Subject to Completion
Preliminary Prospectus dated July 11, 2016

PROSPECTUS

5,000,000 Shares

AUDENTES THERAPEUTICS

Common Stock

This is Audentes Therapeutics, Inc.'s initial public offering. We are selling 5,000,000 shares of our common stock.

We expect the public offering price to be between $14.00 and $16.00 per share. Currently, no public market exists for the shares. We have applied to list our common stock on The NASDAQ Global Market under the symbol "BOLD."

We are an "emerging growth company" as defined under the federal securities laws and, as such, have elected to comply with certain reduced public company reporting requirements.

Investing in the common stock involves risks that are described in the section entitled "Risk Factors" beginning on page 13 of this prospectus.

	Per Share	Total
Public offering price	$	$
Underwriting discounts and commissions(1)	$	$
Proceeds, before expenses, to us	$	$

(1) See the section entitled "Underwriting" for a description of the compensation payable to the underwriters.

Our existing institutional investors associated with our board have indicated an interest in purchasing shares of common stock in this offering with an aggregate value of approximately $30.0 million at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, fewer or no shares in this offering to any of these parties, or any of these parties may determine to purchase more, fewer or no shares in this offering. The underwriters will receive the same underwriting discount on any shares purchased by these entities as they will on any other shares sold to the public in this offering.

The underwriters may also exercise their option to purchase up to an additional 750,000 shares from us, at the public offering price, less the underwriting discounts and commissions, for 30 days after the date of this prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

The shares will be ready for delivery on or about , 2016.

Joint Book-Running Managers

BofA Merrill Lynch **Cowen and Company** **Piper Jaffray**

Co-Manager

Wedbush PacGrow

The date of this prospectus is , 2016.

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Neither we nor the underwriters have authorized anyone to provide any information or to make any representations other than those contained in this prospectus or in any free writing prospectuses we have prepared. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. The information in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of shares of our common stock. Our business, financial condition, results of operations and prospects may have changed since that date.

For investors outside the United States: Neither we nor the underwriters have done anything that would permit our initial public offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than in the United States. Persons outside the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of our common stock and the distribution of this prospectus outside of the United States.

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PROSPECTUS SUMMARY

This summary highlights information contained elsewhere in this prospectus and does not contain all of the information you should consider in making your investment decision. Before deciding to invest in shares of our common stock, you should read this summary together with the more detailed information, including our consolidated financial statements and the accompanying notes, provided elsewhere in this prospectus. You should carefully consider, among other things, the matters discussed in the sections entitled "Risk Factors," "Selected Consolidated Financial Data," our consolidated financial statements and the accompanying notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations," in each case included elsewhere in this prospectus. Some of the statements in this prospectus constitute forward-looking statements that involve risks and uncertainties. See "Special Note Regarding Forward-Looking Statements."

Audentes Therapeutics, Inc.

"Audentes" from the Latin verb *audeo*: Those who have courage; those who have boldness, daring.

Courageous Patients. Bold Effort.

Overview

We are a biotechnology company focused on developing and commercializing gene therapy products for patients suffering from serious, life-threatening rare diseases caused by single gene defects. We believe that gene therapy has powerful potential to treat these diseases through delivery of a functional copy of the affected gene to cells, resulting in production of the normal protein. We have built a compelling portfolio of product candidates, including AT132 for the treatment of X-Linked Myotubular Myopathy, or XLMTM, AT342 for the treatment of Crigler-Najjar Syndrome, or Crigler-Najjar, AT982 for the treatment of Pompe disease and AT307 for the treatment of the CASQ2 subtype of Catecholaminergic Polymorphic Ventricular Tachycardia, or CASQ2-CPVT. We plan to submit Investigational New Drug applications, or INDs, or Clinical Trial Authorisations, or CTAs, for AT982 in the third quarter of 2016, for AT342 in the fourth quarter of 2016 and for AT132 in the first quarter of 2017, and expect to have preliminary data from all three programs in the second half of 2017. We maintain full global rights to all of our product candidates.

Our vision is to become a fully integrated biotechnology company. In pursuit of this goal, we are executing on our core strategic initiatives, which include the development of proprietary in-house manufacturing capabilities and the expansion of our pipeline. We have assembled a world-class team with expertise in gene therapy, rare disease drug development and commercialization, and biologics manufacturing.

Our mission is to dramatically and positively transform the lives of patients suffering from serious, life-threatening rare diseases with limited or no treatment options. For example, we are developing AT132 to treat XLMTM, a disease for which there are no approved therapies and from which approximately 50% of affected children die in the first 18 months of life. We believe our product candidates have the potential to provide long-lasting benefits, changing the lives of patients with these devastating diseases. Given the available clinical and regulatory pathways, we believe that the rarity and severity of the diseases we target may provide advantages for drug development, including the potential for expedited development and regulatory review, and market exclusivity.

We focus on the treatment of rare diseases caused by single gene, or monogenic, defects in DNA that we believe can be effectively addressed using gene therapy. Conventional approaches such as protein therapeutics attempt to replace the deficient protein, but they do not correct the underlying genetic defect causing the disease. In addition, protein therapeutics often require frequent administration by injection or infusion and often result in sub-optimal safety and efficacy. We believe gene therapy is an ideal treatment modality for diseases caused by monogenic defects. Our portfolio of product candidates employs the use of adeno-associated virus, or AAV, a

small, non-pathogenic virus that is genetically engineered to function as a delivery vehicle, or vector, and is administered to a patient to introduce a healthy copy of a mutated gene to the body. AAV gene therapy vectors are modified such that they will not cause an infection like a normal virus, but are capable of delivering therapeutic genes into patients' cells. Vectors derived from AAV have a well-established safety profile in humans and have been shown to effectively deliver genes to the liver, eye, muscle and brain. Preclinical and clinical data demonstrate that AAV vectors are capable of providing durable efficacy with a favorable adverse event profile due at least in part to AAV's low immunogenic potential. AAV vectors can be described by the serotype, or strain, of the original virus isolate that was used to form the outer shell, or capsid, of the vector. We selected AAV8 and AAV9 as our in-licensed vector capsid serotypes, based on their biological properties, which we believe will translate into a positive clinical effect in our target indications. For example, we believe AAV8 is advantageous for the treatment of Crigler-Najjar given its ability to penetrate the liver, the primary organ implicated in this disease pathology.

Our business model is to develop and commercialize a broad portfolio of gene therapy product candidates to treat rare diseases. We use a focused set of criteria to select product candidates that we believe have the best chance of success. These criteria include:

- serious, life-threatening rare diseases;
- monogenic diseases with well-understood biology;
- disease characteristics well-suited for treatment with AAV gene therapy technology;
- high potential for meaningful clinical benefit;
- compelling preclinical data;
- clear measures for evaluation in clinical trials; and
- opportunities for expedited development through established regulatory pathways.

We have built a portfolio of gene therapy product candidates and we intend to further expand our portfolio over time. Set forth below is a table summarizing our development programs.

Product Candidate *(Indication)*	Stage of Development				Planned IND/CTA Submission	Preliminary Data Expected	Commercial Rights
	Discovery	Lead Optimization	IND-Enabling	Phase 1/2			
AT132 *(XLMTM)*					Q1 2017	Q4 2017	AUDENTES THERAPEUTICS
AT342 *(Crigler-Najjar)*					Q4 2016	Q4 2017	AUDENTES THERAPEUTICS
AT982 *(Pompe disease)*					Q3 2016	H2 2017	AUDENTES THERAPEUTICS
AT307 *(CASQ2-CPVT)*					2017		AUDENTES THERAPEUTICS

AT132. We are developing AT132, an AAV8 vector containing a functional copy of the MTM1 gene, for the treatment of XLMTM. XLMTM is characterized by extreme muscle weakness, respiratory failure and

early death with an estimated 50% mortality rate by 18 months of age. The incidence of XLMTM is estimated to be one in 50,000 male births. Currently, only supportive treatment options, such as ventilator use or a feeding tube, are available. Infants with XLMTM are typically born with severe muscle weakness and the majority require chronic mechanical ventilation from birth. Of the patients that survive the infantile period, most are severely incapacitated and do not have a life expectancy beyond early adolescence.

