that give rise to deferred tax assets and liabilities were as follows:

	December 31, 2018	December 31, 2017
Deferred tax assets (liabilities):		
Net operating loss carryforwards	$ 38,761,000	$ 50,029,000
General business credit carryforwards	3,214,000	3,227,000
State credits	3,012,000	3,089,000
Property, equipment and goodwill	(72,000)	(28,000)
Stock options	6,316,000	5,122,000
Derivatives	(57,000)	(57,000)
Deferred revenue	62,000	778,000
Intangible assets	399,000	379,000
Accrued interest	0	156,000
Other	80,000	143,000
Gross deferred tax assets	51,715,000	62,838,000
Valuation allowance	(51,715,000)	(62,838,000)
Net deferred taxes	$ —	$ —

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

At December 31, 2018, we had approximately $184.6 million of net operating loss carryforwards and approximately $3.2 million of general business credit carryforwards. These carryforwards expire as follows:

	Net operating loss carryforwards	General business credit carryforwards
2019	$ 3,306,000	$ 95,000
2020	5,125,000	226,000
2021	5,378,000	56,000
2022	8,230,000	431,000
Thereafter	162,537,000	2,406,000
	$ 184,576,000	$ 3,214,000

We acquired MacroChem Corporation on February 25, 2009 and Somanta Pharmaceuticals, Inc. on January 4, 2008. Both of these corporations were loss-making entities at the time of acquisition. As a result, the net operating losses related to those acquisitions may be subject to annual limitations as well.

On December 22, 2017, President Donald Trump signed the Tax Cuts and Jobs Act (the "Act"). The Act reduces the U.S. federal corporate tax rate from 34% to 21%. At December 31, 2017, we recalculated our deferred tax assets and liabilities based on the rates at which they are expected to reverse in the future, which is generally 21%, and recorded a provisional adjustment of $34,418,000. In 2018, the accounting for the income tax enactment-date effects of the Act were completed and there was no adjustment to the provisional amount recorded in 2017.

NOTE 13 — QUARTERLY FINANCIAL DATA (UNAUDITED)

The following table contains quarterly financial information for 2018 and 2017. We believe that the following information reflects all normal recurring adjustments necessary for a fair statement of the information for the periods presented. The operating results for any quarter are not necessarily indicative of results for any future period.

	Quarters Ended			
	December 31, 2018	September 30, 2018	June 30, 2018	March 31, 2018
Foundation revenues[1][2]	$ 369,000	$ 1,687,000	$ 259,000	$ 481,000
License revenues[1]	—	—	—	—
Royalties[1]	113,000	22,000	17,000	50,000
Research and development	9,470,000	13,150,000	7,916,000	8,162,000
General and administrative	7,631,000	4,970,000	4,627,000	2,878,000
Loss from operations[2]	(18,010,000)	(16,916,000)	(12,557,000)	(10,683,000)
Net loss[2]	(17,479,000)	(16,419,000)	(12,243,000)	(10,530,000)
Basic and diluted loss per common share	(0.36)	(0.34)	(0.26)	(0.22)
Weighted average number of common shares outstanding – basic and diluted	47,944,486	47,794,394	47,303,518	47,060,523

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
Three years ended December 31, 2018

	Quarters Ended			
	December 31, 2017	September 30, 2017	June 30, 2017	March 31, 2017
Foundation revenues[1]	$ —	$ —	$ —	$ —
License revenues[1]	150,000	151,000	150,000	151,000
Royalties[1]	65,000	68,000	67,000	35,000
Research and development	5,706,000	3,277,000	5,808,000	2,198,000
General and administrative	3,113,000	2,166,000	2,642,000	3,022,000
Loss from operations	(8,750,000)	(5,362,000)	(8,440,000)	(5,284,000)
Net loss	(8,450,000)	(5,343,000)	(8,279,000)	(5,247,000)
Basic and diluted loss per common share	(0.19)	(0.13)	(0.21)	(0.13)
Weighted average number of common shares outstanding – basic and diluted	45,598,667	40,377,890	40,270,879	40,254,679

(1) Effective January 1, 2018, we adopted ASU 2014-09, *Revenue from Contracts with Customers*, as amended (ASC 606)), using the modified retrospective transition method. The ASC 606 revenue recognition standard replaced the prior revenue recognition standard ASC 605, *Revenue Recognition*. The statement of operations for each of the quarterly periods in 2018 are presented under ASC 606, while the statement of operations for each of the quarterly periods of 2017 are presented under ASC 605.

(2) We have adjusted the amounts originally reported for the quarters ended March 31, 2018 and June 30, 2018 to correct for an error in the determination of the cumulative effect related to the adoption of ASC 606 as of January 1, 2018. The adjusted amounts for March 31, 2018 reflect a $2,067,000 reduction in foundation revenues and corresponding increases in the loss from operations and net loss of $2,067,000 and an increase in the diluted loss per share of $0.04, as compared to the originally reported amounts. The adjusted amounts for June 30, 2018 reflect a $543,000 reduction in foundation revenues and corresponding increases in the loss from operations and net loss of $543,000 and an increase in the diluted loss per share of $0.01, as compared to the originally reported amounts.

Exhibit 3.2

AMENDED RESTATED BYLAWS
OF
ABEONA THERAPEUTICS INC.

(As amended and restated as of March 14, 2019)

ARTICLE I.

Offices and Agents

1 . Principal Office. The principal office of the Corporation may be located within or without the State of Delaware, as designated by the board of directors. The Corporation may have other offices and places of business at such places within or without the State of Delaware as shall be determined by the directors.

2 . Registered Office. The registered office of the Corporation required by the General Corporation Law of Delaware must be maintained in the State of Delaware, and it may be, but need not be, identical with the principal office, if located in the state of Delaware. The address of the registered office of the Corporation may be changed from time to time as provided by the General Corporation Law of Delaware.

3 . Registered Agent. The Corporation shall maintain a registered agent in the State of Delaware as required by the General Corporation Law of Delaware. Such registered agent may be changed from time to time as provided by the General Corporation Law of Delaware.

ARTICLE II.

Stockholders Meetings

1 . Annual Meetings. Unless otherwise determined by the board of directors, the annual meeting of the stockholders of the Corporation shall be held at a reasonable hour on the second Wednesday of May unless that day be a holiday, in which case said meeting shall be held on the next business day following that day. The annual meeting of the stockholders shall be held for the purpose of electing directors and transacting such other corporate business as may come before the meeting.

2 . Special Meetings. Special meetings of the stockholders of the Corporation may be called at any time by the chairman of the board of directors, if any, by the president or by resolution of the board of directors. The notice or call of a special meeting shall state the purpose or purposes for which the meeting is called.

3 . Place of Meeting. The annual meeting of the stockholders of the Corporation may be held at any place, either within or without the State of Delaware, as may be designated by the board of directors. Except as limited by the following sentence, the person or persons calling any special meeting of the stockholders may designate any place, within or without the State of Delaware, as the place for the meeting. If no designation is made or if a special meeting shall be called other than by the board of directors, the chairman of the board of directors or the president, the place of meeting shall be the principal office of the Corporation. A waiver of notice signed by all stockholders entitled to vote at a meeting may designate any place for such meeting.

4. Notice of Meeting. Except as otherwise provided in these Bylaws or by the laws of the State of Delaware, written or printed notice stating the place, date and hour of the meeting and, in the case of a special meeting, the purpose or purposes for which the meeting is called, shall be delivered either personally or by mail to each stockholder of record entitled to vote at such meeting not less than ten (10) nor more than sixty (60) days before the date of the meeting. If mailed, such notice shall be deemed to be delivered when deposited in the United States mail, postage prepaid, directed to the stockholder at his address as it appears on the records of the Corporation. An affidavit of the secretary, assistant secretary, if any, or transfer agent of the Corporation that notice has been given shall, in the absence of fraud, be prima facie evidence of the facts stated therein.

5. Waiver of Notice. Any stockholder, either before, at, or after any stockholders' meeting, may waive notice of the meeting, and his waiver shall be deemed the equivalent of giving notice. Attendance at a stockholders' meeting, either in person or by proxy, by a person entitled to notice thereof shall constitute a waiver of notice of the meeting unless he attends for the express purpose of objecting, at the beginning of the meeting, to the transaction of any business on the ground that the meeting was not lawfully called or convened.

6. Fixing of Record Date. For the purpose of determining stockholders entitled to notice of or to vote at any meeting of stockholders or any adjournment thereof, to express consent to corporate action in writing without a meeting, or entitled to receive payment of any dividend or other distribution or allotment of any rights, or entitled to exercise any rights in respect of any change, conversion or exchange of stock, or for the purpose of any other lawful action, the board of directors of the Corporation may fix, in advance, a record date, which shall not be more than sixty (60) nor less than ten (10) days before the date of the meeting; not more than ten (10) days after the record date for determining shareholders entitled to express consent is fixed; and not more than sixty (60) days prior to the date of any other action. If no record date is fixed: (i) the record date for determining stockholders entitled to notice of or to vote at a meeting of stockholders shall be the close of business on the day next preceding the day on which notice is given, or, if notice is waived, at the close of business on the day next preceding the day on which the meeting was held; (ii) the record date for determining stockholders entitled to express consent to corporate action in writing without a meeting, when no prior action by the board of directors is necessary, shall be the day on which the first written consent is delivered to the Corporation at its principal place of business or such other place as designated by the boards of directors; (iii) the record date for determining stockholders for any other purpose shall be at the close of business on the day on which the board of directors adopts the resolution relating thereto. A determination of stockholders entitled to notice of or to vote at a meeting of stockholders shall apply to any adjournment of the meeting, provided, however, that the board of directors may fix a new record date for the adjourned meeting.

7 . Voting List. The officer or agent who has charge of the stock ledger of the Corporation shall prepare and make, at least ten (10) days before every meeting of stockholders, a complete list of the stockholders entitled to vote at the meeting, or any adjournment thereof, arranged in alphabetical order, showing the address of and the number of shares registered in the name of each stockholder. Such list shall be open to the examination of any stockholder for any purpose germane to the meeting, during ordinary business hours, for a period of at least ten (10) days prior to the meeting, either at a place within the city where the meeting is to be held, which place shall be specified in the notice of the meeting or, if not so specified, at the place where the meeting is to be held. The list shall also be produced and kept at the time and place of the meeting during the whole time thereof, and may be inspected by any stockholder who is present. The stock ledger shall be the only evidence as to who are the stockholders entitled to examine the stock ledger, the books of the Corporation or to vote in person or by proxy at any meeting of stockholders.

8 . Polls. The date and time of the opening and closing of the polls for each matter upon which the stockholders will vote at a meeting shall be announced at the meeting. No ballot, proxies or votes, nor any revocations thereof or changes thereto, shall be accepted by the inspectors after the closing of the polls unless the Court of Chancery upon application by a stockholder shall determine otherwise.

9 . Proxies. Any stockholder entitled to vote at a meeting of the stockholders, or to express consent or dissent to corporate action in writing without meeting may authorize another person or persons to act for him by proxy. No proxy shall be voted or acted upon after three (3) years from the date of its execution unless the proxy expressly provides for a longer period. A duly executed proxy shall be irrevocable if it states that it is irrevocable and if, and only as long as, it is coupled with an interest sufficient in law to support an irrevocable power. A proxy may be irrevocable regardless of whether the interest with which it is coupled is an interest in the stock itself or an interest in the Corporation generally.

Without limiting the manner in which a stockholder may authorize another person or persons to act for him by proxy, the following shall constitute a valid means by which a stockholder may grant such authority.

A stockholder may execute a writing authorizing another person or persons to act for him as proxy. Execution may be accomplished by the stockholder or his authorized officer, director, employee or agent signing such writing or causing his signature to be affixed to such writing by any reasonable means including but not limited to, by facsimile signature.

A stockholder may authorize another person or persons to act for him as proxy by transmitting or authorizing the transmission of a telegram, cablegram or other means of electronic transmission to the person who will be the holder of the proxy or to a proxy solicitation firm, proxy support service organization or like agent duly authorized by the person who will be the holder of the proxy to receive such transmission, provided that any such telegram, cablegram or other electronic transmission must either set forth or be submitted with information from which it can be determined that the telegram, cablegram or other electronic transmission was authorized by the stockholder. If it is determined that such telegrams, cablegrams or other electronic transmission are valid, the inspectors or, if there are no inspectors, such other persons making that determination shall specify the information upon which they relied.

Any copy facsimile telecommunication or other reliable reproduction of the writing or transmission created pursuant to this Paragraph 9 may be substituted or used in lieu of the original writing or transmission for any and all purposes for which the original writing or transmission could be used, provided that such copy, facsimile telecommunication or other reproduction shall be a complete reproduction of the entire original writing or transmission.

10. Voting Rights. Each outstanding share, regardless of class, shall be entitled to one vote, and each fractional share shall be entitled to a corresponding fractional vote on each matter submitted to a vote at a meeting of stockholders except to the extent that the voting rights of the shares of any class or classes are limited or denied by the Certificate of Incorporation.

At each election for directors every stockholder entitled to vote at such election shall have the right to vote in person or by proxy the number of shares owned by him for as many persons as there are directors to be elected and for whose election he has a right to vote, and cumulative voting in the election of such directors shall be permitted.

Persons holding stock in a fiduciary capacity shall be entitled to vote the shares so held. Persons whose stock is pledged shall be entitled to vote, unless in the transfer by the pledgor on the books of the Corporation he has expressly empowered the pledgee to vote thereon, in which case only the pledgee, or his proxy, may represent such stock and vote thereon.

The Corporation's own capital stock belonging to the Corporation or to another corporation, if a majority of the shares entitled to vote in the election or directors of such other corporation is held, directly or indirectly, by the Corporation, shall neither be entitled to vote nor be counted for quorum purposes. Nothing in this section shall be construed as limiting the right of the Corporation to vote stock, including but not limited to its own stock, held by it in a fiduciary capacity.

Shares which have been called for redemption shall not be deemed to be outstanding shares for the purpose of voting or determining the total number of shares entitled to vote on any matter on and after the date on which written notice of redemption has been sent to holders thereof and a sum sufficient to redeem such shares has been irrevocably deposited or set aside to pay the redemption price to the holders of the shares upon surrender of certificates there for.

If shares or other securities having voting power stand of record in the names of two (2) or more persons, whether fiduciaries, members of a partnership, joint tenants, tenants in common, tenants by the entirety or otherwise, or if two (2) or more persons have the same fiduciary relationship respecting the same shares, unless the secretary of the Corporation is given written notice to the contrary and is furnished with a copy of the instrument or order appointing them or creating the relationship wherein it is so provided, their acts with respect to voting shall have the following effect: (i) if only one (1) votes, his act binds all; (ii) if more than one (1) votes, the act of the majority so voting binds all; (iii) if more than one (1) votes, but the vote is evenly split on any particular matter each faction may vote the securities in question proportionally, or any person voting the shares, or a beneficiary, if any, may apply to the Court of Chancery or such other court as may have jurisdiction to appoint an additional person to act with the persons so voting the shares, which shall then be voted as determined by a majority of such persons and the person appointed by the Court. If the instrument so filed shows that any such tenancy is held in unequal interests, a majority or even split for the purpose of this subsection shall be a majority or even split in interest.