The disease is the result of mutations in the MTM1 gene that affect the production of myotubularin, an enzyme required for normal development and function of skeletal muscle. Mutations in the MTM1 gene result in production of too little or no functional protein. Importantly, we believe that even a modest increase of functional protein may have a significant therapeutic benefit for XLMTM patients. We believe AT132 may provide patients with significantly improved outcomes based on the ability of AAV8 to preferentially treat skeletal muscle, and has the potential to provide long-term clinical benefit to XLMTM patients through persistent expression of the functional protein following a single intravenous administration. We have two robust animal models of XLMTM, a genetically engineered murine model and a naturally occurring canine model. Both models present with disease symptoms similar to that of humans including severe muscle weakness, respiratory failure and early death.

Preclinical study results in both canine and murine models of the disease demonstrated dramatic improvements in all outcomes, including histology, muscle strength, respiratory function and survival. In the naturally occurring Laborador Retriever model, symptom onset occurs at nine to ten weeks of age, and disease progression leads to death at approximately 18 weeks of age. Multiple studies in this model have demonstrated that a single administration of AT132 significantly improves all disease symptoms and survival rates. In two dogs treated in one of our earliest studies, these effects have lasted approximately three and a half years to date and the dogs continue to thrive. Our goal is to achieve these same benefits in XLMTM patients following a single intravenous administration of AT132.

AT342. Crigler-Najjar is a rare, congenital autosomal recessive monogenic disease characterized by severely high levels of bilirubin in the blood and risk of irreversible neurological damage and death. Average life expectancy is reported as being 30 years of age with phototherapy. Crigler-Najjar is estimated to affect approximately one in 1,000,000 newborns. Infants with Crigler-Najjar develop severe jaundice shortly after birth resulting in rapid presentation and diagnosis. Crigler-Najjar is caused by mutations in the gene encoding the UGT1A1 (uridine-diphosphate (UDP)-glucuronosyltransferase (UGT) 1A1) enzyme resulting in an inability to convert unconjugated bilirubin to a water-soluble form that can be excreted from the body. Clinical diagnosis is confirmed via genetic testing of the UGT1A1 gene. The current standard of care for Crigler-Najjar is aggressive management of high bilirubin levels with persistent, daily phototherapy, usually for longer than 12 hours per day using intense fluorescent light focused on the bare skin, while the eyes are shielded. Phototherapy speeds bilirubin decomposition and excretion, lowering serum bilirubin levels. Phototherapy wanes in effectiveness beginning around age four due to thickening of the skin and a reduction in surface area to body mass ratio, and a liver transplant may be required for survival.

We are developing AT342, an AAV8 vector containing a functional version of the UGT1A1 gene. Preclinical data in murine models of the disease demonstrate AAV8-UGT1A1 significantly reduces bilirubin levels, even at UGT1A1 liver expression levels of just five to eight percent of normal. We are advancing AT342 with the goal of administering a single dose that results in a significant, durable reduction in serum bilirubin, a reduction in or elimination of lengthy daily phototherapy, and elimination of the need for a liver transplant. We believe that serum bilirubin levels will be a clinically relevant endpoint and that determination of efficacy of AT342 will be straightforward due to the ease and reliability of measurement.

AT982. Pompe disease is a serious, progressive genetic disease characterized by severe muscle weakness, respiratory failure leading to ventilator dependence and, in infants, increased cardiac mass and heart failure. In untreated infants, the disease is often fatal due to cardio-respiratory failure within the first year of life. The overall incidence is estimated to be approximately one in 40,000 people although frequency and disease

progression varies with age of onset, ethnicity and geography. Pompe disease is caused by mutations in the gene encoding the lysosomal enzyme alpha-glucosidase, or GAA, which results in a deficiency of GAA protein and leads to the accumulation of glycogen. GAA is responsible for degrading glycogen within the lysosome, and dysfunction or absence of functional GAA results in toxic accumulation of glycogen in cells. Tissues and cells most affected by the disease are predominantly skeletal muscle, cardiac muscle and motoneurons. The only approved treatment for Pompe disease is enzyme replacement therapy, or ERT, which is a chronic treatment delivered in bi-weekly intravenous infusions. Despite the availability of ERT, significant medical need still persists, which is primarily due to the inability of ERT to penetrate key tissues affected by the disease and immunogenicity of ERT treatment.

We believe our approach with AT982, which uses an AAV serotype 9 capsid vector containing a functional copy of the GAA gene, can overcome the limitations of ERT and provide long-term improvement in patient symptoms. Further, we believe AT982 may provide patients with superior outcomes based on the ability of AAV9 to penetrate key cells and tissues affected by the disease, such as motoneurons, which are not effectively treated with ERT. Preclinical studies of AT982 have been conducted in a robust and well established genetically modified murine model of Pompe disease. In these studies, treatment resulted in improvement in several measures of efficacy, including enzyme activity, glycogen clearance and skeletal muscle, cardiac and respiratory function. We believe intracellular production of the therapeutic protein may improve efficacy, reduce immunogenicity and deliver a durable therapeutic effect with a single intravenous administration.

AT307. CASQ2-CPVT is a rare monogenic disease that is characterized by life-threatening arrhythmias that may lead to sudden cardiac death. There are currently only limited treatment options with variable efficacy for patients suffering from CPVT, including beta-blockers and a sodium channel blocker. The autosomal recessive form of the disease is caused by mutations in the calsequestrin 2 gene, or CASQ2 gene, and is characterized by stress-induced heartbeat rhythm changes in an otherwise structurally normal heart. The CASQ2 protein plays a key role in the release of calcium within the cardiac muscle cell, which is necessary for normal cardiac contractile function to maintain normal heart rhythm. It is estimated that CPVT occurs in one in 10,000 people, with approximately 2% to 5% due to mutations in the CASQ2 gene. This equates to an approximate prevalence of 6,000 affected people in North America, Europe and other addressable markets. The number of identified cases is likely to increase with the advent of more accessible genetic testing. It is estimated that 30% of people with CASQ2-CPVT will have had a cardiac event by the age of ten, and 79% will have had an event by the age of 40. Untreated, mortality is reported to be in the range of 30% to 50% by the age of 30. Despite available therapies to treat CPVT, which include beta-blockers and the sodium channel blocker flecainide, it is estimated that 30% to 40% of patients still experience significant cardiac events. Patients unresponsive to available therapies may be candidates for implantation of cardiac defibrillators, though their safety and effectiveness is considerably more limited in young patients. Due to the limitations of existing therapies, there remains a significant unmet medical need for patients with CPVT.

AT307 consists of an AAV9 vector that is designed to deliver a functional CASQ2 gene and to increase CASQ2 protein expression in targeted tissues. We are utilizing AAV9 because it is known to effectively penetrate heart tissue.

Preclinical data in murine models of the disease demonstrated an ability to prevent ventricular tachycardia through restoration of CASQ2 protein expression. Initial preclinical proof-of-concept studies were conducted using an AT307 prototype product candidate in a genetically engineered murine model of CASQ2-CPVT. This mouse manifests stress-induced arrhythmias upon epinephrine administration, as well as cellular and molecular manifestations of the disease. In this model, a single administration of the AT307 prototype resulted in a significant improvement in CASQ2 protein expression to a level approaching that of normal animals. We believe AT307 has the potential to provide long-term clinical benefit to CASQ2-CPVT patients through persistent expression of the protein following a single administration, resulting in a significant reduction in life-threatening arrhythmic events and other disease symptoms.

Although we believe our product candidates have the potential to provide long-term improvement in patient symptoms with a single administration, we will need to complete additional preclinical studies and clinical trials to determine the safety and efficacy of our product candidates. The results of these future studies and trials may be different than the results of our earlier studies and trials. We have not received regulatory approval for any of our product candidates, and in order to obtain regulatory approval and commercialize our product candidates, the U.S. Food and Drug Administration or foreign regulatory agencies will need to determine that our product candidates are safe and effective. To date, no gene therapy products have been approved in the United States and two have been approved in Europe.

We believe that our proprietary manufacturing capabilities provide a core strategic advantage. We lease a manufacturing facility in South San Francisco that has been used for commercial manufacture of biologic drug products in the past, and have improved the facility to support our desired research, process development and manufacturing capabilities in accordance with current Good Manufacturing Practices, or cGMP, requirements. We plan to initiate cGMP manufacturing of our products in our facility in the second half of 2016. We have made and will continue to make significant investments to further optimize our manufacturing capabilities to cost-effectively produce high-quality AAV vectors at both clinical and commercial scale. We believe that our manufacturing processes, methods, expertise and facilities will give us a comprehensive manufacturing platform for production of our AAV product candidates at commercial scale.

We have a focused, passionate team with collective expertise in gene therapy, rare disease drug development and commercialization, and biologics manufacturing. Matthew Patterson, our President, Chief Executive Officer and Co-Founder, is a biotechnology leader with over 20 years of experience at Genzyme Corporation, BioMarin Pharmaceutical, Amicus Therapeutics and our company. We are backed by a group of leading life science institutional investors, including 5AM Ventures, Cormorant Asset Management LLC, Cowen Private Investments, Deerfield Management Company, Foresite Capital, OrbiMed, RA Capital Management, Redmile Group, Rock Springs Capital Management LP, Sofinnova Ventures, Venrock and Versant Ventures.

Our Strategy

Our strategy is to leverage the expertise of our team and the transformative potential of gene therapy technology to develop treatments that improve outcomes for patients with serious, life-threatening rare diseases. Key elements of our strategy are:

- ***Constantly focus on serving patients.*** We take pride in our efforts to harness the transformative potential of gene therapy to improve the lives of patients suffering from devastating rare diseases. We intend to continue to engage with patient advocacy groups to better understand the burden of disease and align our efforts with the needs of patients and caregivers.

- ***Advance our four lead product candidates through clinical development.*** We expect to submit INDs or CTAs for our product candidates as follows: AT982 for the treatment of Pompe disease in the third quarter of 2016, AT342 for the treatment of Crigler-Najjar in the fourth quarter of 2016, AT132 for the treatment of XLMTM in the first quarter of 2017 and AT307 for the treatment of CASQ2-CPVT in 2017.

- ***Continue to expand our pipeline with additional gene therapy product candidates targeting serious, life-threatening rare diseases.*** We intend to continue leveraging our expertise and focused selection criteria to expand our pipeline of product candidates. Our relationships with leading academic institutions and other rare disease companies are an important component of our strategy for sourcing additional product candidates.

- ***Continue to build our proprietary manufacturing capabilities and invest in a state-of-the-art cGMP facility.*** We believe the quality, reliability and scalability of our gene therapy manufacturing approach will be a core competitive advantage crucial to our long-term success. We intend to be capable of internal cGMP manufacturing in the second half of 2016.

Our Strengths

We believe our leadership position is based on our following strengths:

- ***Rare disease expertise.*** Led by a management team with over 100 years of combined experience in rare diseases, we are building a fully integrated and industry-leading biotechnology company. Leveraging recent developments in gene therapy, we aim to provide durable and meaningful treatment options to patients suffering from rare monogenic diseases.

- ***Highly focused selection criteria for development programs.*** We employ a disciplined approach to select and expand our pipeline of product candidates. We believe the application of our selection criteria enables the efficient, cost-effective and successful development of our product candidates.

- ***Promising product candidate pipeline.*** On the basis of rigorous preclinical investigation, we are preparing to advance our four lead product candidates into the clinic: AT132 for the treatment of XLMTM, AT342 for the treatment of Crigler-Najjar, AT982 for the treatment of Pompe disease and AT307 for the treatment of CASQ2-CPVT.

- ***Proprietary know-how and capabilities.*** Our proprietary manufacturing capabilities provide a major core strategic advantage, including better control over the cost and timelines of developing our product candidates, superior protection of novel inventions and intellectual property, and expanded possibilities for new programs and partnerships.

- ***Broad network.*** We believe our strong relationships with key opinion leaders and patient advocacy groups will support our product development efforts and our potential for future commercial success. Leveraging our collaborations with these parties allows us to better understand the diseases we target and optimize our research, clinical development and commercial plans.

Risks Related to Our Business

Our business is subject to numerous risks and uncertainties, including those highlighted in the section entitled “Risk Factors” immediately following this prospectus summary. These risks include, but are not limited to, the following:

- we have a limited operating history and are very early in our development efforts, all of our product candidates are still in preclinical development and we may be unable to advance our product candidates to clinical development, obtain regulatory approval and ultimately commercialize our product candidates;

- we have not tested any of our product candidates in clinical trials, and success in early preclinical studies or clinical trials may not be indicative of results obtained in later preclinical studies and clinical trials;

- if we do not achieve our projected development goals in the time frames we announce and expect, the commercialization of our products may be delayed and, as a result, our stock price may decline;

- our product candidates are based on a novel AAV gene therapy technology with which there is little clinical experience, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval;

- ethical and legal concerns about gene therapy and genetic testing may result in additional regulations or restrictions on the development and commercialization of our product candidates;

- even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate and the approval may be for a more narrow indication than we seek;

- delays in establishing that our manufacturing process and facility comply with cGMPs or disruptions in our manufacturing process may delay or disrupt our development and commercialization efforts;

- we may not be successful in our efforts to build a pipeline of additional product candidates;

- 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;

- there have been several adverse side effects identified in clinical trials for other gene therapy product candidates in the past, and our product candidates, which are based on gene therapy technology, may cause undesirable and unforeseen side effects or be perceived by the public as unsafe;

- we have a history of operating losses, and we may not achieve or sustain profitability; and

- all of our current product candidates are licensed from or based upon licenses from third parties, and if any of these license or sublicense agreements are terminated or interpreted to narrow our rights, our ability to advance our current product candidates or develop new product candidates based on these technologies will be materially adversely affected.

Corporate Information

We were incorporated in Delaware in November 2012. Our principal executive offices are located at 600 California Street, 17th Floor, San Francisco, California 94108, and our telephone number is (415) 818-1001. Our website address is www.audentestx.com. The information contained on, or that can be accessed through, our website is not a part of this prospectus. Investors should not rely on any such information in deciding whether to purchase our common stock.

Unless the context indicates otherwise, as used in this prospectus, the terms "company," "Audentes," "Audentes Therapeutics," "Registrant," "we," "us" and "our" refer to Audentes Therapeutics, Inc., a Delaware corporation, and its subsidiaries taken as a whole, unless otherwise noted.

We have registered the trademarks "Audentes," "Audentes Therapeutics" and "Courageous Patients. Bold Effort." in the European Union and we have trademark applications for each of these trademarks pending with the U.S. Patent and Trademark Office. The Audentes logo and all product names are our common law trademarks. All other service marks, trademarks and tradenames appearing in this prospectus are the property of

their respective owners. Solely for convenience, the trademarks and tradenames referred to in this prospectus appear without the ® and ™ symbols, but those references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights, or the right of the applicable licensor to these trademarks and tradenames.

Implications of Being an Emerging Growth Company

As a company with less than $1.0 billion in revenue during our most recently completed fiscal year, we qualify as an "emerging growth company" as defined in Section 2(a) of the Securities Act of 1933, or the Securities Act, as modified by the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. As an emerging growth company, we may take advantage of certain exemptions from various public company reporting requirements, including:

- reduced disclosure of financial information in this prospectus, including two years of audited financial information and two years of selected financial information;
- an exemption from compliance with the auditor attestation requirement on the effectiveness of our internal control over financial reporting;
- an exemption from compliance with any requirement that the Public Company Accounting Oversight Board may adopt regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure about our executive compensation arrangements; and
- exemptions from the requirements to obtain a non-binding advisory vote on executive compensation or a stockholder approval of any golden parachute arrangements.