11. Inspectors or Election. Prior to holding any meeting of stockholders, the Corporation shall appoint one or more inspectors to act at the meeting and make a written report thereof. The Corporation may designate one or more persons as alternate inspectors to replace any inspector who fails to act. If no inspector or alternate is able to act at a meeting of stockholders, the person presiding at the meeting shall appoint one or more inspectors to act at the meeting. Each inspector, before entering upon the discharge of his duties, shall take and sign an oath faithfully to execute the duties of inspector with strict impartiality and according to the best of his ability.

The inspectors shall (i) ascertain the number of shares outstanding and the voting power of each; (ii) determine the shares represented at a meeting and the validity of proxies and ballots; (iii) count all votes and ballots: (iv) determine and retain for a reasonable period a record of the disposition of any challenges made to any determination by the inspectors, and (v) certify their determination of the number of shares represented at the meeting and their count of all votes and ballots. The inspectors may appoint or retain other persons or entities to assist the inspectors in the performance of the duties of the inspectors.

In determining the validity and counting of proxies and ballots, the inspectors shall be limited to an examination of the proxies, any envelopes submitted with those proxies, any information provided in accordance with Article II, Paragraph 9 of these Bylaws, any records of the Corporation, except that the inspectors may consider other reliable information for the limited purpose of reconciling proxies and ballots submitted by or on behalf of banks, brokers, their nominees or similar persons which represent more votes than the holder of a proxy is authorized by the record owner to cast or more votes than the stockholder holds of record. If the inspectors consider other reliable information for the limited purpose permitted herein, the inspectors at the time they make their certification shall specify the precise information considered by them including the person or persons from whom they obtained the information, when the information was obtained, the means by which the information was obtained and the basis for the inspectors' belief that such information is accurate and reliable.

12. Quorum. Except as otherwise provided in the Certificate of Incorporation, the presence, in person or by proxy, of the holders of a majority of the shares outstanding and entitled to vote shall constitute a quorum at meetings of the stockholders. In all matters, other than the election of directors, the affirmative vote of a majority of the shares present in person or represented by proxy at the meeting and actually voting on the subject matter shall be the act of the stockholders. Directors shall be elected by a plurality of the votes of the shares present in person or represented by proxy at the meeting and entitled to vote on the election of directors. In the event any stockholders withdraw from a duly organized meeting at which a quorum was initially present, the remaining shares represented shall constitute a quorum for the purpose of continuing to do business, and the affirmative vote of the majority of the remaining shares represented at the meeting and entitled to vote on the subject matter shall be the act of the stockholders unless the vote of a greater number or voting by classes is required by the General Corporation Law of Delaware or the Certificate of Incorporation.

13. Adjournments. If less than a quorum of the outstanding shares entitled to vote is represented at any meeting of the stockholders, a majority of the shares so represented may adjourn the meeting from time to time for a period not to exceed thirty (30) days at any one adjournment, without further notice, provided the time and place thereof are announced at the meeting at which the adjournment is taken. At the adjourned meeting, the Corporation may transact any business which might have been transacted at the original meeting. Any meeting of the stockholders may adjourn from time to time until its business is completed. If the adjournment is for more than thirty (30) days, or if after the adjournment a new record date is fixed for the adjourned meeting, a notice of the adjourned meeting shall be given to each stockholder of record entitled to vote at the meeting.

14. Informal Act by Shareholders. Any action required to be taken at a meeting of shareholders, or any action which may be taken at a meeting of shareholders, may be taken without a meeting, without prior notice and without a vote, if a consent or consents in writing setting forth the action so taken, shall be signed by the holders of outstanding stock having not less than the minimum number of votes that would be necessary to authorize or take such action at a meeting at which all shares entitled to vote thereon were present and voted shall be delivered to the Corporation by said consent or consents delivered at its principal place of business or such other place as designated by the board of directors. Delivery made to the Corporation shall be by hand or by certified or registered mail, return receipt requested.

ARTICLE III.

Board of Directors

1. Number, Qualifications and Term of Office. Except as otherwise provided in the Certificate of Incorporation or the General Corporation Law of Delaware, the business and affairs of the Corporation shall be managed under the direction of a board of directors consisting of from three to fifteen members. Each director shall be a natural person of the age of fifteen years or older, but does not need to be a resident of the state of Delaware or a stockholder of the Corporation. The board of directors, by resolution, may increase or decrease the number of directors from time to time. Except as otherwise provided in these Bylaws or in the Certificate of Incorporation, the board of directors shall be divided into three (3) classes as nearly equal in number as possible. Each director in each class shall be elected at the appropriate annual meeting of stockholders, as determined by the Certificate of Incorporation, and shall hold office for a term of three (3) years and until his successor is elected and qualified or until his earlier resignation or removal. No decrease in the number of directors shall have the effect of shortening the term of any incumbent director.

2. Vacancies and Newly Created Directorships. Vacancies and newly created directorships resulting from any increase in the authorized number of directors elected by all of the stockholders having the right to vote as a single class shall be filled solely by a majority of the directors then in office, although less than a quorum, or by a sole remaining director. Any directors so chosen shall hold office until the next election of the class for which such director shall have been chosen, and until their successors shall be elected and qualified. No decrease in the number of directors constituting the board of directors shall shorten the term of any incumbent director.

If at any time of filling any vacancy or newly created directorship, the directors then in office shall constitute less than a majority of the whole board, the Court of Chancery may, upon application of any stockholder or stockholders holding at least ten percent (10%) of the total number of shares at the time outstanding having the right to vote for such directors, summarily order an election to be held to fill any such vacancies or newly created directorships, or to replace the directors chosen by the directors then in office as aforesaid, which election shall be governed by Section 211 of the General Corporation Law of Delaware.

Any director may resign at any time by giving written notice to the president or to the secretary of the Corporation. Such resignation shall take effect at the future time specified therein; and unless otherwise specified therein, the acceptance of such resignation shall not be necessary to make it effective. Any vacancy occurring on the board of directors created by the resignation of a director, may be filled by the affirmative vote of a majority of directors then in office, including those who have so resigned. The vote thereon shall take effect when such resignation or resignations shall become effective. A director elected to fill a vacancy shall be elected for the unexpired term of his predecessor in office.

3. Removal. Any director or the entire board of directors may be removed in accordance with the provisions of Article VII Subparagraph D of the Certificate of Incorporation.

4. Compensation. Any director may be paid any one or more of the following: his expenses, if any, of attendance at meetings; a fixed sum for attendance at each meeting; or a stated salary as director. No such payment shall preclude any director from serving the Corporation in any other capacity and receiving compensation therefor. A director shall also be entitled to receive options for the acquisition of shares of stock of the corporation.

ARTICLE IV.

Meetings of the Board

1. Place of Meetings. The regular or special meetings of the board of directors or any committee designated by the board may be held at the principal office of the Corporation or at any other place within or without the State of Delaware that a majority of the board of directors or any such committee, as the case may be, may designate from time to time by resolution.

2 . Regular Meetings. The board of directors shall meet each year immediately after the annual meeting of the stockholders for the purpose of electing officers and transacting such other business as may come before the meeting. The board of directors or any committee designated by the board may provide, by resolution, for the holding of additional regular meetings without other notice than such resolution.

3 . Special Meetings. Special meetings of the board of directors or any committee designated by the board may be called at any time by the chairman of the board, if any, by the president or by a majority of the members of the board of directors or any such committee, as the case may be.

4. Notice of Meetings. Notice of the regular meetings of the board of directors or any committee designated by the board need not be given. Except as otherwise provided by these Bylaws or the laws of the State of Delaware, written notice of each special meeting of the board of directors or any such committee setting forth the time and the place of the meeting shall be given to each director not less than two (2) days prior to the time fixed for the meeting. Notice of special meetings may be either given personally, personally by telephone, or by sending a copy of the notice through the United States mail or by telegram, telex or telecopy, charges prepaid, to the address of each director appearing on the books of the Corporation. If mailed, such notice shall be deemed to be delivered when deposited in the United States mail so addressed, with postage prepaid thereon. If notice is given by telegram, telex or telecopy, such notice shall be deemed to be delivered when the telegram is delivered to the telegraph, telex or telecopy operator. Neither the business to be transacted at, nor the purpose of, any regular or special meeting of the board of directors need be specified in the notice or waiver of notice of such meeting.

5. Waiver of Notice. A director may in writing waive notice of any special meeting of the board of directors or any committee, either before, at, or after the meeting; and his waiver shall be deemed the equivalent of giving notice. Attendance of a director at a meeting shall constitute waiver of notice of that meeting unless he attends for the express purpose of objecting to the transaction of business because the meeting has not been lawfully called or convened.

6. Quorum. At meetings of the board of directors or any committee designated by the board a majority of the number of directors fixed by these Bylaws or a majority of the members of any such committee, as the case may be, shall be necessary to constitute a quorum for the transaction of business. If a quorum is present, the act of the majority of directors in attendance shall be the act of the board of directors or any such committee, as the case may be, unless the act of a greater number is required by these Bylaws, the Certificate of Incorporation or the General Corporation Law of Delaware. One or more directors may participate in meetings of the board of directors as authorized by Subparagraph 11 of this Article IV by conference telephone, while the remaining director or directors are physically present at the meeting.

7. Presumption of Assent. A director who is present at a meeting of the board or committee designated by the board when corporate action is taken is deemed to have assented to the action taken unless: (i) he objects at the beginning of such meeting to the holding of the meeting or the transacting of business at the meeting; (ii) he contemporaneously requests that his dissent from the action taken be entered in the minutes of such meeting; or (iii) he gives written notice of his dissent to the presiding officer of such meeting before its adjournment or to the secretary of the Corporation immediately after adjournment of such meeting. The right of dissent as to a specific action taken in a meeting of a board or committee thereof is not available to a director who votes in favor of such action.

8. Reliance on Books of Account or Reports. Any member of the board of directors or any committee designated by the board of directors shall, in the performance of his duties, be fully protected in relying in good faith upon the records of the Corporation and upon such information, opinions, reports or statements presented to the Corporation by any of its officers, or employees, or committees of the board of directors, or by any other person as to matters the members reasonably believes are within such other persons professional or expert competence and who has been selected with reasonable care by or on behalf of the Corporation, or in relying in good faith upon other records of the Corporation.

9 . Committees. The board of directors may, by a resolution passed by a majority of the whole board designate one (1) or more committees, each committee to consist of one (1) or more directors of the corporation. The board may designate one or more directors as alternate members of any committee who may replace any absent or disqualified member at any meeting of the committee. In the absence or disqualification of a member of a committee, the member or members present at any meeting and not disqualified from voting, whether or not he or they constitute a quorum, may unanimously appoint another member of the board of directors to act at the meeting in the place of any such absent or disqualified member.

Any such committee to the extent provided in the resolution of the board of directors shall have and may exercise all of the powers and authority of the board of directors in the management of the business and affairs of the Corporation and may authorize the seal of the Corporation to be affixed to all papers which it may acquire. No such committee shall have the power or authority of the board of directors to: (i) amend the Certificate of Incorporation; (ii) adopt an agreement of merger or consolidation; (iii) recommend to the stockholders the sale, lease or exchange of all or substantially all of the Corporation's property and assets; (iv) recommend to the stockholders a dissolution of the Corporation or a revocation of a dissolution; (v) amend the Bylaws of the Corporation; (vi) or unless expressly provided for by resolution, or in the Certificate of Incorporation, declare a dividend, authorize the issuance of stock or to adopt a certificate of ownership and merger. To the extent authorized by resolution or resolutions providing for the issuance of shares of stock, adopted by the board, a committee may: (i) fix the designations and any of the preferences or rights of such shares relating to dividends, redemption, dissolution, any distribution of assets of the Corporation or the conversion into, or the exchange of such shares for, shares of any other class or classes or any other series of the same or any other class or classes of stock of the Corporation; or (ii) fix the number of shares of any series of stock or authorize the increase or decrease of the shares of any series. If any such delegation of the authority of the board of directors is made as provided herein, all references to the board of directors contained in these Bylaws, the Certificate of Incorporation, the General Corporation Law of Delaware or any other applicable law or regulation relating to the authority so delegated shall be deemed to refer to such committee.

10. Informal Action by Directors. Any action required or permitted to be taken at a meeting of the board of directors or any committee thereof, may be taken without a meeting if all the members of the board or committee, as the case may be, consent thereto in writing, and the writing or writings are filed with the minutes of proceedings of the board or committee. Such consent shall have the same force and effect as a unanimous vote of the directors and may be stated as such in any articles or documents filed with the Secretary of State of Delaware under the General Corporation Law of Delaware.

11. Telephonic Meetings. Members of the board of directors or any committee designated by the board may participate in meeting of such board or committee by means of a conference telephone or similar communications equipment by which all persons participating in the meeting can hear each other at the same time. Participation in such a meeting shall constitute presence in person at the meeting.

ARTICLE V.

Officers and Agents

1 . General. The executive officers of the Corporation shall be elected annually by the board of directors at the first meeting of the board held after each annual meeting of the stockholders. If the election of such officers shall not be held at such meeting, such election shall take place as soon thereafter as a meeting may conveniently be held. The officers of the Corporation shall consist of a president, a secretary and a treasurer, or a secretary/treasurer; in addition, one or more vice presidents, a chairman of the board of directors and such other officers, assistant officers, agents and employees that the board of directors may from time to time deem necessary may be elected by the board of directors or be appointed in a manner prescribed by the board.

Two or more offices may be held by the same person. Officers shall hold office until their successors are elected and qualified, unless they are sooner removed from office as provided in these Bylaws. All officers of the Corporation shall be natural persons of the age of eighteen years or older. Officers of the Corporation need not be residents of the State of Delaware or directors or stockholders of the Corporation.

2 . General Duties. All officers and agents of the Corporation, as between themselves and the Corporation, shall have such authority and shall perform such duties in the management of the Corporation as may be provided in these Bylaws or as may be determined by resolution of the board of directors not inconsistent with these Bylaws. In all cases where the duties of any officer, agent or employee are not prescribed by the Bylaws or by the board of directors, such officer, agent or employee shall follow the orders and instructions of the president.

Any officer shall have the power to execute and deliver on behalf of and in the name of the Corporation any instrument requiring the signature of an officer of the Corporation, except as otherwise provided in these Bylaws or where the execution and delivery thereof shall be expressly delegated by the board of directors to some other officer or agent of the Corporation. Unless authorized to do so by these Bylaws or by the board of directors, no officer, agent or employee shall have any power or authority to bind the Corporation in any way, to pledge its credit or to render it liable pecuniarily for any purpose or in any amount.

3. Vacancies. When a vacancy occurs in one of the executive offices by reason of death, resignation or otherwise, it shall be filled by a resolution of the board of directors. The officer so selected shall hold office until his successor is chosen and qualified.

4 . Salaries. The board of directors shall fix the salaries of the officers of the Corporation. The salaries of other agents and employees of the Corporation may be fixed by the board of directors, or by any committee designated by the board or by an officer to whom that function has been delegated by the board. No officer shall be prevented from receiving such salary by reason of the fact that he is also a director of the Corporation.

5 . Removal. Any officer or agent of this Corporation may be removed by the board of directors whenever in its judgment the best interests of the Corporation may be served thereby, but such removal shall be without prejudice to the contract rights, if any, of the person so removed. Election or appointment of an officer or an agent shall not of itself create contract rights.

6. Chairman of the Board. The chairman of the board, if any, shall preside as chairman at meetings of the stockholders and the board of directors. He shall, in addition, have such other duties as the board may prescribe that he perform. At the request of the president, the chairman of the board may, in the case of the president's absence or inability to act, temporarily act in his place. In the case of death of the president or in the case of his absence or inability to act without having designated the chairman of the board to act temporarily in his place, the chairman of the board shall perform the duties of the president, unless the board of directors, by resolution, provides otherwise. If the chairman of the board shall be unable to act in place of the president, any vice president may exercise such powers and perform such duties as provided in section 8 below.