We may take advantage of these exemptions for up to five years or such earlier time that we are no longer an emerging growth company. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock. We would cease to be an emerging growth company upon the earliest to occur of: the last day of the fiscal year in which we have more than $1.0 billion in annual revenue; the date we qualify as a "large accelerated filer," with at least $700 million of equity securities held by non-affiliates; the issuance, in any three-year period, by us of more than $1.0 billion in non-convertible debt securities; and the last day of the fiscal year ending after the fifth anniversary of this offering.

The JOBS Act also permits us, as an emerging growth company, to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies and thereby allows us to delay the adoption of those standards until those standards would apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

THE OFFERING

Common stock to be offered	5,000,000 shares
Common stock to be outstanding immediately following this offering	21,020,378 shares
Option to purchase additional shares	We have granted to the underwriters the option, exercisable for 30 days from the date of this prospectus, to purchase up to 750,000 additional shares of our common stock.
Potential insider participation	Our existing institutional investors associated with our board have indicated an interest in purchasing shares of common stock in this offering with an aggregate value of approximately $30.0 million at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, fewer or no shares in this offering to any of these parties, or any of these parties may determine to purchase more, fewer or no shares in this offering. The underwriters will receive the same underwriting discount on any shares purchased by these entities as they will on any other shares sold to the public in this offering.
Use of proceeds	We estimate that the net proceeds from the sale of our common stock sold in this offering will be approximately $66.1 million, assuming an initial public offering price of $15.00 per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses. We intend to use the net proceeds from this offering to advance preclinical and clinical development of AT132 for the treatment of XLMTM, AT342 for the treatment of Crigler-Najjar, AT982 for the treatment of Pompe disease and AT307 for the treatment of CASQ2-CPVT; to operate and expand our internal manufacturing facility; and for working capital and other general corporate purposes. See the section entitled “Use of Proceeds.”
Risk factors	You should read the section entitled “Risk Factors” and other information included in this prospectus for a discussion of factors you should consider carefully before deciding to invest in shares of our common stock.
Proposed NASDAQ Global Market symbol	“BOLD”

The number of shares of our common stock to be outstanding following this offering is based on (i) 2,200,077 shares of our common stock outstanding as of March 31, 2016 and (ii) the automatic conversion of shares of our convertible preferred stock outstanding as of March 31, 2016 into 13,820,301 shares of our common stock effective immediately prior to the completion of this offering.

The number of shares of our common stock to be outstanding after this offering excludes:

- 2,279,086 shares of our common stock issuable upon the exercise of outstanding options as of March 31, 2016, with a weighted-average exercise price of approximately $3.99 per share;
- 82,739 shares of our common stock issuable upon the exercise of outstanding options granted after March 31, 2016, with an exercise price of $7.54 per share;
- 101,127 shares of our common stock issuable upon the exercise of options that we expect to grant on the date of this prospectus, with an exercise price equal to the initial public offering price of our common stock; and
- 2,208,646 shares of common stock reserved for future issuance under our stock-based compensation plans as of March 31, 2016, consisting of (i) 599,773 shares of common stock reserved for future issuance under our 2012 Equity Incentive Plan as of March 31, 2016 (consisting of 682,512 shares reserved as of March 31, 2016, reduced by 82,739 shares underlying stock options granted after March 31, 2016), (ii) 1,398,873 shares of common stock reserved for future issuance under our 2016 Equity Incentive Plan (consisting of 1,500,000 shares, reduced by 101,127 shares underlying stock options that we expect to grant on the date of this prospectus), which will become effective on the date immediately prior to the date of this prospectus and (iii) 210,000 shares of common stock reserved for future issuance under our 2016 Employee Stock Purchase Plan, which will become effective on the date of this prospectus. Upon completion of this offering, any remaining shares available for issuance under our 2012 Equity Incentive Plan will be added to the shares reserved for future issuance under our 2016 Equity Incentive Plan and we will cease granting awards under our 2012 Equity Incentive Plan. Our 2016 Equity Incentive Plan and 2016 Employee Stock Purchase Plan will also provide for automatic annual increases in the number of shares reserved under the plans each year, as more fully described in the section entitled "Executive Compensation—Employee Benefit and Stock Compensation Plans."

Unless otherwise noted, the information in this prospectus reflects and assumes the following:

- the automatic conversion of all outstanding shares of our convertible preferred stock into an aggregate of 13,820,301 shares of our common stock effective immediately prior to the completion of this offering;
- a 2.22977-to-1 reverse stock split, which became effective on July 7, 2016;
- the filing and effectiveness of our restated certificate of incorporation in Delaware and the adoption of our restated bylaws, both of which will occur immediately prior to the completion of this offering;
- no exercise of outstanding options; and
- no exercise of the underwriters' option to purchase additional shares.

SUMMARY CONSOLIDATED FINANCIAL DATA

The following tables summarize our consolidated statements of operations and consolidated balance sheet data. We derived our summary statements of operations data for the years ended December 31, 2014 and 2015 from our audited consolidated financial statements included elsewhere in this prospectus. We derived our summary consolidated statements of operations data for the three months ended March 31, 2015 and 2016 and our summary consolidated balance sheet data as of March 31, 2016 from our unaudited interim condensed consolidated financial statements included elsewhere in this prospectus. Our unaudited interim condensed consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles on the same basis as our audited annual consolidated financial statements and, in the opinion of management, reflect all adjustments, consisting only of normal, recurring adjustments, necessary for the fair presentation of those unaudited interim condensed consolidated financial statements. Our historical results are not necessarily indicative of the results that may be expected in any future period, and the results for the three months ended March 31, 2016 are not necessarily indicative of operating results to be expected for the full year ending December 31, 2016 or any other period.

The summary consolidated financial data below should be read in conjunction with the sections entitled, "Selected Consolidated Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements and related notes included elsewhere in this prospectus.

	Year Ended December 31,		Three Months Ended March 31,	
	2014	2015	2015	2016
	(in thousands, except share and per share amounts)		(unaudited)	
Consolidated Statements of Operations Data:				
Operating expenses:				
Research and development	$ 9,280	$ 20,235	$ 3,080	$ 7,906
General and administrative	1,670	6,491	1,083	2,632
Total operating expenses	10,950	26,726	4,163	10,538
Loss from operations	(10,950)	(26,726)	(4,163)	(10,538)
Interest income	6	245	61	97
Other income (expense), net	125	23	47	(23)
Net loss	$ (10,819)	$ (26,458)	$ (4,055)	$ (10,464)
Net loss per share, basic and diluted[(1)]	$ (21.56)	$ (23.03)	$ (6.63)	$ (4.85)
Shares used in computing net loss per share, basic and diluted[(1)]	501,707	1,148,827	612,039	2,159,081
Pro forma net loss per share, basic and diluted (unaudited)[(1)]		$ (2.28)		$ (0.65)
Shares used in computing pro forma net loss per share, basic and diluted (unaudited)[(1)]		11,621,249		15,979,382

(1) See Notes 2 and 13 to our audited consolidated financial statements and Note 8 to our unaudited interim condensed consolidated financial statements included elsewhere in this prospectus for an explanation of the calculations of our basic and diluted net loss per share, basic and diluted pro forma net loss per share and the shares used in computing basic and diluted net loss per share and basic and diluted pro forma net loss per share.

	March 31, 2016		
	Actual	Pro Forma[1]	Pro Forma As Adjusted[2]
		(in thousands) (unaudited)	
Consolidated Balance Sheet Data:			
Cash, cash equivalents and investments	$ 80,272	$ 80,272	$146,322
Working capital	76,150	76,150	142,200
Total assets	110,555	110,555	176,605
Convertible preferred stock	135,750	—	—
Accumulated deficit	(51,207)	(51,207)	(51,207)
Total stockholders' equity	91,642	91,642	157,692

(1) The pro forma consolidated balance sheet data as of March 31, 2016 reflects the automatic conversion of shares of our convertible preferred stock outstanding as of March 31, 2016 into 13,820,301 shares of our common stock effective immediately prior to the completion of this offering.

(2) The pro forma as adjusted consolidated balance sheet data reflects (i) the pro forma adjustment set forth above and (ii) the sale and issuance of 5,000,000 shares of our common stock in this offering, at an assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus after deducting the estimated underwriting discounts and commissions and estimated offering expenses. Each $1.00 increase (decrease) in the assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, would increase (decrease) each of cash, cash equivalents and investments, working capital, total assets and total stockholders' equity by $4.7 million, assuming that the number of shares offered, as set forth on the cover page of this prospectus, remains the same, and after deducting the estimated underwriting discounts and commissions. Similarly, each increase (decrease) of one million shares in the number of shares offered by us would increase (decrease) each of cash, cash equivalents and investments, working capital, total assets and total stockholders' equity by approximately $14.0 million, assuming the assumed initial public offering price remains the same and after deducting the estimated underwriting discounts and commissions.

RISK FACTORS

Investing in our common stock involves a high degree of risk. You should consider carefully the risks and uncertainties described below, together with all of the other information in this prospectus, including the consolidated financial statements, the notes thereto and the section entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations" included elsewhere in this prospectus before deciding whether to invest in shares of our common stock. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties that we are unaware of or that we deem immaterial may also become important factors that adversely affect our business. If any of the following risks actually occur, our business, financial condition, results of operations and future prospects could be materially and adversely affected. In that event, the market price of our stock could decline, and you could lose part or all of your investment.

Risks Related to Product Development and Regulatory Approval

We are very early in our development efforts. All of our product candidates are still in preclinical development. If we are unable to advance our product candidates to clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.

We are very early in our development efforts and all of our lead product candidates are still in preclinical development. We, or our collaborators, have only recently completed initial preclinical studies for our AT132, AT342, AT982 and AT307 programs. We have invested substantially all of our efforts and financial resources in the identification and preclinical development of our current product candidates, AT132 for X-Linked Myotubular Myopathy, or XLMTM, AT342 for the treatment of Crigler-Najjar Syndrome, or Crigler-Najjar, AT982 for the treatment of Pompe disease and AT307 for the treatment of the CASQ2 subtype of Catecholaminergic Polymorphic Ventricular Tachycardia, or CASQ2-CPVT. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of our product candidates, which may never occur. We currently generate no revenue from sales of any product and we may never be able to develop or commercialize a marketable product.

Each of our programs and product candidates will require additional preclinical and clinical development, regulatory approval in multiple jurisdictions, obtaining manufacturing supply, capacity and expertise, building of a commercial organization, substantial investment and significant marketing efforts before we generate any revenue from product sales. Our product candidates must be authorized for marketing by the U.S. Food and Drug Administration, or the FDA, or certain other foreign regulatory agencies, such as the European Medicines Agency, or EMA, before we may commercialize our product candidates.

The success of our product candidates will depend on several factors, including the following:

- successful completion of preclinical studies and successful enrollment and completion of clinical trials, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable, under the FDA's current Good Clinical Practices, or cGCPs, and current Good Laboratory Practices, or cGLPs;

- effective Investigational New Drug applications, or INDs, or Clinical Trial Authorisations, or CTAs, that allow commencement of our planned clinical trials or future clinical trials for our product candidates;

- positive results from our future clinical programs that support a finding of safety and effectiveness and an acceptable risk-benefit profile of our product candidates in the intended populations;

- receipt of regulatory approvals from applicable regulatory authorities;
- establishment of arrangements with third-party manufacturers for clinical supply and, where applicable, commercial manufacturing capabilities;
- successful development of our internal manufacturing processes or transfer to larger-scale facilities operated by either a contract manufacturing organization, or CMO, or by us;
- establishment and maintenance of patent and trade secret protection or regulatory exclusivity for our product candidates;
- commercial launch of our product candidates, if and when approved, whether alone or in collaboration with others;
- acceptance of our product candidates, if and when approved, by patients, the medical community and third-party payors;
- effective competition with other therapies;
- establishment and maintenance of healthcare coverage and adequate reimbursement;
- enforcement and defense of intellectual property rights and claims; and
- maintenance of a continued acceptable safety profile of our product candidates following approval.

If we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

We have not tested any of our product candidates in clinical trials. Success in early preclinical studies or clinical trials may not be indicative of results obtained in later preclinical studies and clinical trials.

Though viral vectors similar to ours have been evaluated by others in clinical trials, our product candidates have never been evaluated in human clinical trials, and we may experience unexpected or adverse results in the future. We will be required to demonstrate through adequate and well-controlled clinical trials that our product candidates are safe and effective, with a favorable benefit-risk profile, for use in their target indications before we can seek regulatory approvals for their commercial sale. Earlier gene therapy clinical trials, which we believe serve as proof-of-concept for our product candidates, utilized adeno-associated viral vectors, or AAV vectors, similar to ours. For example, in October 2015, a gene therapy company publicly reported positive top-line results from a Phase 3 trial of a product candidate intended to treat rare genetic blinding conditions. However, this study or others like it should not be relied upon as evidence that our planned clinical trials will succeed. Trial designs and results from previous trials are not necessarily predictive of our future clinical trial designs or results, and initial positive results we may observe may not be confirmed upon full analysis of the complete trial data. In addition, the positive results we have observed for our product candidates in preclinical animal models may not be predictive of our future clinical trials in humans. Our product candidates may also fail to show the desired safety and efficacy in later stages of clinical development even if they successfully advance through initial clinical trials.

Many companies in the biotechnology industry have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development and there is a high failure rate for product candidates proceeding through clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. For example, we may want to use the RECENSUS retrospective medical chart review as a historical control for our planned Phase 1/2 ASPIRO

trial of AT132. However, because the patient population, supportive care used or other factors may be different than those used in the ASPIRO trial, we may be unable to use the RECENSUS study to demonstrate statistical significance of results in our planned ASPIRO trial, which may delay the development of AT132. Even if we demonstrate statistical significance, regulatory agencies may not accept the use of the historical control. Regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development. We cannot be certain that we will not face similar setbacks.

If we do not achieve our projected development goals in the time frames we announce and expect, the commercialization of our products may be delayed and, as a result, our stock price may decline.

From time to time, we estimate the timing of the accomplishment of various scientific, clinical, regulatory, manufacturing and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of preclinical studies and clinical trials and the submission of regulatory filings. From time to time, we may publicly announce the expected timing of some of these milestones. For example, throughout this prospectus, we state that we plan to submit INDs or CTAs for AT982 in the third quarter of 2016, for AT342 in the fourth quarter of 2016, for AT132 in the first quarter of 2017 and for AT307 in 2017, and expect to have preliminary data from our AT982, AT342 and AT132 programs in the second half of 2017. We also state that we intend to be capable of internal Current Good Manufacturing Practices, or cGMP, manufacturing in the second half of 2016 and capable of providing cGMP supply at scale suitable for commercial production by 2018. All of these milestones are, and will be, based on a variety of assumptions. The actual timing of these milestones can vary significantly compared to our estimates, in some cases for reasons beyond our control. We may experience numerous unforeseen events during, or as a result of, any future clinical trials that we conduct that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

- the FDA and other governmental regulators, Institutional Review Boards, or IRBs, or ethics committees may not authorize or may delay authorizing us or our investigators to commence a clinical trial or conduct a clinical trial at all or at a prospective trial site, such as by requiring us to conduct additional preclinical studies and to submit additional data or imposing other requirements before permitting us to initiate a clinical trial;

- we may experience delays in reaching, or fail to reach, agreement on acceptable terms with prospective trial sites and prospective contract research organizations, or CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;

- clinical trials of our product candidates may produce negative or inconclusive results and we may decide, or regulators may require us, to conduct preclinical studies in addition to those we currently have planned or additional clinical trials or we may decide to abandon drug development programs;

- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials or fail to return for post-treatment follow-up at a higher rate than we anticipate;

- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all, or may deviate from the clinical trial protocol or drop out of the trial, which may require that we add new clinical trial sites or investigators;

- we may elect to, or regulators, IRBs or ethics committees may require that we or our investigators, suspend or terminate clinical trials for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to health risks;

- the cost of planned clinical trials of our product candidates may be greater than we anticipate;

- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; and

- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or IRBs or ethics committees to suspend or terminate the trials, or reports may arise from preclinical or clinical testing of other gene therapies that raise safety or efficacy concerns about our product candidates.