6a. Executive Chairman. The board of directors may from time to time elect or appoint the chairman of the board, if any, to serve as executive chairman. To the extent such position is filled, all other executive officers shall report to the executive chairman, unless delegated otherwise by the executive chairman. The executive chairman shall have general and active management and supervision of the business and affairs of the Corporation.

7. President. The president shall be the chief executive officer of the Corporation (unless the board of directors appoints another executive to be the chief executive officer of the Corporation with such duties and responsibilities as the board of directors shall delegate from time to time), and, subject to the control of the board of directors, shall have general supervision of the business and affairs of the Corporation. In the event the position of chairman of the board shall not be occupied or the chairman shall be absent or otherwise unable to act, the president shall preside at meetings of the stockholders and directors and shall discharge the duties of the presiding officer. At each annual meeting of the stockholders the president shall give a report of the business of the Corporation for the preceding fiscal year and shall perform whatever other duties the board of directors may from time to time prescribe. The president may sign, with the secretary or any other proper officer of the Corporation thereunto authorized by the board of directors, certificates for shares of the Corporation, any deeds, mortgages, bonds, contracts, or other instruments which the board of directors has authorized to be executed, except in cases where the signing and execution thereof shall be expressly delegated by the board of directors or by these Bylaws to some other officer or agent of the Corporation, or shall be required by law to be otherwise signed or executed.

8. Vice Presidents. Each vice president shall have such powers and perform such duties as the board of directors may from time to time prescribe or as the president may from time to time delegate to him. At the request of the president, in the case of the president's absence or inability to act, any vice president may temporarily act in his place. In the case of the death of the president, or in the case of his absence or inability to act without having designated a vice president or vice presidents to act temporarily in his place, the board of directors, by resolution, may designate a vice president or vice presidents, to perform the duties of the president. If no such designation shall be made, the chairman of the board of directors, if any, shall exercise such powers and perform such duties, as provided in Section 6 above, but if the Corporation has no chairman of the board of directors, or if the chairman is unable to act in place of the president, all the vice presidents may exercise such powers and perform such duties.

9 . Secretary. The secretary shall keep or cause to be kept in books provided for that purpose the minutes of the meetings of the stockholders, executive committee, if any, and any other committees, and of the board of directors; shall see that all notices are duly given in accordance with the provisions of these Bylaws and as required by law; shall be custodian of the records and of the seal of the Corporation and see that the seal is affixed to all documents, the execution of which on behalf of the Corporation under its seal is duly authorized and in accordance with the provisions of these Bylaws; keep a register of the post office address of each stockholder which shall be furnished to the secretary by such stockholder, sign with the president certificates for shares of the Corporation, the issuance of which shall have been authorized by resolution of the board of directors; have a general charge of the stock transfer books of the Corporation; and, in general, shall perform all duties incident to the office of secretary and such other duties as may, from time to time, be assigned to him by the board of directors or by the president. In the absence of the secretary or his inability to act, the assistant secretaries, if any, shall act with the same powers and shall be subject to the same restrictions as are applicable to the secretary.

10. Treasurer. The treasurer shall have custody of corporate funds and securities. He shall keep full and accurate accounts of receipts and disbursements and shall deposit all corporate monies and other valuable effects in the name and to the credit of the Corporation in the depository or depositories of the Corporation selected by the board of directors, and shall render an account of his transactions as treasurer and of the financial condition of the Corporation to the president and/or the board of directors upon request. Such power given to the treasurer to deposit and disburse funds shall not, however, preclude any other officer or employee of the Corporation from also depositing and disbursing funds when authorized to do so by the board of directors. The treasurer shall, if required by the board of directors, give the Corporation a bond in such amount and with such surety or sureties as may be ordered by the board of directors for the faithful performance of duties of his office. The treasurer shall have such other duties as may be from time to time prescribed by the board of directors or the president. In the absence of the treasurer or his inability to act, the assistant treasurers, if any, shall act with the same authority and shall be subject to the same restrictions as are applicable to the treasurer.

11. Delegation of Duties. Whenever an officer is absent, or whenever, for any reason, the board of directors may deem it desirable, the board may delegate the powers and duties of an officer to any other officer or officers or to any director or directors.

12. Bond of Officers. The board of directors may require any officer to give the Corporation a bond in such sum and with such surety or sureties as shall be satisfactory to the board of directors for such terms and conditions as the board of directors may specify, including without limitation for the faithful performance of his duties and for the restoration to the Corporation of all property in his possession or under his control belong to the Corporation.

13. Loans to Director, Officers, Employees. The Corporation may lend money to, guarantee the obligations of and otherwise assist directors, officers and employees of the Corporation, or directors of another corporation of which the Corporation owns a majority of the voting stock to the extent of and in compliance with the General Corporation Laws of Delaware.

ARTICLE VI.

Stock Certificates and the Transfer of Shares

1. Stock Certificates; Uncertificated Shares. The shares of the Corporation shall be represented by certificates, provided that the board of directors of the Corporation may provide by resolution or resolutions that some or all of any or all classes or series of its stock shall be uncertificated shares. Any such resolution shall not apply to shares represented by a certificate until such certificate is surrendered to the corporation. Notwithstanding the adoption of such a resolution by the board of directors, every holder of stock represented by certificates and upon request every holder of uncertificated shares shall be entitled to have a certificate signed by, or in the name of the Corporation by the chairman or vice-chairman of the board of directors, or the president or vice-president, and by the treasurer or an assistant treasurer, or the secretary or an assistant secretary of the Corporation representing the number of shares registered in certificate form. Any or all the signatures on the certificate may be a facsimile. In case any officer, transfer agent or registrar who has signed or whose facsimile signature has been placed upon a certificate shall have ceased to be such officer, transfer agent or registrar before such certificate is issued, it may be issued by the Corporation with the same effect as if he were such officer, transfer agent or registrar at the date of issue.

2. Consideration for Shares. Shares shall be issued for such consideration as shall be fixed from time to time by the board of directors. Consideration for shares shall be expressed in dollars, and shall not be less than the par value or stated value therefor, as the case may be. The par value for shares, if any, shall be stated in the Certificate of Incorporation, and the stated value for shares, if any, shall be fixed from time to time by the board of directors. Treasury shares may be disposed of by the Corporation for such consideration expressed in dollars as may be fixed from time to time by the board. Consideration for shares may consist, in whole or in part, of money, other property whether tangible, intangible or both, or in labor or services actually performed for the Corporation, but the promise of future services of a subscriber or direct purchaser of shares from the Corporation shall not constitute payment or part payment for shares.

3 . Lost Certificates. The board of directors may direct a new certificate of stock or uncertificated share in place of any certificate issued by it, alleged to have been lost, stolen or destroyed if the owner makes an affidavit or affirmation of that fact and produces such evidence of loss or destruction as the board may require. The board, in its discretion, may as a condition precedent to the issuance of a new certificate require the owner to give the Corporation a bond sufficient to indemnify it against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of the certificate or the issuance of such new certificate.

4 . Transfer of Shares. Shares of the Corporation shall only be transferred on its books upon the surrender to the Corporation of the share certificates duly endorsed or accompanied by proper evidence of succession, assignment or authority to transfer and such documentary stamps as may be required by law. In that event, the surrendered certificates shall be cancelled, new certificates issued to the persons entitled to them, and the transaction recorded on the books or the Corporation.

5. Registered Stockholders. The Corporation shall be entitled to treat the holder of record of shares as the holder in fact and, except as otherwise provided by the laws of Delaware, shall not be bound to recognize any equitable or other claim to or interest in the shares.

The board of directors may adopt by resolution a procedure whereby a stockholder may certify in writing to the Corporation that all or a portion of the shares registered in the name of such stockholder are held for the account of a specified person or persons. Such resolution shall set forth: (i) the classification of stockholder who may certify; (ii) the purpose or purposes for which the certification may be made; (iii) the form of certification and information to be contained therein; (iv) if the certification is with respect to a record date or closing of the stock transfer books within which the certification must be received by the Corporation; and (v) such other provisions with respect to the procedure as are deemed necessary or desirable.

Upon receipt by the Corporation of a certification complying with the procedure, the persons specified in the certification shall be deemed, for the purpose or purposes set forth in the certification, to be the holders of record of the number of shares specified in place of the stockholder making the certification.

6 . Stock Ledger. An appropriate stock journal and ledger shall be kept by the secretary or such registrars or transfer agents as the directors by resolution may appoint in which all transactions in the shares of stock of the Corporation shall be recorded.

7 . Location. The books, accounts and records of the Corporation may be kept at such place or places within or outside the State of Delaware as the board of directors may from time to time determine.

8 . Inspection. The books, accounts and records of the Corporation shall be open for inspection by any member of the board of directors at all times, and open to inspection by the stockholders at such times, and subject to such regulations as the board of directors may prescribe, except as otherwise provided by statute.

ARTICLE VII.

Seal and Fiscal Year

1. Seal. The Corporation shall have a seal in the form impressed to the left of this paragraph of the Bylaws.

2 . Fiscal Year. The fiscal year of the Corporation shall be determined by the board of directors and set forth in the minutes of the directors. Said fiscal year may be changed from time to time by the board of directors in its discretion.

ARTICLE VIII.

Dividends

Dividends shall be declared and paid out of the surplus or net profits for the fiscal year in which the dividend is declared, and/or the preceding fiscal year as often and at such times as the board of directors may determine. If the capital of the Corporation, computed in accordance with the General Corporation Law of Delaware, shall have been diminished by depreciation in the value of its property, or by losses, or otherwise, to an amount less than the aggregate amount of the capital represented by the issued and outstanding stock; the board of directors shall not declare and pay out of net profits any dividends upon any shares of its capital stock until the deficiency in the amount of capital represented by issued and outstanding stock shall have been repaired. No unclaimed dividend shall bear interest against the Corporation.

ARTICLE IX.

Amendments

Subject to repeal or change by action of the stockholders in accordance with the Certificate of Incorporation, the board of directors may amend, supplement or repeal these Bylaws or adopt new Bylaws, and all such changes shall affect and be binding upon the holders of all shares heretofore as well as hereafter authorized, subscribed for or offered.

ARTICLE X.

Miscellaneous

1 . Gender. Whenever required by the context, the singular shall include the plural, the plural the singular, and one gender shall include all genders.

2 . Invalid Provision. The invalidity or unenforceability of any particular provision of these Bylaws shall not affect the other provisions herein, and these Bylaws shall be construed in all respects as if such invalid or unenforceable provision was omitted.

3. Governing Law. These Bylaws shall be governed by and construed in accordance with the laws of the State of Delaware.

4. Severability. If any provision (or any part thereof) or provisions of these Bylaws shall be held to be invalid, illegal or unenforceable as applied to any circumstance for any reason whatsoever: (i) the validity, legality and enforceability of such provisions in any other circumstance and of the remaining provisions of these Bylaws (including, without limitation, each portion of any section of these Bylaws containing any such provision held to be invalid, illegal or unenforceable that is not itself held to be invalid, illegal or unenforceable) shall not in any way be affected or impaired thereby and (ii) to the fullest extent possible, the provisions of these Bylaws (including, without limitation, each such portion containing any such provision held to be invalid, illegal or unenforceable) shall be construed for the benefit of the Corporation to the fullest extent permitted by law so as to (a) give effect to the intent manifested by the provision held invalid, illegal or unenforceable, and (b) permit the Corporation to protect its directors, officers, employees and agents from personal liability in respect of their good faith service. Reference herein to laws, regulations or agencies shall be deemed to include all amendments thereof, substitutions therefor and successors thereto, as the case may be.

ARTICLE XI.

Exclusive Jurisdiction for Certain Actions

1. Forum for Adjudication of Disputes. Unless the Corporation consents in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware shall, to the fullest extent permitted by law, be the sole and exclusive forum for (a) any derivative action or proceeding brought on behalf of the Corporation, (b) any action asserting a claim of breach of fiduciary duty owed by, or other wrongdoing by, any director, officer, employee or agent of the Corporation to the Corporation or the Corporation's stockholders, creditors or other constituents, (c) any action asserting a claim arising pursuant to any provision of the General Corporation Law of Delaware or the Certificate of Incorporation or these Bylaws of the Corporation, (d) any action to interpret, apply, enforce or determine the validity of the Certificate of Incorporation or these Bylaws of the Corporation or (e) any action asserting a claim governed by the internal affairs doctrine, in each case subject to said Court of Chancery of the State of Delaware having personal jurisdiction over the indispensable parties named as defendants therein; provided that if and only if the Court of Chancery of the State of Delaware dismisses any such action for lack of subject matter jurisdiction, such action may be brought in another state or federal court sitting in the State of Delaware. To the fullest extent permitted by applicable law, any person or entity purchasing or otherwise acquiring any interest in shares of capital stock of the Corporation shall be deemed to have notice of and consented to the provisions of this Article XI of these Bylaws.

ARTICLE XII.

Litigation Costs

Except to the extent prohibited by the General Corporation Law of Delaware, and unless the board of directors or one of its committees otherwise approves in accordance with Section 141 of the General Corporation Law of Delaware, the Certificate of Incorporation and these Bylaws, in the event that any current or prior stockholder or anyone on their behalf (a "Claiming Party") (a) initiates, asserts, joins, offers substantial assistance to or has a direct financial interest in (i) any derivative action or proceeding brought on behalf of the Corporation, (ii) any action asserting a claim of breach of a fiduciary duty owed by any director, officer or other employee of the Corporation to the Corporation or the Corporation's stockholders, (iii) any action asserting a claim against the Corporation arising pursuant to any provision of the General Corporation Law of Delaware or the Corporation's Certificate of Incorporation or Bylaws, (iv) any action to interpret, apply, enforce or determine the validity of the Certificate of Incorporation or these Bylaws or (v) any action asserting a claim against the Corporation governed by the internal affairs doctrine (each, a "Covered Proceeding"), and (b) such Claiming Party does not obtain a judgment on the merits that substantially achieves, in substance and amount, the full remedy sought by such Claiming Party, then each such Claiming Party shall be obligated to reimburse the Corporation and any such director, officer or other employee for all fees, costs and expenses of every kind and description (including, but not limited to, all attorneys' fees and other litigation expenses) that the Corporation or any such director, officer or other employee actually incurs in connection with the Covered Proceeding. Any person or entity purchasing or otherwise acquiring any interest in the shares of capital stock of the Corporation shall be deemed to have notice of and consented to the provisions of this Article XII.

Exhibit 10.18

CONFIDENTIAL TREATMENT REQUESTED

EXECUTION COPY

LICENSE AGREEMENT

This LICENSE AGREEMENT ("Agreement") is entered into as of November 4, 2018 ("Effective Date") by and between REGENXBIO Inc., a corporation organized under the laws of the State of Delaware, with offices at 9600 Blackwell Road, Suite 210, Rockville, MD 20850 ("Licensor"), and Abeona Therapeutics Inc., a corporation organized under the laws of the State of Delaware, with offices at 1330 Avenue of the Americas, 33rd Floor, New York, NY 10019 ("Licensee"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor has rights under certain Licensed Patents (as defined herein) pertaining to adeno-associated virus serotype 9;

WHEREAS, Licensee desires to obtain from Licensor an exclusive license under the Licensed Patents under the terms set forth herein; and

WHEREAS, the Licensee has determined, and the Licensor has relied upon such determination, that the Agreement does not require regulatory approval under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended.