For instance, safety signals have been observed at the highest dose in mouse disease model studies of AT132 and AT982 that we conducted, and in a non-cGLP study of AT982 that was conducted by other researchers in an un-validated rat model of the disease. To appropriately understand these findings, and after discussion with regulatory authorities in the United States and Europe, we have implemented a robust plan to gather additional information and plan to engage in further discussions with regulatory authorities prior to submitting INDs and CTAs to start clinical trials. In both programs we have completed initial large animal studies in which similar safety signals were not observed. We continue to conduct preclinical studies across our portfolio of product candidates in order to enable IND and CTA submissions. If we observe additional unexpected safety signals in these studies or are unable to explain to the regulatory authorities' satisfaction the safety signals we have observed to date, we may decide or be required to delay or halt initial or further clinical development of these product candidates.

In addition, for our first in human trial of AT132, the FDA as part of their initial feedback to us has suggested that we first study the product candidate in adults. The agency has provided us with an opportunity to justify our position that we do not need to first dose adults. Similarly, the Medicines Healthcare products Regulatory Agency has, in its initial feedback to us, suggested we first study AT982 in adults. These issues, or others, could delay our clinical development program. If we do not meet these milestones as publicly announced, the commercialization of our product candidates may be delayed and, as a result, our stock price may decline.

Our product candidates are based on a novel AAV gene therapy technology with which there is little clinical experience, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. Currently, no gene therapy products have been approved in the United States and only two gene therapy products have been approved in Europe.

Our product candidates are based on gene therapy technology and our future success depends on the successful development of this novel therapeutic approach. We cannot assure you that any development problems we or other gene therapy companies experience in the future related to gene therapy technology will not cause significant delays or unanticipated costs in the development of our product candidates, or that such development problems can be solved. In addition, the clinical study requirements of the FDA, 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 extensively studied product candidates. Further, as we are developing novel treatments for diseases in which there is little clinical experience with new endpoints and methodologies, there is heightened risk that the FDA, EMA or comparable foreign regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. To date, no gene therapy product has been approved in the United States and only two gene therapy products have been approved in Europe, which makes it 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 European Union, or EU, or other jurisdictions. Further, approvals by EMA and the European Commission may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene therapy products have evolved and may continue to change in the future. For example, the FDA established the Office of Cellular, Tissue and Gene Therapies within its Center

for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. These and other regulatory review agencies, committees and advisory groups and the requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval limitations or restrictions.

The FDA, the National Institutes of Health, or NIH, the EMA and other regulatory agencies have demonstrated caution in their regulation of gene therapy treatments, and ethical and legal concerns about gene therapy and genetic testing may result in additional regulations or restrictions on the development and commercialization of our product candidates, which may be difficult to predict.

The FDA, NIH, other regulatory agencies at both the federal and state level in the United States, U.S. congressional committees, and the EMA and other foreign governments, have expressed interest in further regulating the biotechnology industry, including gene therapy and genetic testing. For example, the EMA advocates a risk-based approach to the development of a gene therapy product. Any such further regulation may delay or prevent commercialization of some or all of our product candidates. For example, in 1999, a patient died during a gene therapy clinical trial that utilized an adenovirus vector and it was later discovered that adenoviruses could generate an extreme immune system reaction that can be life-threatening. In January 2000, the FDA halted that trial and began investigating 69 other gene therapy trials underway in the United States, 13 of which required remedial action. In 2003, the FDA suspended 27 additional gene therapy trials involving several hundred patients after learning that some patients treated in a clinical trial in France had subsequently developed leukemia.

Regulatory requirements in the United States and abroad governing gene therapy products have changed frequently and may continue to change in the future. Prior to submitting an IND, our planned clinical trials will be subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or RAC. Following an initial review, RAC members make a recommendation as to whether the protocol raises important scientific, safety, medical, ethical or social issues that warrant in-depth discussion at the RAC's quarterly meetings. Even though the FDA decides whether individual gene therapy protocols may proceed under an IND, the RAC's recommendations are shared with the FDA and the RAC public review process, if undertaken, can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and has not objected to its initiation or has notified the sponsor that the study may begin. Conversely, the FDA can put an IND on a clinical hold even if the RAC has provided a favorable review or has recommended against an in-depth, public review. Moreover, under guidelines published by the NIH, patient enrollment in our planned clinical trials cannot begin until the investigator for such clinical trial has received a letter from the Office of Biotechnology Activities indicating that the RAC review process has been completed; and Institutional Biosafety Committee, or IBC, approval as well as all other applicable regulatory authorizations have been obtained. In addition to the government regulators, the IBC and IRB of each institution at which we conduct our planned clinical trials, would need to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of any of our product candidates. Similarly, the EMA governs the development of gene therapies in the European Union and may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies or trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development

of such product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

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

Prior to commercialization, our product candidates must be approved by the FDA pursuant to a BLA in the United States and by the EMA and similar regulatory authorities outside the United States. The process of obtaining marketing approvals, both in the United States and abroad, is expensive and takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction. We have no experience in submitting and supporting the applications necessary to gain marketing approvals. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate.

Approval of our product candidates may be delayed or refused for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that our product candidates are safe and effective for any of their proposed indications;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that our product candidates' clinical and other benefits outweigh their safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical programs or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of a BLA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;
- the facilities of the third-party manufacturers with which we contract may not be adequate to support approval of our product candidates; and

- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials and the review process.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or Risk Evaluation and Mitigation Strategies, or REMS. These regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and materially and adversely affect our business, financial condition, results of operations and prospects.

Further, the regulatory authorities may require concurrent approval or the CE mark, indicating conformity with applicability with European Community directives, of a companion diagnostic device. For the product candidates we currently are 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, or CLIA, are sufficient to diagnose patients and will be permitted by the FDA. For future product candidates, however, it may be necessary to use FDA-cleared or FDA-approved diagnostic tests to diagnose patients or to assure the safe and effective use of product candidates in trial subjects. The FDA refers to such tests as *in vitro* companion diagnostic devices. In August 2014, the FDA issued 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 an *in vitro* diagnostic device is essential to the safe and effective use of a therapeutic product, the FDA generally will require approval or clearance of the diagnostic device at the same time that the FDA approves the therapeutic product. At this point, it is unclear how the FDA will apply this policy to our current or future gene therapy product candidates. Should the FDA deem genetic tests used for diagnosing 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 of a BLA for our product candidates. In the EU, the European Commission has proposed substantial revisions to the current regulations governing *in vitro* diagnostic medical devices. If adopted in their current form, these revisions may impose additional obligations on us that may impact the development and authorization of our product candidates in the EU.

We may never obtain FDA approval for any of our product candidates in the United States, and even if we do, we may never obtain approval for or commercialize any of our product candidates in any other jurisdiction, which would limit our ability to realize their full market potential.

In order to eventually market any of our product candidates in any particular foreign jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a jurisdiction-by-jurisdiction basis regarding safety and efficacy. Approval by the FDA in the United States, if obtained, does not ensure approval by regulatory authorities in other countries or jurisdictions. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional preclinical studies or clinical trials which could be costly and time-consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. The foreign regulatory

approval process involves all of the risks associated with FDA approval. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of our products will be unrealized.