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE 1: DEFINITIONS

1.1 "AAV9" means (a) the recombinant adeno-associated virus serotype 9 vector with the specified sequence set forth in GenBank **** and (b) any recombinant adeno-associated virus derivatives of such serotype 9 vector that are covered by the claims of the Licensed Patents.

1.2 "Affiliate" means any legal entity directly or indirectly, during the term of this Agreement, controlling, controlled by, or under common control with another entity. For purposes of this Agreement, "control" means the direct or indirect ownership of more than 50% of the outstanding voting securities of a legal entity, or the right to receive more than 50% of the profits or earnings of a legal entity, or the right to control the policy decisions of a legal entity. An entity may be or become an Affiliate of an entity and may cease to be an Affiliate of an entity, in each case, during the term of this Agreement.

1.3 "Calendar Quarter" means each three-month period or any portion thereof, beginning on January 1, April 1, July 1, and October 1.

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

1.4 "Change of Control" means (i) any transaction or series of related transactions following which the holders of Licensee's capital stock or membership or equity interests immediately prior to such transaction or series of related transactions collectively are the owners of less than 50% of the outstanding equity interests of Licensee entitled to (a) vote with respect to the election of directors (or positions having a similar function) or (b) receive the proceeds upon any sale, liquidation or dissolution of Licensee; (ii) a sale, transfer, or other disposition, in a single transaction or series of related transactions, of all or a material portion of Licensee's interest in the Licensed Products; (iii) a sale, transfer, or other disposition, in a single transaction or series of related transactions, of all or a material portion of Licensee's right title, or interest in its assets taken as a whole; or (iv) the merger of Licensee with a Third Party by operation of law or otherwise.

1.5 "CLN1 Field" means the treatment of Neuronal Ceroid Lipfuscinosis-1, also known as infantile Batten disease, in humans by *in vivo* gene therapy using AAV9 to deliver the PPT1 gene.

1.6 "CLN3 Field" means the treatment of Neuronal Ceroid Lipfuscinosis-3, also known as juvenile Batten disease, in humans by *in vivo* gene therapy using AAV9 to deliver the CLN3 gene.

1.7 "Confidential Information" means and includes all technical information, inventions, developments, discoveries, software, know-how, methods, techniques, formulae, animate and inanimate materials, data, processes, finances, business operations or affairs, and other proprietary ideas, whether or not patentable or copyrightable, of either Party that are (a) marked or otherwise identified as confidential or proprietary at the time of disclosure in writing; or (b) if disclosed orally, visually, or in another non-written form, identified as confidential at the time of disclosure and summarized in reasonable detail in writing as to its general content within 30 days after original disclosure. The Parties acknowledge that (i) the terms and conditions of this Agreement and (ii) the records and reports referred to in Section 3.6 will be deemed the Confidential Information of both Parties, regardless of whether such information is marked or identified as confidential. In addition, information provided to Licensee pursuant to the provisions of Section 7.1 will be deemed the Confidential Information of Licensor, regardless of whether such information is marked or identified as confidential. Notwithstanding the foregoing, Confidential Information will not include the following, in each case, to the extent evidenced by competent written proof of the Receiving Party:

1.7.1 information that was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party;

1.7.2 information that was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

1.7.3 information that became generally available to the public or otherwise part of the public domain after its disclosure, other than through any act or omission of the Receiving Party in breach of this Agreement;

1.7.4 information that is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or

1.7.5 information that was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

1.8 "Conflicting License" means the license agreement **** that licenses rights under the Licensed Technology on a non-exclusive, sublicensable basis for the treatment of MPSIII Type A in human beings by *in vivo* administration.

1.9 "Disclosing Party" has the meaning set forth in Section 5.1.

1.10 "Domain Antibody" ****.

1.11 "FDA" means the United States Food and Drug Administration, or a successor agency in the United States with responsibilities comparable to those of the United States Food and Drug Administration.

1.12 "Fields" means the CLN1 Field, the CLN3 Field, the MPS IIIA Field and the MPS IIIB Field (individually, the CLN1 Field, the CLN3 Field, the MPS IIIA Field and the MPS IIIB Field are hereinafter referred to as a "Field").

1.13 "GSK Agreement" means that certain License Agreement entered into between Licensor and SmithKline Beecham Corporation, effective on March 6, 2009, as amended by that certain Amendment to License Agreement dated April 15, 2009, and as further amended from time to time.

1.14 "First Commercial Sale" of a Licensed Product means the first transfer by Licensee, its Affiliates or Sublicensees for value in an arms'-length transaction to an independent third party distributor, agent or end user in a country after obtaining all approvals from Regulatory Authorities necessary for such transfer in such country. For clarity, sales or transfers of a product for clinical trial purposes, compassionate or similar use or indigent programs shall not constitute a "First Commercial Sale" hereunder.

1.15 "Know-How" means any and all ideas, information, know-how, data, research results, writings, inventions, discoveries, and other technology (including any proprietary materials), whether or not patentable or copyrightable.

1.16 Licensed Back Improvements" has the meaning ascribed to it in Section 2.5.2.

1.17 "Licensed Know-How" means any Know-How Licensor provides to Licensee pursuant to Section 2.6.

1.18 "Licensed Patents" means, to the extent they cover AAV9, (a) all United States patents and patent applications listed in Exhibit A, including patents arising from such patent applications; (b) any re-examination certificates thereof; (c) the foreign counterparts of the patents and patent applications in subsections (a) and (b); (d) extensions, continuations, divisionals, and re-issue applications of the patents and patent applications in subsections (a) through (c); and (e) continuations-in-part to the extent that the continuations-in-part contain one or more claims directed to the invention or inventions disclosed in the patent and patent applications in subsections (a) through (c); provided that "Licensed Patents" will not include any claim of a patent or patent application covering any Manufacturing Technology.

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1.19 "Licensed Product" means (a) any AAV9 product that is made, made for, used, sold, offered for sale, or imported by Licensee, its Affiliates, and any of its or their Sublicensees, (i) the manufacture, use, sale, offer for sale, or import of which product, in the absence of the license granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import, including products manufactured by a process that would infringe or is covered by at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import or (ii) that incorporates, was developed using, or is produced or manufactured through the use of Licensed Know-How (it being understood that Licensee shall be deemed to be using Licensed Know-How based upon the license it receives under this Agreement); or (b) any service sold by Licensee, its Affiliates, and any of its or their Sublicensees with respect to the administration of any AAV9 product to patients that (i) in the absence of the license granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim in the country of sale or (ii) that incorporates, was developed using, or is produced or manufactured through the use of Licensed Know-How (it being understood that Licensee shall be deemed to be using Licensed Know-How based upon the license it receives under this Agreement).

1.20 "Licensed Technology" means, collectively, the Licensed Patents and Licensed Know-How.

1.21 "Manufacturing Technology" means any and all patents, patent applications, Know-How, and all intellectual property rights associated therewith that are owned or controlled by Licensor, and including all tangible embodiments thereof, that claim, cover or relate to the manufacture of adeno-associated viruses, adeno-associated virus vectors, research or commercial reagents related thereto, Licensed Products, or other products, including manufacturing processes, technical information relating to the methods of manufacture, protocols, standard operating procedures, batch records, assays, formulations, quality control data, specifications, scale up methods, any and all improvements, modifications, and changes thereto, and any and all activities associated with such manufacture. Any and all chemistry, manufacturing, and controls (CMC), drug master files (DMFs), or similar materials provided to Regulatory Authorities and the information contained therein are deemed Manufacturing Technology.

1.22 "MPS IIIA Field" means the treatment of Mucopolysaccharidosis type IIIA, also known as Sanfilippo Syndrome Type A, in humans by *in vivo* gene therapy using AAV9 to deliver the SGSH gene.

1.23 "MPS IIIB Field" means the treatment of Mucopolysaccharidosis type IIIB, also known as Sanfilippo Syndrome Type B, in humans by *in vivo* gene therapy using AAV9 to deliver the NAGLU gene.

1.24 "Net Sales" means the gross receipts from sales or other disposition of a Licensed Product (including fees for services within the definition of "Licensed Product") by Licensee and/or its Affiliates and/or any Sublicensees to Third Parties less the following deductions that are directly attributable to a sale, specifically and separately identified on an invoice or other documentation and actually borne by Licensee, its Affiliates, or any Sublicensees: ****. In the event consideration other than cash is paid to Licensee, its Affiliates, or any Sublicensees, for purposes of determining Net Sales, the Parties shall use the cash consideration that Licensee, its Affiliates, or any Sublicensees would realize from an unrelated buyer in an arm's length sale of an identical item sold in the same quantity and at the time and place of the transaction, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

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1.25 "Penn Agreement" means that certain License Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective on February 24, 2009, as amended by that letter agreement dated March 6, 2009, by that certain Second Amendment to License Agreement effective on September 9, 2014, and by that certain Third Amendment to License Agreement effective on April 29, 2016, and as further amended from time to time.

1.26 "Prosecute" means preparation, filing, and prosecuting patent applications and maintaining patents, including any reexaminations, reissues, oppositions, inter partes review, and interferences.

1.27 "Receiving Party" has the meaning set forth in Section 5.1.

1.28 "REGENXBIO Licensors" means SmithKline Beecham Corporation (or any successor thereto under the GSK Agreement) and The Trustees of the University of Pennsylvania (or any successor thereto under the Penn Agreement), if any Licensed Technology is sublicensed from the Penn Agreement.

1.29 "Regulatory Authority" means any national, supra-national, regional, state or local regulatory agency, department, bureau, commission, council or other governmental entity with authority over (a) the distribution, importation, exportation, manufacture, production, use, storage, transport, clinical testing or sale of a Licensed Product, including the FDA, or (b) setting the price and/or reimbursement for a Licensed Product.

1.30 "Retained Rights" has the meaning set forth in Section 2.2.

1.31 "Sublicensee" means (i) any Third Party or Affiliate to whom Licensee grants a sublicense of some or all of the rights granted to Licensee under this Agreement as permitted by this Agreement; and (ii) any other Third Party or Affiliate to whom a sublicensee described in clause (i) has granted a further sublicense as permitted by this Agreement.

1.32 "Third Party" means any person or entity other than a Party to this Agreement or Affiliates of a Party to this Agreement.

1.33 "Valid Claim" means a claim of an issued and unexpired patent (including any patent claim the term of which is extended by any extension, supplementary protection certificate, patent term restoration, or the like) included within the Licensed Patents or a claim of a pending patent application included within the Licensed Patents, that has not lapsed, been abandoned, been held revoked, or been deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction.

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ARTICLE 2: LICENSE GRANTS

2.1 License Grant. Subject to the terms and conditions of this Agreement, including the Retained Rights, Licensor hereby grants to Licensee an exclusive, sublicensable (as provided in Section 2.4 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license under the Licensed Technology to make, have made, use, import, sell, and offer for sale Licensed Products using AAV9 solely in the Fields, including, for the avoidance of doubt, the right to conduct research and development; provided that with respect to the MPSIIIA Field, the license granted under this Section 2.1 shall be exclusive with the exception of those rights Licensor has granted under the Conflicting License.

2.2 Retained Rights. Except for the rights and licenses specified in Section 2.1, no license or other rights are granted to Licensee under any intellectual property of Licensor, whether by implication, estoppel, or otherwise and whether such intellectual property is subordinate, dominant, or otherwise useful for the practice of the Licensed Technology. Notwithstanding anything to the contrary in this Agreement, Licensor may use and permit others to use the Licensed Technology for any research, development, commercial, or other purposes outside of the Fields. Without limiting the foregoing, and notwithstanding anything in this Agreement to the contrary, Licensee acknowledges and agrees that the following rights are retained by Licensor and the REGENXBIO Licensors (individually and collectively, the "Retained Rights"), whether inside or outside the Fields:

2.2.1 The rights and licenses granted in Section 2.1 shall not include any right (and Licensor and the REGENXBIO Licensors retain the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Technology to make, have made, use, sell, offer to sell, and import Domain Antibodies that are expressed by an adeno-associated vector, including AAV9.

2.2.2 Licensor and the REGENXBIO Licensors retain the following rights with respect to the Licensed Technology:

(a) A non-exclusive, sublicensable right under the Licensed Technology to make, have made, use, sell, offer to sell, and import products that deliver RNA interference and antisense drugs using an adeno-associated vector, including AAV9; and

(b) A non-exclusive right for the REGENXBIO Licensors (which right is sublicensable by the REGENXBIO Licensors) to use the Licensed Technology for non-commercial research purposes and to use the Licensed Technology for such REGENXBIO Licensors' discovery research efforts with non-profit organizations and collaborators.

2.2.3 The rights and licenses granted in Section 2.1 shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Technology:

(a) to conduct commercial reagent and services businesses, which includes the right to make, have made, use, sell, offer to sell, and import research reagents, including any viral vector construct; provided that, for clarity, such rights retained by Licensor shall not include the right to conduct clinical trials in humans in the Fields; or

(b) to use the Licensed Technology to provide services to any Third Parties; provided that Licensee's license under Section 2.1 does include the right to provide the service of the administration of Licensed Products to patients.

2.2.4 Licensor retains the fully sublicensable right under the Licensed Technology to grant non-exclusive research and development licenses to Affiliates and Third Parties; provided that such research and development rights granted by Licensor shall not include the right to conduct clinical trials in humans in the Fields or any rights to sell products in the Fields.

2.2.5 The Trustees of the University of Pennsylvania may use and permit other non-profit organizations or other non-commercial entities to use the Licensed Technology for educational and research purposes.

2.3 Government Rights. Licensee acknowledges that the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant, or similar agreement with a federal agency. The license grants hereunder are expressly subject to all applicable United States government rights, including any applicable requirement that products resulting from such intellectual property sold in the United States must be substantially manufactured in the United States.

2.4 Sublicensing.

2.4.1 The license granted pursuant to Section 2.1 is sublicensable by Licensee to any Affiliates or Third Parties; provided that any such sublicense must comply with the provisions of this Section 2.4 (including Section 2.4.2).

2.4.2 The right to sublicense granted to Licensee under this Agreement is subject to the following conditions:

(a) Licensee may only grant sublicenses pursuant to a written sublicense agreement with the Sublicensee. Licensor must receive written notice as soon as practicable following execution of any such sublicenses. Any further sublicenses granted by any Sublicensees (to the extent permitted hereunder) must comply with the provisions of this Section 2.4 (including Section 2.4.2) to the same extent as if Licensee granted such sublicense directly.

(b) In each sublicense agreement, the Sublicensee must be required to comply with the terms and conditions of this Agreement to the same extent as Licensee has agreed and must acknowledge that Licensor is an express third party beneficiary of such terms and conditions under such sublicense agreement.

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(c) The official language of any sublicense agreement shall be English.

(d) Within **** after entering into a sublicense, Licensor must receive a copy of the sublicense written in the English language for Licensor's records and to share with the REGENXBIO Licensors. The copy of the sublicense may be redacted to exclude confidential information of the applicable Sublicensee, but such copy shall not be redacted to the extent that it impairs Licensor's (or the REGENXBIO Licensors') ability to ensure compliance with this Agreement; provided that, if either of the REGENXBIO Licensors requires a complete, unredacted copy of the sublicense, Licensee shall provide such complete, unredacted copy.

(e) Licensee's execution of a sublicense agreement will not relieve Licensee of any of its obligations under this Agreement. Licensee is and shall remain **** to Licensor for all of Licensee's duties and obligations contained in this Agreement and for any act or omission of an Affiliate or Sublicensee that would be a breach of this Agreement if performed or omitted by Licensee, and Licensee will be deemed to be in breach of this Agreement as a result of such act or omission.

2.5 Improvements.

2.5.1 Licensee hereby grants to Licensor a non-exclusive, worldwide, royalty-free, transferable, sublicensable, irrevocable, perpetual license:

(a) to use any Licensed Back Improvements (and any intellectual property rights with respect thereto) consummate in scope to the Retained Rights, and

(b) to practice the Licensed Back Improvements (and any intellectual property rights with respect thereto) in connection with AAV9, including the right to research, develop, make, have made, use, offer for sale, and sell products and services; provided that Licensor shall have no right, under the license in this Section 2.5.1(b), to practice the Licensed Back Improvements in the Fields.

2.5.2 For purposes of this Agreement, "Licensed Back Improvements" means any patentable modifications or improvements developed by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim within the Licensed Patents.

2.5.3 Licensee agrees to provide prompt notice to Licensor upon the filing of any patent application covering any Licensee Invention and/or any Licensed Back Improvement, together with a reasonably detailed description of, or access to, such Licensee Inventions and/or Licensed Back Improvement to permit the practice of any such invention or improvement.

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2.6 Transfer of Licensed Know-How. During the **** following the Effective Date, at Licensee's sole expense, to the extent not previously disclosed or provided to Licensee, (a) Licensor will deliver to Licensee copies of Licensed Know-How set forth in Exhibit B in the form that such Licensed Know-How then exists; and (b) Licensor will disclose to Licensee any development Know-How, including through in-person or telephonic meetings at such times and places as agreed to by the Parties, relating to the Licensed Products that Licensor agrees to provide to Licensee following a written request from Licensee. Licensee acknowledges and agrees that all Licensed Know-How disclosed pursuant to this Section 2.6 will be deemed "Confidential Information" of Licensor, regardless of whether such information is marked or identified as confidential and without an obligation to summarize oral information.

2.7 Regulatory Rights. Upon request, Licensee shall grant Licensor a "right of reference" for the FDA or any equivalent foreign regulatory body to any submissions containing ****. Upon request, Licensor shall grant Licensee a "right of reference" for the FDA or any equivalent foreign regulatory body to any submissions containing ****.

2.8 Collaboration Activities. Solely upon the written request of Licensee, Licensor may, in its sole discretion after receipt of such request, engage in collaboration activities with Licensee related to ****.

2.9 Section 365(n) of the Bankruptcy Code. All rights and licenses granted to Licensee or Licensor under or pursuant to this Agreement are and will otherwise be deemed to be, for purposes of Section 365(n) of the United States Bankruptcy Code (Title 11, U.S. Code), as amended (the "Bankruptcy Code") or any comparable law outside the United States, licenses of rights to "intellectual property" as defined in Section 101(35A) of the Bankruptcy Code. The Parties will retain and may fully exercise all of their respective rights and elections under the Bankruptcy Code and any comparable law outside the United States.

ARTICLE 3: CONSIDERATION

3.1 Initial Fee. In partial consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay Licensor an initial fee of $20,000,000, which shall be payable as follows: (a) $10,000,000 within **** after the Effective Date; and (b) $10,000,000 within twelve (12) months of the Effective Date; provided that any unpaid portion of the initial fee shall be immediately payable upon termination of this Agreement or a Change of Control.

3.2 Annual Fees. In partial consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay Licensor annual fees of $100,000,000, which shall be payable as follows: (i) $20,000,000 on the second anniversary of the Effective Date; (ii) $20,000,000 on the third anniversary of the Effective Date; (iii) $20,000,000 on the fourth anniversary of the Effective Date; (iv) $20,000,000 on the fifth anniversary of the Effective Date; and (v) $20,000,000 on the sixth anniversary of the Effective Date. In the event of termination of the Agreement prior to the second anniversary of the Effective Date, Licensee shall pay Licensor any unpaid amounts recited in (i) within **** of such termination. In the event of a Change of Control and to the extent that payments (i), (ii), (iii), (iv) and/or (v) above have not been received by Licensor from Licensee, Licensee shall pay Licensor any such unpaid amounts recited in (i), (ii), (iii), (iv) or (v) (subject to adjustments in amounts due for any terminated Fields in accordance with Section 6.6.6, provided such termination is effective prior to the Change of Control) within **** of the Change of Control.

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3.3 Milestone Fees. In partial consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay Licensor the following milestone payments:

Milestone	**Milestone Payment**
1. Cumulative Net Sales of Licensed Products first reaching ****	****
2. Cumulative Net Sales of Licensed Products first reaching ****	****
Total:	$60,000,000

3.3.1 For clarity, each milestone payment set forth in Section 3.3 is payable only once.

3.4 Royalties. In further consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay to Licensor a royalty of **** on Net Sales of Licensed Products, subject to the reductions in royalty rates set forth in Section 3.4.1.

3.4.1 Third Party Royalties Stacking Provision. If Licensee must obtain a license from a Third Party to avoid infringement of such Third Party's rights in order to manufacture, use, or commercialize a given Licensed Product and if the royalties required to be paid to such Third Party for such license, together with those royalties payable to Licensor, in the aggregate, exceed **** of Net Sales for any Licensed Product, then the royalty owed to Licensor for that Licensed Product will be reduced by an amount calculated as follows:

STACKING ROYALTY CALCULATIONS

$$R = (C * (A / (A+B)))$$

Where
R = reduction of Licensor royalty,
A = unreduced Licensor royalty,
B = sum of all Third Party royalties,
C = increment of projected total royalty above ****.

Example Calculation:

Assume: i) all Third Party royalties = ****
ii) unreduced Licensor royalty = ****
iii) projected total royalty = ****

R = (**** - ****) * (**** / (**** + ****))
R = (**** * ****)
R = ****
Licensor Stacked Royalty = **** – **** = **** (but subject to the cap described below which limits the reduction to **** in the aggregate)

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Notwithstanding the foregoing, Licensee will pay to Licensor no less than **** of the royalties that Licensee would otherwise pay to Licensor with respect to Net Sales of Licensee if there were no royalties due to Third Parties.

3.4.2 Adjustment of Royalties For Licenses. On a Licensed Product-by-Licensed Product, country-by-country basis, upon the date on which the manufacture, use, sale, offer for sale, or import of a Licensed Product does not infringe or is not covered by a Valid Claim in such country, then the royalty percentage applicable to Net Sales of such Licensed Product under this Section 3.4 in such country shall be reduced by ****.

3.4.3 Royalty Payment Period. Licensee's obligation hereunder for payment of a royalty under this Section 3.4 on the Net Sales of Licensed Products in a given country will end on a country-by-country, Licensed Product-by-Licensed Product basis on the later of: (i) expiration, lapse, abandonment, or invalidation of the last Valid Claim of the Licensed Patents to expire, lapse, become abandoned or become unenforceable for the applicable Licensed Product in the applicable country, or (ii) **** from the First Commercial Sale of the applicable Licensed Product in the applicable country.

3.5 Sublicense Fees.

3.5.1 In further consideration of the rights and licenses granted to Licensee under this Agreement, Licensee will pay Licensor **** of any sublicense fees (****) received by Licensee or its Affiliates from a Third Party for the Licensed Technology from any Sublicensee or from any person or entity granted any option to obtain a sublicense ("Sublicensing Revenue").

3.5.2 With respect to the obligations under this Section 3.5, Licensee shall not be required to submit any amounts received from a Third Party for the following:

(a) Reimbursement or payment of Licensee's actual costs or on an arm's length cost plus arrangement for research, development, and/or manufacturing activities performed by Licensee or its Affiliates corresponding directly to the research, development and/or manufacturing of Licensed Products pursuant to a specific agreement;

(b) Any and all amounts paid to Licensee or its Affiliates by a Sublicensee as royalties on sales of Licensed Product sold by the Sublicensee under a sublicense agreement; and

(c) Consideration received for the purchase of an equity interest in Licensee or its Affiliates at fair market value or in the form of loans at arm's length rates of interest.

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3.5.3 If Licensee or its Affiliates receives sublicense fees from Sublicensees or from any person or entity granted any option to obtain a sublicense under this Agreement in the form of non-cash consideration, then Licensee shall pay Licensor a cash payment as required under Section 3.5 determined based on the fair market value of such non-cash consideration. If Licensee or its Affiliate enters into any sublicense that is not an arm's length transaction, fees due under this Section 3.5 will be calculated based on the fair market value of such transaction, at the time of the transaction, assuming an arm's length transaction made in the ordinary course of business, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

3.5.4 To the extent Licensee or its Affiliates receives payment from a Third Party relating to one or more of the milestone events set forth in the table in Section 3.3, then the amount of the payment made to Licensor under such Section 3.3 with respect to such milestone event shall not be deemed sublicense fees under this Section 3.5; instead, the amounts due under this Section 3.5 shall be calculated by applying the applicable sublicense fee rate set forth in Section 3.5 above to the sublicense fees received by Licensee or its Affiliates from such Third Party after deducting the amount of the payment under Section 3.3.

3.6 Reports and Records.

3.6.1 Licensee must deliver to Licensor within **** after the end of each Calendar Quarter after the First Commercial Sale of a Licensed Product a report setting forth the calculation of the royalties due to Licensor for such Calendar Quarter on a Licensed Product by Licensed Product basis, including:

(a) Number of Licensed Products included within Net Sales, listed by country;

(b) Gross consideration for Net Sales of Licensed Product, including all amounts invoiced, billed, or received, listed by country;

(c) Qualifying costs to be excluded from the gross consideration, as described in Section 1.24, listed by category of cost and by country;

(d) Net Sales of Licensed Products listed by country;

(e) A detailed accounting of any royalty reductions applied pursuant to Section 3.4.1;

(f) Royalties owed to Licensor; and

(g) The computations for any applicable currency conversions.

3.6.2 Licensee shall pay the royalties due under Section 3.4 within **** following the last day of the Calendar Quarter in which the royalties accrue. Licensee shall send the royalty payments along with the report described in Section 3.6.1.

3.6.3 Within **** after the occurrence of a milestone event described in Section 3.3, Licensee must deliver to Licensor a report describing the milestone event that occurred, together with a payment of the applicable amount due to Licensor pursuant to Section 3.3.

3.6.4 Within **** after the receipt of any fees from any Third Party as described in Section 3.5, Licensee must deliver to Licensor a report describing the fees received, together with a payment of the applicable amount due to Licensor pursuant to Section 3.5.

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3.6.5 All financial reports under this Section 3.6 will be certified by the chief financial officer of Licensee or Licensee's qualified financial representative.

3.6.6 Licensee shall maintain and require its Affiliates and all Sublicensees to maintain, complete, and accurate books and records that enable the royalties, fees, and payments payable under this Agreement to be verified. The records must be maintained for **** after the submission of each report under Article 3. Upon reasonable prior written notice to Licensee, Licensee and its Affiliates and all Sublicensees will provide Licensor and/or the REGENXBIO Licensors (and their respective accountants) with access to all of the relevant books, records, and related background information required to conduct a review or audit of the royalties, fees, and payments payable to Licensor under this Agreement to be verified. Access will be made available: (a) during normal business hours; (b) in a manner reasonably designed to facilitate the auditing party's review or audit without unreasonable disruption to Licensee's business; and (c) no more than once each calendar year during the term of this Agreement and for a period of **** thereafter. Licensee will promptly pay to Licensor the amount of any underpayment determined by the review or audit, plus accrued interest. If the review or audit determines that Licensee has underpaid any payment by **** or more, then Licensee will also promptly pay the costs and expenses of Licensor and the REGENXBIO Licensors and their respective accountants in connection with the review or audit. If the review or audit determines that Licensee has overpaid any payment, then Licensor shall refund the overpayment to Licensee.

3.7 Currency, Interest.

3.7.1 All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments to Licensor under this Agreement must be made in United States dollars.

3.7.2 If Licensee receives payment in a currency other than United States dollars for which a royalty or fee or other payment is owed under this Agreement, then (a) the payment will be converted into United States dollars at the conversion rate for the foreign currency as published in the eastern edition of the *Wall Street Journal*, N.Y. edition or other equivalent publication as mutually agreed upon by the Parties, as of the last business day of the Calendar Quarter in which the payment was received by Licensee; and (b) the conversion computation will be documented by Licensee in the applicable report delivered to Licensor under Section 3.6.

3.7.3 All amounts that are not paid by Licensee when due will accrue interest from the date due until paid at a rate equal to 1.5% per month (or the maximum allowed by law, if less).

3.8 Taxes and Withholding.

3.8.1 All payments hereunder will be made free and clear of, and without deduction or deferment in respect of, and Licensee shall pay and be responsible for, and shall hold Licensor harmless from and against, any taxes, duties, levies, fees, or charges, including sales, use, transfer, excise, import, and value added taxes (including any interest, penalties, or additional amounts imposed with respect thereto) but excluding withholding taxes to the extent provided in Section 3.8.2. At the request of Licensee, Licensor will give Licensee such reasonable assistance, which will include the provision of documentation as may be required by the relevant tax authority, to enable Licensee to pay and report and, as applicable, claim exemption from or reduction of, such tax, duty, levy, fee, or charge.

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3.8.2 If any payment made by Licensee hereunder becomes subject to withholding taxes with respect to Licensor's gross or net income under the laws of any jurisdiction, then Licensee will deduct and withhold the amount of such taxes for the account of Licensor to the extent required by law and will pay the amounts of such taxes to the proper governmental authority in a timely manner and promptly transmit to Licensor appropriate proof of payment of such withholding taxes. At the request of Licensor, Licensee will give Licensor such reasonable assistance, which will include the provision of appropriate certificates of such deductions made together with other supporting documentation as may be required by the relevant tax authority, to enable Licensor to claim exemption from or reduction of, or otherwise obtain repayment of, such withholding taxes, and will upon request provide such additional documentation from time to time as is reasonably required to confirm the payment of withholding tax.

ARTICLE 4: DILIGENCE

4.1 Diligence Obligations. Licensee will use commercially reasonable efforts to develop, commercialize, market, promote, and sell Licensed Products in the Field. Commercially reasonable efforts means efforts equivalent to those utilized by ****. Without limiting the foregoing, Licensee will meet the following: acceptance by the FDA or foreign equivalent of an investigational new drug application for a Licensed Product in the CLN1 Field and CLN3 Field by no later than****; provided, however, that, if Licensee expects not to achieve the milestone on or before the specified deadline in either the CLN1 Field or the CLN3 Field, Licensee may extend any deadline in either or both Field(s) for an additional **** per extension by paying Licensor an extension fee of **** per Field on or before such deadline and the deadline shall then be extended by an additional ****. Licensee will only be****.

4.2 Development Plans.

4.2.1 For each Licensed Product in the Field, Licensee will prepare and deliver to Licensor a development plan and budget (each a "Development Plan"). The initial Development Plans for the Licensed Product in the Field will be delivered within **** after the Effective Date.

4.2.2 Each Development Plan will cover the next 2 years, and will include future development activities to be undertaken by Licensee, its Affiliates, or any Sublicensees during the next reporting period under Section 4.3 relating directly to the Licensed Product, Licensee's strategy to bring the Licensed Product to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy.