Delays in establishing that our manufacturing process and facility comply with cGMPs or disruptions in our manufacturing process may delay or disrupt our development and commercialization efforts. To date, no gene therapy product has received approval from the FDA so the requirements for the manufacture of a gene therapy product are uncertain.

We have established relationships with research facilities, CMOs and our collaborators to manufacture and supply our product candidates for preclinical and clinical studies. We plan to manufacture AT132 in our own facilities, and anticipate that AT342 will be initially manufactured by a CMO. AT982 is currently manufactured by the University of Florida in a facility that we believe complies with cGMPs. We are currently investing in a state-of-the-art cGMP facility to develop and implement novel in-house production technologies. As we establish and scale our internal manufacturing capabilities, we plan to transition all process development and manufacturing activities to our own facilities. Before we can begin to manufacture our product candidates in our own facility for our planned clinical trials or for commercial production, we must demonstrate to the FDA that our planned chemistry, manufacturing and controls for our gene therapy product candidates meet applicable requirements. A manufacturing authorization must be obtained from the appropriate European Union regulatory authorities. Because no gene therapy product has yet been approved in the United States, there is no manufacturing facility that has demonstrated the ability to comply with FDA requirements, and, therefore, the timeframe for demonstrating compliance to the FDA's satisfaction is uncertain.

We expect that development of our own manufacturing facility would provide us with enhanced control of material supply for both clinical trials and the commercial market, enable the more rapid implementation of process changes and allow for better long-term margins. However, we have no experience as a company in developing a manufacturing facility and may never be successful in developing our own manufacturing facility or capability. Additionally, given that cGMP gene therapy manufacturing is a nascent industry, there are only a small number of CMOs with the experience necessary to manufacture our product candidates and we may have difficulty finding or maintaining relationships with such CMOs or hiring experts for internal manufacturing and, accordingly, our production capacity may be limited. Even if we are successful, our manufacturing capabilities could be affected by cost-overruns, unexpected delays, equipment failures, lack of capacity, labor shortages, natural disasters, power failures and numerous other factors that could prevent us from realizing the intended benefits of our manufacturing strategy and have a material adverse effect on our business.

In addition, we must pass a pre-approval inspection of our manufacturing facility by the FDA before any of our product candidates can obtain marketing approval. To date, neither we nor our contract manufacturers has manufactured or attempted to manufacture batches of our products that comply with cGMPs. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMPs, 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 cGMPs, 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. If we or our CMOs are unable to reliably produce products to specifications acceptable to the FDA or other regulatory authorities, we may not obtain or maintain the approvals we need to commercialize such products. Even if we obtain regulatory approval for any of our product candidates, there is no assurance that either we or our CMOs will be able to manufacture the approved product to specifications acceptable to the FDA or other regulatory authorities, to produce it in sufficient quantities to meet the requirements for the potential launch of the product or to meet potential future demand. Any of these challenges could delay initiation of, or completion of, clinical trials, require bridging clinical trials or the repetition of one or

more clinical trials, increase clinical trial costs, delay approval of our product candidate, impair commercialization efforts, increase our cost of goods and have an adverse effect on our business, financial condition, results of operations and growth prospects.

We may not be successful in our efforts to build a pipeline of additional product candidates.

Our business model is centered on applying our expertise in rare diseases by establishing focused selection criteria to develop and advance a broad portfolio of gene therapy product candidates through development into commercialization. We may not be able to continue to identify and develop new product candidates in addition to the pipeline of product candidates that our research and development efforts to date have resulted in. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development. For example, they may be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be drugs that will receive marketing approval and achieve market acceptance. If we do not successfully develop and commercialize product candidates based upon our approach, we will not be able to obtain product revenue in future periods, which likely would result in significant harm to our financial position and adversely affect our stock price.

Our product candidates based on gene therapy technology may cause undesirable and unforeseen side effects or be perceived by the public as unsafe, which could delay or prevent their advancement into clinical trials or regulatory approval, limit the commercial potential or result in significant negative consequences.

As discussed above, there have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia and death seen in other trials using other vectors. While new AAV vectors have been developed to reduce these side effects, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material.

Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration which, while not necessarily adverse to the patient's health, could substantially limit the effectiveness of the treatment. In previous clinical trials involving AAV vectors for gene therapy, some subjects experienced the development of a T-cell response, whereby after the vector is within the target cells, the cellular immune response system triggers the removal of transduced cells by activated T-cells. If our vectors demonstrate a similar effect we may decide or be required to halt or delay further clinical development of our product candidates.

In addition to side effects caused by the product candidate, the administration process or related procedures also can cause adverse side effects. For AT307, potential procedure-related events are similar to those associated with standard coronary diagnostic procedures, and may include vascular injury (for example, damage to the femoral, radial or brachial arteries at the site of vascular access, or damage to the coronary arteries) or myocardial injury. If any such adverse events occur, our clinical trials could be suspended or terminated. If we are unable to demonstrate that any adverse events were caused by the administration process or related procedures, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. Even if we are able to demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to not initiate, delay, suspend or terminate any future clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

Additionally, if any of our product candidates receives marketing approval, the FDA could require us to adopt a REMS to ensure that the benefits of the product outweigh its risks, which may include, among other things, a Medication Guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by our product candidate, several potentially significant negative consequences could result, including:

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

Any of these occurrences may harm our business, financial condition and prospects significantly.

The diseases we seek to treat have low prevalence and it may be difficult to identify patients with these diseases, which may lead to delays in enrollment for our trials or slower commercial revenue if approved.

Genetically defined diseases generally, and especially those for which our current product candidates are targeted, have low incidence and prevalence. For example, we estimate that the incidence of XLMTM is approximately one in 50,000 male births, that the incidence of Crigler-Najjar is approximately one in 1,000,000 births, that the incidence of Pompe disease is one in 40,000 people, and that there are approximately 6,000 people in North America, Europe and other addressable markets with CASQ2-CPVT. In addition, some of our potential patients may have neutralizing antibodies to AAV, which may affect the therapeutic efficacy of our product candidates. Moreover, following administration of any AAV vector, patients are likely to develop neutralizing antibodies specific to the vector administered. These could be significant obstacles to the timely recruitment and enrollment of a sufficient number of eligible patients into our trials. Patient enrollment may be affected by other factors including:

- the severity of the disease under investigation;
- design of the study protocol;
- the eligibility criteria for the study;
- the perceived risks, benefits and convenience of administration of the product candidate being studied;
- our efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians; and
- the proximity and availability of clinical trial sites to prospective patients.

Our inability to enroll a sufficient number of patients with these diseases for our planned clinical trials would result in significant delays and could require us to not initiate or abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our product

candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

Additionally, our projections of both the number of people who have XLMTM, Crigler-Najjar, Pompe disease and CASQ2-CPVT, as well as the people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. The total addressable market opportunity for our product candidates will ultimately depend upon, among other things, the final labeling for each of our product candidates, if our product candidates are approved for sale in our target indications, acceptance by the medical community and patient access, drug pricing and reimbursement. The number of patients globally may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business. Our products may potentially be dosed on a one-time basis, which means that patients who enroll in our clinical trials may not be eligible to receive our products on a commercial basis if they are approved, leading to lower revenue potential.

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

We plan to seek a Breakthrough Therapy Designation for our product candidates if the clinical data support such a designation for one or more product candidates. A breakthrough therapy is defined as a drug or biologic that is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug, or biologic in our case, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For product candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Biologics designated as breakthrough therapies by the FDA may also be eligible for accelerated approval.

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

A Fast Track Designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process, and does not increase the likelihood that our product candidates will receive marketing approval.

We do not currently have Fast Track Designation for any of our product candidates but intend to seek such designation for some or all of our product candidates. If a drug or biologic, in our case, is intended for the treatment of a serious or life-threatening condition and the biologic demonstrates the potential to address unmet medical needs for this condition, the biologic sponsor may apply for FDA Fast Track Designation. The FDA has broad discretion whether or not to grant this designation. Even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development program. Many biologics that have received Fast Track Designation have failed to obtain approval.