4.2.3 Following receipt by Licensor of each Development Plan, Licensor will promptly notify Licensee of any comments or requested revisions, and the Parties will thereupon negotiate any appropriate revisions in good faith. With respect to development milestones to be set forth in the initial Development Plans for the Field, the Parties will agree upon reasonable milestones and completion dates to be set forth in the Development Plan (and any amendments thereto).

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4.3 Reporting. Within **** after the Effective Date and within **** of each December 1 thereafter, Licensee shall provide Licensor with written progress reports, setting forth in such detail as Licensor may reasonably request, the progress of the development, evaluation, testing, and commercialization of each Licensed Product. Licensee will also notify Licensor within **** of the First Commercial Sale by Licensee, its Affiliates, or any Sublicensees of each Licensed Product. Such a report ("Development Progress Report"), setting forth the current stage of development of Licensed Products, shall include:

4.3.1 Date of Development Progress Report and time covered by such report;

4.3.2 Major activities and accomplishments completed by Licensee, its Affiliates, and any Sublicensees relating directly to the Licensed Products since the last Development Progress Report;

4.3.3 Significant research and development projects relating directly to the Licensed Products currently being performed by Licensee, its Affiliates, and any Sublicensees and good faith, but non-binding, projected dates of completion;

4.3.4 A development plan covering the next two years at least, which will include future development activities to be undertaken by Licensee, its Affiliates, or any Sublicensees during the next reporting period relating directly to the Licensed Products, Licensee's strategy to bring the Licensed Products to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy;

4.3.5 Projected total development remaining before product launch of each Licensed Product; and

4.3.6 Summary of significant development efforts using the Licensed Technology being performed by Third Parties, including the nature of the relationship between Licensee and such Third Parties.

4.4 Confidential Information. The Parties agree that Development Progress Reports shall be deemed Licensee's Confidential Information; provided that Licensor may share a copy of such reports with the REGENXBIO Licensors.

4.5 Improvements. Simultaneously with the Development Progress Report, Licensee shall deliver a detailed description of any Licensed Back Improvements, if not previously provided pursuant to Section 2.5.3.

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CONFIDENTIAL TREATMENT REQUESTED

ARTICLE 5: CONFIDENTIALITY

5 . 1 Treatment of Confidential Information. Each Party, as a receiving party (a "Receiving Party"), agrees that it will (a) treat Confidential Information of the other Party (the "Disclosing Party") as strictly confidential; (b) protect the Confidential Information of the Disclosing Party with at least the same degree of care as it protects its own confidential and proprietary information, and in any event with not less than a reasonable degree of care; (c) not disclose such Confidential Information to Third Parties without the prior written consent of the Disclosing Party, except as may be permitted in this Agreement; provided that any disclosure permitted hereunder shall be under confidentiality agreements with provisions at least as stringent as those contained in this Agreement; and (d) not use such Confidential Information for purposes other than those authorized expressly in this Agreement. The Receiving Party agrees to ensure that its employees who have access to Confidential Information are obligated in writing to abide by confidentiality obligations at least as stringent as those contained under this Agreement.

5.2 Public Announcements.

5.2.1 The Parties agree they will issue a joint press release in the form attached hereto as Exhibit C. Except as provided in Section 5.2.2, any press releases by either Party with respect to the other Party or any other public disclosures concerning the existence of or terms of this Agreement shall be subject to review and approval by the other Party. Once the joint press release or any other written statement is approved for disclosure by both Parties, either Party may make subsequent public disclosure of the contents of such statement without the further approval of the other Party.

5.2.2 Notwithstanding Section 5.2.1, Licensor has the right to publish (through press releases, scientific journals, or otherwise) and refer to any clinical, regulatory, or research results related to Licensee's Licensed Product or AAV9 program that have been publicly disclosed by Licensee, including referring to Licensee by name as a licensee of Licensor, which publication or referral by Licensor shall not require the prior consent of Licensee.

5 . 3 Authorized Disclosure. Notwithstanding the provisions of Section 5.1 or 5.2, either Party may disclose the other's Confidential Information or make such a disclosure of the existence of and/or terms of this Agreement to any ****; provided that, in each case, such recipient of Confidential Information is obligated to keep such information confidential on terms no less stringent than those set forth in this Agreement. Furthermore, Licensee agrees that Licensor may share a copy of this Agreement, reports and notices provided by Licensee to Licensor pursuant to the terms of this Agreement, and copies of sublicense agreements provided to Licensor hereunder, with the REGENXBIO Licensors to the extent required by the GSK Agreement and the Penn Agreement, under confidentiality. In the event that the Receiving Party receives service of legal process that purports to compel disclosure of the Disclosing Party's Confidential Information or becomes obligated by law, rule, regulation or rules of a security exchange, to disclose the Confidential Information of the Disclosing Party or the existence of or terms of this Agreement to any governmental authority, then, to the extent legally permitted, the Receiving Party shall promptly notify the Disclosing Party, so that the Disclosing Party may seek an appropriate protective order or other remedy with respect to narrowing the scope of such requirement and/or waive compliance by the Receiving Party with the provisions of this Agreement. The Receiving Party will, at the Disclosing Party's request and expense, provide the Disclosing Party with reasonable assistance in obtaining such protective order or other remedy. If, in the absence of such protective order or other remedy, the Receiving Party is nonetheless required by law, rule, regulation or rules of a security exchange, to disclose the existence of or terms of this Agreement or other Confidential Information of the Disclosing Party, then the Receiving Party may disclose such Confidential Information without liability hereunder; provided that the Receiving Party shall furnish only such portion of the Confidential Information that is legally required to be disclosed and only to the extent required by law.

5 . 4 Term of Confidentiality. The obligations of this Article 5 shall continue for a period of **** following the expiration or termination of this Agreement.

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

CONFIDENTIAL TREATMENT REQUESTED

ARTICLE 6: TERM AND TERMINATION

6 . 1 Term of Agreement . This Agreement will commence on the Effective Date and continue in effect on a country-by-country, Licensed Product-by-Licensed Product basis until the later of: (i) the expiration, lapse, abandonment, or invalidation of the last Valid Claim of the Licensed Patents to expire, lapse, become abandoned, become unenforceable for the applicable Licensed Product in the applicable country, or (ii) 10 years from the First Commercial Sale of the applicable Licensed Product in the applicable country, unless sooner terminated in accordance with Section 6.2, Section 6.3, Section 6.4 or Section 6.5 of this Agreement. Upon expiration of the Agreement with respect to a Licensed Product in a country, the license grant to Licensee pursuant to Section 2.1 shall become irrevocable, perpetual, fully paid-up and royalty-free with respect such Licensed Product and such country.

6.2 Licensee's Right to Terminate. Licensee may, upon six months' prior written notice to Licensor, terminate this Agreement for any reason, with or without cause. In exercising such termination right, Licensee may terminate the Agreement in its entirety or, if desired, Licensee may specify in the written notice that this Agreement is terminating only with respect to one or more Field.

6.3 Termination for Breach.

6.3.1 Licensor may terminate this Agreement, if Licensee is late in paying to Licensor royalties, fees, or any other monies due under this Agreement, and if Licensee does not pay Licensor in full within 15 days upon written demand from Licensor, which termination shall be effective immediately upon the expiration of such 15-day cure period.

6.3.2 Either Party may terminate this Agreement, if the other Party materially breaches this Agreement and does not cure such material breach within 30 days after written notice of the breach, which termination shall be effective immediately upon the expiration of such 30-day cure period. Notwithstanding the above, if Licensee disputes in good faith that such material breach exists, and gives Licensor written notice of such dispute within 30 days following Licensee's receipt of Licensor's notice of default, then, Licensor may not terminate this agreement until the dispute is resolved in accordance with Section 10.6; provided that Licensor shall be entitled to terminate this Agreement at the end of the original 30-day cure period, without waiting for resolution of the dispute in accordance with Section 10.6, if the breach by Licensee of this Agreement would cause Licensor to be in breach of the GSK Agreement or the Penn Agreement.

6 . 4 Termination for Insolvency . Licensor shall have the right to terminate this Agreement, upon notice to the Licensee, in the event that:

(a) Licensee shall have: (i) voluntarily commenced any proceeding or filed any petition seeking relief under the bankruptcy, insolvency or other similar laws of any jurisdiction, (ii) applied for, or consented to, the appointment of a receiver, trustee, custodian, sequestrator, conciliator, administrator or similar official for it or for all or substantially all of its property, (iii) filed an answer admitting the material allegations of a petition filed against or in respect of it in any such proceeding, (iv) made a general assignment for the benefit of creditors of all or substantially all of its assets, (v) admitted in writing its inability to pay all or substantially all of its debts as they become due, or (vi) taken corporate action for the purpose of effecting any of the foregoing; or

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(b) An involuntary proceeding shall have been commenced, or any involuntary petition shall have been filed, in a court of competent jurisdiction seeking: (i) relief in respect of Licensee, or of its property, under the bankruptcy, insolvency or similar laws of any jurisdiction, (ii) the appointment of a receiver, trustee, custodian, sequestrator, conciliator, administrator or similar official for the Licensee or for all or substantially all of its property, or (iii) the winding-up or liquidation of the Licensee; and, in each case, such proceeding or petition shall have continued undismissed for 60 days, or an order or decree approving or ordering any of the foregoing shall have continued unstayed, unappealed and in effect for 30 days.

6.5 Patent Challenge.

6.5.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, upon the commencement by Licensee or any of its Affiliates of a Patent Challenge.

6.5.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee commences a Patent Challenge; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee commences a Patent Challenge. If a Sublicensee commences a Patent Challenge and Licensee fails to terminate the applicable sublicense agreement, then Licensor may terminate this Agreement, effective immediately upon written notice to the Licensee.

6.5.3 For purposes of this Section 6.5, "Patent Challenge" means any action against Licensor or the REGENXBIO Licensors, including an action for declaratory judgment, to declare or render invalid or unenforceable the Licensed Patents, or any claim thereof.

6.6 Effects of Termination. The effects of termination by Licensee pursuant to Section 6.2, by either Party, as applicable, under Section 6.3, or by Licensor pursuant to Section 6.4 or 6.5 shall be as follows:

6.6.1 The applicable licenses granted by Licensor hereunder shall terminate, and Licensee, its Affiliates, and (unless the sublicense agreement is assigned pursuant to Section 6.6.2) all Sublicensees shall cease to make, have made, use, import, sell, and offer for sale all Licensed Products and shall cease to otherwise practice the Licensed Technology under the terminated licenses; provided that Licensee, its Affiliates, and its Sublicensees shall have the right to continue to sell their existing inventories of Licensed Products under the terminated licenses for a period not to exceed **** after the effective date of such termination;

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

6.6.2 If termination is by Licensor pursuant to Sections 6.3, 6.4 or 6.5, then, at Licensor's request, Licensee shall assign to Licensor any or all sublicenses granted to Third Parties to the extent of the rights licensed to Licensee hereunder and sublicensed to the Sublicensee; provided that (i) prior to such assignment, Licensee shall advise Licensor whether such Sublicensee is then in full compliance with all terms and conditions of its sublicense and continues to perform thereunder, and, if such Sublicensee is not in full compliance or is not continuing to perform, Licensor may elect not to have such sublicense assigned; and (ii) following such assignment, Licensor shall not be liable to such Sublicensee with respect to any obligations of Licensee to the Sublicensee that are not consistent with, or not required by, Licensor's obligations to Licensee under this Agreement; and all sublicenses not requested to be assigned to Licensor shall terminate. If termination is for any other reason, then all sublicenses shall terminate;

6.6.3 If termination is by Licensee pursuant to Section 6.2 or by Licensor pursuant to Section 6.3, 6.4, or 6.5, then Licensee shall grant, and hereby grants, to Licensor a non-exclusive, perpetual, irrevocable, worldwide, royalty-free, transferable, sublicensable license under any patentable modifications or improvements (and any intellectual property rights with respect thereto) developed by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim within any of the Licensed Patents, for use by Licensor for the research, development, and commercialization of products in any therapeutic indication;

6.6.4 Licensee shall pay all monies then-owed to Licensor under this Agreement;

6.6.5 Each Receiving Party shall, at the Disclosing Party's request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party's obligations; and

6.6.6 If termination is with respect to one or more Field, but not all Fields, then the provisions of this Section 6.6 shall only apply with respect to the terminated Field(s), and this Agreement shall continue with respect to the non-terminated Field(s); provided that if termination is by Licensee pursuant to Section 6.2 with respect to one or more Fields, but not all Fields at any time, then the amount of any unpaid annual fee(s) due under Section 3.2 shall be reduced by **** for each terminated Field; provided further that such reduction shall not apply to the amount due under Section 3.2(i).

6 . 7 Survival. Licensee's obligation to pay all monies due and owed to Licensor under this Agreement that have matured as of the effective date of termination or expiration shall survive the termination or expiration of this Agreement. In addition, the provisions of Section 2.2, (Retained Rights), Section 2.3 (Government Rights), Section 2.5 (Improvements), Article 3 (Consideration) (with respect to any final reports or to the extent any amounts are due but unpaid), Section 3.6 (Reports and Records), Section 4.4 (Confidential Information), Article 5 (Confidentiality), Article 6 (Term and Termination), Section 8.4 (Disclaimer of Warranties, Damages), Section 8.5 (Indemnification), Section 8.6 (Insurance), Article 9 (Use of Name), and Article 10 (Additional Provisions) shall survive such termination or expiration of this Agreement in accordance with their respective terms.

ARTICLE 7: PATENT MAINTENANCE; PATENT INFRINGEMENT

7.1 Prosecution of Licensed Patents. As between Licensor and Licensee, the Parties agree as follows:

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

7.1.1 Licensor shall have the sole right, but not the obligation, to Prosecute patent applications and issued patents within Licensed Patents, in Licensor's sole discretion. Subject to Section 7.1.3, Licensor shall provide Licensee with a reasonable opportunity to review and provide comments in connection with the Prosecution of the Licensed Patents; and Licensor shall keep Licensee reasonably informed as to all material developments with respect to such Licensed Patents and shall supply to Licensee copies of material communications received and filed in connection with the Prosecution of such Licensed Patents.

7.1.2 Nothing in this Agreement obligates Licensor to continue to Prosecute any patent applications or issued patents, and Licensee acknowledges that Licensor shall have no obligation to undertake any inter-party proceedings, such as oppositions, inter partes review, or interferences, or to undertake any re-examination or re-issue proceedings, in either case, with respect to the Licensed Patents.

7.1.3 Licensee acknowledges that The Trustees of the University of Pennsylvania control Prosecution of the Licensed Patents, with Licensor having certain rights to review. Licensee acknowledges and agrees that (a) the rights and obligations under this Section 7.1 are subject to the rights of the REGENXBIO Licensors set forth in the GSK Agreement and Penn Agreement with respect to the Licensed Patents, and (b) Licensor's obligations under this Agreement only apply to the extent of Licensor's rights with respect to participation in Prosecuting the Licensed Patents under the GSK Agreement and the Penn Agreement. Licensor shall have the sole right, but not the obligation, to Prosecute patent applications and issued patents within Licensed Patents, in Licensor's sole discretion.

7.2 Infringement Actions Against Third Parties.

7.2.1 Licensee is responsible for notifying Licensor promptly of any infringement of Licensed Patents (other than Retained Rights) that may come to Licensee's attention, including any "patent certification" filed in the United States under 21 U.S.C. § 355(b)(2) or 21 U.S.C. § 355(j)(2) or similar provisions in other jurisdictions alleging the invalidity, unenforceability or non-infringement of any Licensed Patents, and any notification received pursuant to subsection (k) of 42 U.S.C. § 262 for any Licensed Product that becomes a "reference product." However, Licensee is under no obligation to search for potential infringers.

7.2.2 As between Licensor and Licensee, but subject to any obligations of Licensor to the REGENXBIO Licensors, Licensor shall have the sole right, but not the obligation, to prosecute any such infringement ****. In any action to enforce any of the Licensed Patents, Licensee, at the request and expense of Licensor, shall cooperate to the fullest extent reasonably possible, including in the event that, if Licensor is unable to initiate or prosecute such action solely in its own name, Licensee shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute, maintain, and settle such action. Nothing in this Agreement obligates Licensor to bring or prosecute lawsuits against Third Parties for infringement of any Licensed Patents.

7.2.3 Licensee shall have no right to undertake prosecution of any such infringement.

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7.3 Defense of Infringement Claims. In the event Licensee or Licensor becomes aware that Licensee's or any of its Affiliates' or any Sublicensees' practice of the Licensed Patents is the subject of a claim for patent infringement by a Third Party, that Party shall promptly notify the other, and the Parties shall consider the claim and the most appropriate action to take. Licensee shall cause each of its Affiliates and each Sublicensee to notify Licensee promptly in the event such entity becomes aware that its practice of the Licensed Patents is the subject of a claim of patent infringement by another. To the extent Licensor takes any action, Licensor (or the REGENXBIO Licensors) shall have the right to require Licensee's reasonable cooperation in any such suit, upon written notice to Licensee; and Licensee shall have the obligation to participate upon Licensor's request, in which event, Licensor shall bear the cost of Licensee's participation. Without Licensor's prior written permission, which shall not be unreasonably withheld or denied, Licensee must not settle or compromise any such suit in a manner that imposes any material obligations or restrictions on Licensor or the REGENXBIO Licensors or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

ARTICLE 8: COVENANTS, WARRANTIES; INDEMNIFICATION

8.1 Covenant not to Sue. Licensor agrees that it shall not commence any action against Licensee or any Third Party solely for the work that Third Party is performing on behalf of Licensee for **** prior to the Effective Date.

8.2 Representations and Warranties by Licensor. Licensor represents and warrants to Licensee as of the Effective Date:

8.2.1 Licensor has the right, power, and authority to enter into this Agreement and to grant to Licensee the rights specified in this Agreement;

8.2.2 This Agreement when executed shall become the legal, valid, and binding obligation of it, enforceable against it, in accordance with its terms;

8.2.3 There are no actions, suits, proceedings, or arbitrations pending or, to Licensor's knowledge, threatened against Licensor relating to the Licensed Patents that would be inconsistent with the rights granted to Licensee under this Agreement;

8.2.4 To Licensor's knowledge, (a) the Licensed Patents are solely owned by The Trustees of the University of Pennsylvania, and (b) no Third Party (other than the REGENXBIO Licensors) has any right, interest, or claim in or to such Licensed Patents in the Fields that are inconsistent with those granted to Licensee in the Fields under this Agreement (it being understood that the rights previously granted in the Conflicting License shall not be deemed to conflict with the licenses granted under this Agreement);

8.2.5 Licensor has not received any written notice from any Third Party patentee alleging infringement of such Third Party's patents by the practice of the Licensed Patents in the Fields; and

8.2.6 Licensor shall not, without the prior written consent of Licensee (such consent not to be unreasonably withheld or delayed), amend the Conflicting License in a manner that would materially and adversely affect Licensee's rights and benefits under this Agreement during the term of this Agreement.

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8.3 Representations and Warranties by Licensee. Licensee represents and warrants to Licensor as of the Effective Date that:

8.3.1 Licensee has the right, power, and authority to enter into this Agreement and to grant the rights granted by it hereunder;

8.3.2 This Agreement when executed shall become the legal, valid, and binding obligation of it, enforceable against it, in accordance with its terms;

8.3.3 Licensee has the ability and the resources, including financial resources, necessary to carry out its obligations under this Agreement; and

8.3.4 There are no actions, suits, proceedings, or arbitrations pending or, to Licensee's knowledge, threatened against Licensee that would impact Licensee's activities under this Agreement.

8.4 Disclaimer of Warranties, Damages. EXCEPT AS SET FORTH IN SECTION 8.2, THE LICENSED TECHNOLOGY, LICENSED PRODUCTS, AND ALL RIGHTS LICENSED UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS, AND LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, EXCEPT AS SET FORTH IN SECTION 8.2, LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, AND HEREBY DISCLAIMS ALL EXPRESS AND IMPLIED REPRESENTATIONS AND WARRANTIES, (i) OF COMMERCIAL UTILITY, ACCURACY, COMPLETENESS, PERFORMANCE, TITLE, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, VALIDITY OR ENFORCEABILITY OF THE LICENSED TECHNOLOGY, AND PROFITABILITY; OR (ii) THAT THE USE OF THE LICENSED TECHNOLOGY, OR LICENSED PRODUCTS WILL NOT INFRINGE ANY PATENT, COPYRIGHT, TRADEMARK, OR OTHER PROPRIETARY RIGHTS OF THIRD PARTIES. EXCEPT AS SET FORTH HEREIN, NONE OF LICENSOR AND THE REGENXBIO LICENSORS SHALL BE LIABLE TO LICENSEE, LICENSEE'S SUCCESSORS OR ASSIGNS, ANY SUBLICENSEES, OR ANY THIRD PARTY WITH RESPECT TO: (a) ANY CLAIM ARISING FROM USE OF THE LICENSED TECHNOLOGY, LICENSED PRODUCTS, AND ANY OR ALL RIGHTS LICENSED UNDER THIS AGREEMENT OR FROM THE DEVELOPMENT, TESTING, MANUFACTURE, USE, OR SALE OF LICENSED PRODUCTS; OR (b) ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL, INCIDENTAL, EXEMPLARY, PUNITIVE, OR CONSEQUENTIAL DAMAGES OF ANY KIND, INCLUDING ANY ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT OR THE EXERCISE OF RIGHTS HEREUNDER, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES. NOTHING IN THIS SECTION 8.4 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER SECTION 8.5 OR TO LIMIT A PARTY'S LIABILITY FOR BREACHES OF ITS OBLIGATION REGARDING CONFIDENTIALITY UNDER ARTICLE 5.

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

8.5 Indemnification.

8.5.1 By Licensee. Licensee shall defend, indemnify, and hold harmless Licensor, the REGENXBIO Licensors, and their respective shareholders, members, officers, trustees, faculty, students, contractors, agents, and employees (individually, a "Licensor Indemnified Party" and, collectively, the "Licensor Indemnified Parties") from and against any and all Third Party liability, loss, damage, action, claim, fee, cost, or expense (including attorneys' fees) (individually, a "Third Party Liability" and, collectively, the "Third Party Liabilities") suffered or incurred by the Licensor Indemnified Parties from claims of such Third Parties that result from or arise out of: ****; provided, however, that Licensee shall not be liable for claims to the extent based on any breach by Licensor of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensor Indemnified Parties. Without limiting the foregoing, Licensee must defend, indemnify, and hold harmless the Licensor Indemnified Parties from and against any Third Party Liabilities resulting from:

(a) any **** or other claim of any kind related to the **** by a Third Party of a Licensed Product that **** by Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors;

(b) any claim by a Third Party that the ****; and

(c) **** conducted by or on behalf of Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors relating to the Licensed Technology or Licensed Products, including any claim by or on behalf of a ****.

8.5.2 Indemnification Procedure. Licensee, as an indemnifying party (an "Indemnifying Party"), shall not be permitted to settle or compromise any claim or action giving rise to Third Party Liabilities in a manner that imposes any restrictions or obligations on Licensor, the REGENXBIO Licensors or any indemnified party (an "Indemnified Party") without Licensor's prior written consent that grants any rights to the Licensed Technology or Licensed Products other than those Licensee has the right to grant under this Agreement without Licensor's prior written consent. The Indemnifying Party shall be permitted to control any litigation or potential litigation involving the defense of any claim subject to indemnification pursuant to this Section 8.5, including the selection of counsel, with the reasonable approval of the Indemnified Party. If an Indemnifying Party fails or declines to assume the defense of any such claim or action within **** after notice thereof, then the Indemnified Party may assume the defense of such claim or action at the cost and risk of the Indemnifying Party, and any Third Party Liabilities related thereto shall be conclusively deemed a Third Party Liability of the Indemnifying Party. The indemnification rights of an Indemnified Party contained in this Agreement are in addition to all other rights that such Indemnified Party may have at law or in equity or otherwise. The Indemnifying Party will pay directly all Third Party Liabilities incurred for defense or negotiation of any claim hereunder or will reimburse the Indemnified Party for all documented Third Party Liabilities incident to the defense or negotiation of any such claim within **** after the Indemnifying Party's receipt of invoices for such fees, expenses, and charges.

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8.6 Insurance. Licensee will procure and maintain insurance policies for the following coverages with respect to product liability, personal injury, bodily injury, and property damage arising out of Licensee's (and its Affiliates' and any Sublicensees') performance under this Agreement: (a) during the term of this Agreement, comprehensive general liability, including broad form and contractual liability, in a minimum amount of **** combined single limit per occurrence (or claim) and **** in the aggregate annually; (b) prior to the commencement of clinical trials involving Licensed Products and thereafter for a period of not less than **** (or such longer period as Licensee is required by applicable law to continue to monitor the participants in the clinical trial), clinical trials coverage in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and **** in the aggregate annually; and (c) from prior to the First Commercial Sale of a Licensed Product until **** after the last sale of a Licensed Product, product liability coverage, in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and **** in the aggregate annually. Licensor may review periodically the adequacy of the minimum amounts of insurance for each coverage required by this Section 8.6, and Licensor reserves the right to require Licensee to adjust the limits accordingly. The required minimum amounts of insurance do not constitute a limitation on Licensee's liability or indemnification obligations to the Licensor Indemnified Parties under this Agreement. The policies of insurance required by this Section 8.6 will be issued by an insurance carrier with an A.M. best rating of **** or better and will name Licensor as an additional insured with respect to Licensee's performance (and its Affiliates' and any Sublicensees') under this Agreement. Licensee will provide Licensor with insurance certificates evidencing the required coverage within **** after the Effective Date and the commencement of each policy period and any renewal periods. Each certificate will provide that the insurance carrier will notify Licensor in writing at least **** prior to the cancellation or material change in coverage. Licensee will cause all Sublicensees to comply with the terms of this Section 8.6 to the same extent as Licensee.

ARTICLE 9: USE OF NAME

9.1 Licensee, its Affiliates, any Sublicensees, and all of its and their employees and agents must not use Licensor's, the University of Pennsylvania's, or SmithKline Beecham Corporation's name, seal, logo, trademark, or service mark (or any adaptation thereof) or the name, seal, logo, trademark, or service mark (or any adaptation thereof) of any of such entities' representative, school, organization, employee, or student in any way without the prior written consent of Licensor or such entity, as applicable, unless required to do so pursuant to applicable law, rule, regulation or rules of a securities exchange; provided, however that Licensee may acknowledge the existence and general nature of this Agreement, subject to Section 5.2 or 5.3, as applicable.

9.2 Licensor and all of its employees and agents must not use Licensee's name, seal, logo, trademark, or service mark (or any adaptation thereof) in any way without the prior written consent of Licensee; provided, however that Licensor may acknowledge the existence and general nature of this Agreement, subject to Section 5.2 or 5.3, as applicable, and refer to Licensee as a licensee of Licensor.

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

CONFIDENTIAL TREATMENT REQUESTED

ARTICLE 10: ADDITIONAL PROVISIONS

10.1 Relationship. Nothing in this Agreement shall be deemed to establish a relationship of principal and agent between Licensee and Licensor, nor any of their agents or employees for any purpose whatsoever, nor shall this Agreement be construed as creating any other form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party.

10.2 Assignment. The rights and obligations of Licensee and Licensor hereunder shall inure to the benefit of, and shall be binding upon, their respective permitted successors and assigns. Licensee may not assign or otherwise transfer (by operation of law or otherwise) this Agreement or any of its rights or obligations under this Agreement without the prior written consent of Licensor, which consent is in the absolute discretion of Licensor (except Licensee shall have the right to assign this Agreement without Licensor's consent to a wholly owned Affiliate, in which case Licensee shall remain responsible for the performance of this Agreement by such Affiliate); provided, however, Licensee shall be permitted to transfer (by operation of law or otherwise) this Agreement without Licensor's consent in connection with a Change of Control; provided that, Licensee: (i) requires any transferee or successor to agree in writing to be legally bound by this Agreement to the same extent as Licensee and provides Licensor with a copy of such undertaking; (ii) provides Licensor with written notice of the Change of Control to Licensor within five days of the consummation of the transaction resulting in a Change of Control of Licensee; (iii) provides Licensor with a copy of the definitive agreement for the Change of Control of Licensee with five days of the consummation of the transaction (provided, that Licensee shall be entitled to include customary redactions in such copy provided to Licensor, to the extent such redacted information is not necessary to verify compliance with the terms of this Agreement or otherwise required by the Penn Agreement and/or GSK Agreement); and (iv) makes all payments required as a result of a Change of Control under Article 3. Notwithstanding anything to the contrary in this Agreement, for clarity, in case of a Licensee Change of Control, in no event shall any intellectual property rights owned or controlled by the acquirer or its Affiliates immediately prior to such Licensee Change of Control be included in any of the licenses granted to Licensor under this Agreement. Licensor may assign this Agreement and its rights and obligations without the consent of Licensee. No assignment shall relieve the assigning Party of responsibility for the performance of any accrued obligations that it has prior to such assignment. Any attempted assignment by Licensee in violation of this Section 10.2 shall be null and void and of no legal effect.

10.3 Waiver. A waiver by either Party of a breach of any provision of this Agreement will not constitute a waiver of any subsequent breach of that provision or a waiver of any breach of any other provision of this Agreement.

10.4 Notices. Notices, payments, statements, reports, and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date received if sent by public courier (*e.g.*, Federal Express), by Express Mail, receipt requested, by facsimile, or by electronic mail (with a copy of such facsimile or electronic mail also sent by one of the other methods of delivery) and addressed as follows:

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

If for Licensor:

REGENXBIO Inc.
9600 Blackwell Road
Suite 210
Rockville, MD 20850
USA
Attn: Chief Executive Officer
Telephone: 240-552-8181
Facsimile: 240-652-9692

with a copy to:

REGENXBIO Inc.
9600 Blackwell Road
Suite 210
Rockville, MD 20850
USA
Attn: General Counsel
Telephone: 240-552-8181
Facsimile: 240-652-9692

If for Licensee:

Abeona Therapeutics Inc.
1330 Avenue of the Americas, 33rd Floor
New York, NY 10019
USA
Attention: Chief Executive Officer
Telephone: 646-813-4713
Facsimile:

with a copy to:

Morgan, Lewis & Bockius LLP
One Federal Street
Boston, MA 02210
USA
Attention: Jack Concannon, Esq.
Telephone: 617-951-8000
Facsimile: 617-951-7326

Either Party may change its official address upon written notice to the other Party in accordance with this Section 10.4.

10.5 Applicable Law. This Agreement shall be construed and governed in accordance with the laws of the State of New York, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. Subject to Section 10.6, the Parties hereby submit to the exclusive jurisdiction of and venue in the courts located in the State of Delaware with respect to any and all disputes concerning the subject of this Agreement.

10.6 Dispute Resolution. In the event of any controversy or claim arising out of or relating to this Agreement, the Parties shall first attempt to resolve such controversy or claim through good faith negotiations for a period of not less than **** following notification of such controversy or claim to the other Party. If such controversy or claim cannot be resolved by means of such negotiations during such period, then such controversy or claim shall be resolved by binding arbitration administered by the American Arbitration Association ("AAA") in accordance with the Commercial Arbitration Rules of the AAA in effect on the date of commencement of the arbitration, subject to the provisions of this Section 10.6. The arbitration shall be conducted as follows:

10.6.1 The arbitration shall be conducted by three arbitrators, each of whom by training, education, or experience has knowledge of the research, development, and commercialization of biological therapeutic products in the United States. The arbitration shall be conducted in English and held in New York, New York.

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

10.6.2 In its demand for arbitration, the Party initiating the arbitration shall provide a statement setting forth the nature of the dispute, the names and addresses of all other parties, an estimate of the amount involved (if any), the remedy sought, otherwise specifying the issue to be resolved, and appointing one neutral arbitrator. In an answering statement to be filed by the responding Party within **** after confirmation of the notice of filing of the demand is sent by the AAA, the responding Party shall appoint one neutral arbitrator. Within **** from the date on which the responding Party appoints its neutral arbitrator, the first two arbitrators shall appoint a chairperson.

10.6.3 If a Party fails to make the appointment of an arbitrator as provided in Section 10.6.2, the AAA shall make the appointment. If the appointed arbitrators fail to appoint a chairperson within the time specified in Section 10.6.2 and there is no agreed extension of time, the AAA shall appoint the chairperson.

10.6.4 The arbitrators will render their award in writing and, unless all Parties agree otherwise, will include an explanation in reasonable detail of the reasons for their award. Judgment upon the award rendered by the arbitrators may be entered in any court having jurisdiction thereof, including in the courts described in Section 10.5. The arbitrators will have the authority to grant injunctive relief and other specific performance; provided that the arbitrators will have no authority to award damages in contravention of this Agreement, and each Party irrevocably waives any claim to such damages in contravention of this Agreement. The arbitrators will, in rendering their decision, apply the substantive law of the State of Delaware, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. The decision and award rendered by the arbitrators will be final and non-appealable (except for an alleged act of corruption or fraud on the part of the arbitrator).

10.6.5 The Parties shall use their reasonable efforts to conduct all dispute resolution procedures under this Agreement as expeditiously, efficiently, and cost-effectively as possible.

10.6.6 All expenses and fees of the arbitrators and expenses for hearing facilities and other expenses of the arbitration will be borne equally by the Parties unless the Parties agree otherwise or unless the arbitrators in the award assess such expenses against one of the Parties or allocate such expenses other than equally between the Parties. Each of the Parties will bear its own counsel fees and the expenses of its witnesses except to the extent otherwise provided in this Agreement or by applicable law.

10.6.7 Compliance with this Section 10.6 is a condition precedent to seeking relief in any court or tribunal in respect of a dispute, but nothing in this Section 10.6 will prevent a Party from seeking equitable or other interlocutory relief in the courts of appropriate jurisdiction, pending the arbitrators' determination of the merits of the controversy, if applicable to protect the confidential information, property, or other rights of that Party or to otherwise prevent irreparable harm that may be caused by the other Party's actual or threatened breach of this Agreement.

10.7 No Discrimination. Licensee, its Affiliates, and Licensee shall use reasonable efforts to require that any Sublicensees, in their respective activities under this Agreement, shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual, or affectional preference, age, religion, national, or ethnic origin, handicap, or because he or she is a disabled veteran or a veteran (including a veteran of the Vietnam Era).

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

10.8 Compliance with Law. Licensee (and its Affiliates' and any Sublicensees') must comply with all prevailing laws, rules, and regulations that apply to its activities or obligations under this Agreement. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes, and other commodities, articles, and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979 and that Licensee's obligations are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by Licensee that Licensee shall not export data or commodities to certain foreign countries without prior approval of such agency. Licensor neither represents that a license is not required nor that, if required, it will issue.

10.9 Entire Agreement. This Agreement embodies the entire understanding between the Parties relating to the subject matter hereof and supersedes all prior understandings and agreements, whether written or oral, including that certain Mutual Non-Disclosure Agreement dated May 16, 2018 between the Parties. All "Confidential Information" (as defined in such Mutual Non-Disclosure Agreement) disclosed by one Party to the other Party pursuant to such Mutual Non-Disclosure Agreement shall be deemed "Confidential Information" of such disclosing Party under this Agreement (unless and until it falls within one of the exclusions set forth in Section 1.7). This Agreement may not be varied except by a written document signed by duly authorized representatives of both Parties.

10.10 Marking. Licensee, its Affiliates, and any Sublicensees shall mark any Licensed Product (or their containers or labels) made, sold, or otherwise distributed by it or them with any notice of patent rights necessary or desirable under applicable law to enable the Licensed Patents to be enforced to their full extent in any country where Licensed Products are made, used, sold, offered for sale, or imported.

10.11 Severability and Reformation. If any provision of this Agreement is held to be invalid or unenforceable by a court of competent jurisdiction, then such invalid or unenforceable provision will be automatically revised to be a valid or enforceable provision that comes as close as permitted by law to the Parties' original intent; provided that, if the Parties cannot agree upon such valid or enforceable provision, then the remaining provisions of this Agreement will remain in full force and effect, unless the invalid or unenforceable provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid or unenforceable provisions.

10.12 Further Assurances. Each Party hereto agrees to execute, acknowledge, and deliver such further instruments, and to do all other reasonable acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

10.13 Interpretation; Construction. The captions to the several Articles and Sections of this Agreement are included only for convenience of reference and shall not in any way affect the construction of, or be taken into consideration in interpreting, this Agreement. In this Agreement, unless the context requires otherwise, (a) the word "including" shall be deemed to be followed by the phrase "without limitation" or like expression; (b) references to the singular shall include the plural and vice versa; (c) references to masculine, feminine, and neuter pronouns and expressions shall be interchangeable; (d) the words "herein" or "hereunder" relate to this Agreement; (e) "or" is disjunctive but not necessarily exclusive; (f) the word "will" shall be construed to have the same meaning and effect as the word "shall"; (g) all references to "dollars" or "$" herein shall mean U.S. Dollars; (h) unless otherwise provided, all reference to Sections, Articles, and exhibits in this Agreement are to Sections, Articles, and exhibits of and in this Agreement; and (i) whenever this Agreement refers to a number of days, such number shall refer to calendar days unless business days are specified. Business days shall mean a day on which banking institutions in Washington, D.C. are open for business. Each Party 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 will apply against the Party which drafted such terms and provisions.

10.14 Cumulative Rights and Remedies. The rights and remedies provided in this Agreement and all other rights and remedies available to either Party at law or in equity are, to the extent permitted by law, cumulative and not exclusive of any other right or remedy now or hereafter available at law or in equity. Neither asserting a right nor employing a remedy shall preclude the concurrent assertion of any other right or employment of any other remedy, nor shall the failure to assert any right or remedy constitute a waiver of that right or remedy.

10.15 Counterparts. This Agreement may be executed in one or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

CONFIDENTIAL TREATMENT REQUESTED

IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this License Agreement to be executed by their duly authorized representatives.

REGENXBIO INC.

By: /s/ Kenneth Mills
Name: Kenneth Mills
Title: President & CEO

ABEONA THERAPEUTICS INC.

By: /s/ Frank Carsten Thiel
Name: Frank Carsten Thiel
Title: Chief Executive Officer

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A
Licensed Patents (****)

Application #	Patent #	Filing Date	Country	Status
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****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit B
Licensed Know-How

****

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit C
Press Release

REGENXBIO and Abeona Therapeutics Announce Worldwide Exclusive Licenses for the Treatment of Four Rare Lysosomal Storage Disorders Using NAV AAV9 Vector

- *REGENXBIO grants Abeona new licenses to NAV AAV9 for the development and commercialization of treatments for MPS IIIA, MPS IIIB, CLN1 and CLN3 Batten Disease*
- *REGENXBIO could receive up to $180 million, including $40 million in guaranteed payments*

ROCKVILLE, Md., Nov. 5, 2018 (PRNEWSWIRE) — REGENXBIO Inc. (Nasdaq:RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV® Technology Platform, and Abeona Therapeutics Inc. (Nasdaq: ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel cell and gene therapies for life-threatening rare genetic diseases, today announced a license agreement to REGENXBIO's NAV AAV9 vector for the treatment of four diseases: Sanfilippo syndrome type A (MPS IIIA), Sanfilippo syndrome type B (MPS IIIB), Infantile Batten Disease, also known as neuronal ceroid lipofuscinosis type 1 (CLN1 Disease), and Juvenile Batten Disease, also known as neuronal ceroid lipofuscinosis type 3 (CLN3 Disease).

Under the terms of the agreement, REGENXBIO has granted Abeona an exclusive worldwide license (subject to certain non-exclusive rights previously granted for MPS IIIA), with rights to sublicense, to REGENXBIO's NAV AAV9 vector for the development and commercialization of gene therapies for the treatment of MPS IIIA, MPS IIIB, CLN1 Disease and CLN3 Disease. In return for these rights, REGENXBIO will receive a guaranteed $20 million upfront payment, $10 million of which will be paid upon signing and $10 million of which will be paid within 12 months of the effective date. In addition, REGENXBIO will receive a total of $100 million in annual fees, payable upon the second through sixth anniversaries of the agreement, $20 million of which is guaranteed. REGENXBIO is also eligible to receive potential commercial milestone payments of up to $60 million. REGENXBIO will also receive low double-digit royalties on net sales of products incorporating the licensed intellectual property.

"This license agreement further validates the potential of NAV AAV9 for the treatment of systemic and CNS manifestations of lysosomal storage diseases, as well as the strength of our intellectual property portfolio," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are pleased to initiate our partnership with Abeona as they continue to advance multiple programs using NAV AAV9 through and towards clinical trials in indications with significant unmet medical need."

"This agreement is an important milestone that underpins the therapeutic potential we see in our Sanfilippo syndrome and Batten disease programs featuring the NAV AAV9 vector, which have the potential to transform the lives of patients," said Carsten Thiel, Ph.D., Chief Executive Officer of Abeona. "Data from our clinical and preclinical programs and the success of the NAV AAV9 vector observed in other indications strongly positions the platform as a leading technology for investigational gene therapies for the systemic and CNS manifestations of lysosomal storage diseases."

About Sanfilippo Syndrome
Sanfilippo syndrome, or MPS type III, is a group of rare genetic lysosomal storage diseases with no approved treatments. MPS III is characterized by aggressive behavior, seizures, loss of speech or vision, an inability to sleep, and premature death. An estimated 70% of children with MPS III do not reach age 18. The underlying cause of the syndrome is a missing enzyme that is essential to breaking down heparan sulfate. As a result, partially synthesized heparan sulfate accumulates in the central nervous system, including the brain and spinal cord, causing progressive damage. MPS III is categorized by the single gene defects associated with each type of the syndrome - A, B, C or D. The hallmark feature of MPS IIIA is a deficiency in the SGSH enzyme, while MPS IIIB is distinguished by a marked decrease in NAGLU enzyme activity.

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

CONFIDENTIAL TREATMENT REQUESTED

About Batten Disease
Infantile and juvenile forms of Batten disease, known as CLN1 and CLN3, are rare autosomal recessive genetic disorders with no approved treatments. Batten disease is fatal, and most do not live past their twenties or thirties. The underlying cause of the disorder is a deficiency in proteins critical to lysosomal function that lead to abnormal buildup of lipopigments, and result in neuroinflammation and neurodegeneration. CLN1 and CLN3 are differentiated by mutations of their respective genes, yet the first noticeable sign of all forms of Batten disease is often vision impairment that can progress to blindness. Developmental regression is another hallmark of the disease, as children lose the ability to speak in complete sentences and to walk or sit, among other manifestations. Later in life, affected children may have recurrent seizures, heart problems, behavioral problems, and difficulty sleeping.

About REGENXBIO Inc.
REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates in multiple therapeutic areas.

About Abeona Therapeutics Inc.
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and ABO-101 (AAV-NAGLU), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type B (MPS IIIB). Abeona is also developing ABO-201 (AAV-CLN3) gene therapy for CLN3 disease, ABO-202 (AAV-CLN1) for treatment of CLN1 disease, EB-201 for epidermolysis bullosa (EB), ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder and ABO-302 using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, Abeona is developing a proprietary vector platform, AIM™, for next generation product candidates. For more information, visit www.abeonatherapeutics.com.

REGENXBIO Forward-Looking Statements
This press release includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's future operations and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2017 and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (SEC) and are available on the SEC's website at www.sec.gov. All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

CONFIDENTIAL TREATMENT REQUESTED

REGENXBIO CONTACT:

Investors
Natalie Wildenradt, 646-681-8192
natalie@argotpartners.com

Media
Adam Pawluk, 202-591-4063
apawluk@jpa.com

****Certain information has been omitted and filed separately with the Commission. Confidential treatment has been requested with respect to the omitted portions.

EXHIBIT 21

SUBSIDIARIES OF THE REGISTRANT

Abeona Therapeutics LLC, an Ohio company

Abeona Therapeutics Europe, S.L., a Spanish company

MacroChem Corporation, a Delaware company

Virium Pharmaceuticals, Inc., a Delaware company

EXHIBIT 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in Registration Statements on Form S-1 (File No. 333-197220), Form S-3 (File Nos. 333-205128, 333-204179 and 333-224867), and Form S-8 (File Nos. 333-214846, 333-204055, 333-189985, 333-169067, 333-161642, 333-125796 and 333-221552) of our report dated March 18, 2019, relating to the consolidated financial statements of Abeona Therapeutics Inc. and Subsidiaries and our report dated March 18, 2019, relating to effectiveness of internal control over financial reporting appearing in this Annual Report on Form 10-K of Abeona Therapeutics Inc. and Subsidiaries for the year ended December 31, 2018.

/s/ WHITLEY PENN LLP

Plano, Texas
March 18, 2019

EXHIBIT 31.1

PRINCIPAL EXECUTIVE OFFICER CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Steven H. Rouhandeh, certify that:

1. I have reviewed this Annual Report on Form 10-K of Abeona Therapeutics Inc.

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: March 18, 2019

/s/ Steven H. Rouhandeh

Steven H. Rouhandeh
Executive Chairman
Principal Executive Officer

EXHIBIT 31.2

PRINCIPAL FINANCIAL OFFICER CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Christine Silverstein, certify that:

1. I have reviewed this Annual Report on Form 10-K of Abeona Therapeutics Inc.

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: March 18, 2019

/s/ Christine Silverstein

Christine Silverstein
Chief Financial Officer
Principal Financial Officer

EXHIBIT 32

**CERTIFICATION PURSUANT TO 18 U.S.C.
SECTION 1350 AS ADOPTED PURSUANT TO SECTION 906
OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Annual Report of Abeona Therapeutics Inc. (the "Company") on Form 10-K for the year ended December 31, 2018 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), we, Steven H. Rouhandeh, Executive Chairman of the Company, and Christine Silverstein, Chief Financial Officer of the Company, certify, pursuant to 18 U.S.C. 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

(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.

/s/ Steven H. Rouhandeh	/s/ Christine Silverstein
Steven H. Rouhandeh Executive Chairman Principal Executive Officer	Christine Silverstein Chief Financial Officer Principal Financial Officer
Dated: March 18, 2019	Dated: March 18, 2019