We may also seek accelerated approval for products that have obtained Fast Track Designation. Under the FDA's accelerated approval program, the FDA may approve a biologic for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. For biologics granted accelerated approval, post-marketing confirmatory trials are required to describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. These confirmatory trials must be completed with due diligence and, in some cases, the FDA may require that the trial be designed and/or initiated prior to approval. Moreover, the FDA may withdraw approval of any product candidate or indication approved under the accelerated approval pathway if, for example:

- the trial or trials required to verify the predicted clinical benefit of the product candidate fail to verify such benefit or do not demonstrate sufficient clinical benefit to justify the risks associated with the biologic;
- other evidence demonstrates that the product candidate is not shown to be safe or effective under the conditions of use;
- we fail to conduct any required post-approval trial of the product candidate with due diligence; or
- we disseminate false or misleading promotional materials relating to the product candidate.

We may be unable to maintain the benefits associated with Orphan Drug Designation, including the potential for market exclusivity, for AT132, AT342, AT982 and AT307, and may be unsuccessful in obtaining Orphan Drug Designation or transfer of designations obtained by others for our other current or future product candidates.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs, or biologics in our case, intended to treat relatively small patient populations as orphan drugs. Under the U.S. Orphan Drug Act, the FDA may designate a biologic as an orphan drug if it is intended to treat a rare disease or condition, which is defined as a patient population of fewer than 200,000 individuals in the United States. In the United States, Orphan Drug Designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax credits for qualified clinical research costs, and prescription drug user fee waivers. Similarly, in the European Union, the European Commission grants Orphan Drug Designation after receiving the opinion of the EMA's Committee for Orphan Medicinal Products on an Orphan Drug Designation application. In the European Union, Orphan Drug Designation is intended to promote the development of biologics that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the European Union and for which no satisfactory method of diagnosis, prevention or treatment has been authorized (or the product would be a significant benefit to those affected). In the European Union, Orphan Drug Designation entitles a party to financial incentives such as reduction of fees or fee waivers.

Generally, if a biologic with an Orphan Drug Designation subsequently receives the first marketing approval for the indication for which it has such designation, the biologic is entitled to a period of marketing exclusivity, which precludes EMA or the FDA from approving another marketing application for the same biologic and indication for that time period, except in limited circumstances. If our competitors are able to obtain orphan drug exclusivity prior to us for products that constitute the same active moiety and treat the same indications as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time. The applicable period is seven years in the United States and ten years in the European Union. The European Union exclusivity period can be reduced to six years if a drug no

longer meets the criteria for Orphan Drug Designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified.

As part of our business strategy, we have sought and received Orphan Drug Designation for AT132, AT342, AT982 and AT307 in the United States and Europe. However, Orphan Drug Designation does not guarantee future orphan drug marketing exclusivity.

Additionally, even though we have obtained an Orphan Drug Designation for AT132, AT342, AT982 and AT307, and even if we obtain orphan drug exclusivity for these product candidates and other product candidates, that exclusivity may not effectively protect AT132, AT342, AT982 and AT307 from competition because drugs with different active moieties can be approved for the same condition. Even after an orphan drug is approved, the FDA can also subsequently approve a later application for the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer in a substantial portion of the target populations, more effective or makes a major contribution to patient care. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Moreover, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to manufacture sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Orphan Drug Designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

We rely on third parties to conduct our preclinical studies, will rely on them to conduct clinical trials and rely on them to perform other tasks for us. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

Although we have recruited a team that has experience with clinical trials, as a company we have no experience in conducting clinical trials. Moreover, we do not have the ability to independently conduct preclinical studies and clinical trials, and we have relied upon, and plan to continue to rely upon medical institutions, clinical investigators, contract laboratories and other third parties, or our CROs, to conduct preclinical studies and future clinical trials for our product candidates. We expect to rely heavily on these parties for execution of preclinical and future clinical trials for our product candidates and control only certain aspects of their activities. Nevertheless, we will be responsible for ensuring that each of our preclinical and clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards and our reliance on CROs will not relieve us of our regulatory responsibilities. For any violations of laws and regulations during the conduct of our preclinical studies and clinical trials, we could be subject to warning letters or enforcement action that may include civil penalties up to and including criminal prosecution.

We and our CROs will be required to comply with regulations, including cGCPs for conducting, monitoring, recording and reporting the results of preclinical and clinical trials to ensure that the data and results are scientifically credible and accurate and that the trial patients are adequately informed of the potential risks of participating in clinical trials and their rights are protected. These regulations are enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities for any drugs in clinical development. The FDA enforces cGCP regulations through periodic inspections of clinical trial sponsors, principal investigators and trial sites. If we or our CROs fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, the FDA will determine that any of our future clinical trials will comply with cGCPs. In addition, our clinical trials must be conducted with product candidates produced in accordance with the requirements in cGMP regulations. Our failure or the failure of our

CROs to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process and could also subject us to enforcement action.

Although we intend to design our planned clinical trials for our product candidates, for the foreseeable future CROs will conduct all of our planned clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct future preclinical studies and clinical trials will also result in less day-to-day control over the management of data developed through preclinical studies and clinical trials than would be the case if we were relying entirely upon our own staff.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, any preclinical studies or clinical trials with which such CROs are associated with may be extended, delayed or terminated. In such cases, we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates in the subject indication could be harmed, our costs could increase and our ability to generate revenue could be delayed.

Any product candidate for which we obtain marketing approval will be subject to extensive post-marketing regulatory requirements and could be subject to post-marketing restrictions or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our product candidates, when and if any of them are approved.

Our product candidates and the activities associated with their development and potential commercialization, including their testing, manufacture, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMPs, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, including periodic inspections by the FDA and other regulatory authorities and requirements regarding the distribution of samples to physicians and recordkeeping.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of any approved product. The FDA closely regulates the post-approval marketing and promotion of drugs and biologics to ensure drugs and biologics are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding use of their products. If we promote our product candidates beyond their potentially approved indications, we may be subject to enforcement action for off-label promotion. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our product candidates, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such product candidates, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;

- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of any approved product from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of product candidates;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our product candidates;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Non-compliance with European requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with Europe's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

Our product candidates for which we intend to seek approval may face competition from biosimilars sooner than anticipated.

With the enactment of the Biologics Price Competition and Innovation Act of 2009, or BPCIA, an abbreviated pathway for the approval of biosimilar and interchangeable biological products was created. The abbreviated regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as interchangeable based on its similarity to an existing reference product. Under the BPCIA, an application for a biosimilar product cannot be approved by the FDA until 12 years after the original branded product is approved under a biologics license application, or BLA. On March 6, 2015, the FDA approved the first biosimilar product under the BPCIA. However, the law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have a material adverse effect on the future commercial prospects for our biological products.

We believe that if any of our product candidates are approved as a biological product under a BLA it should qualify for the 12-year period of exclusivity. However, there is a risk that the FDA will not consider any of our product candidates to be reference products for competing products, potentially creating the opportunity for biosimilar competition sooner than anticipated. Additionally, this period of regulatory exclusivity does not apply to companies pursuing regulatory approval via their own traditional BLA, rather than via the abbreviated pathway. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. Finally, there has been public discussion of potentially decreasing the period of exclusivity from the current 12 years. If such a change were to be enacted, our product candidates, if approved, could have a shorter period of exclusivity than anticipated.

Our strategy of obtaining rights to key technologies through in-licenses may not be successful.

We seek to expand our product candidate pipeline in part by in-licensing the rights to key technologies, including those related to gene delivery. The future growth of our business will depend in part on our ability to in-license or otherwise acquire the rights to additional product candidates or technologies. We cannot assure you that we will be able to in-license or acquire the rights to any product candidates or technologies from third parties on acceptable terms or at all.

The in-licensing and acquisition of these technologies is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire product candidates or technologies that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to license rights to us. Furthermore, we may be unable to identify suitable product candidates or technologies within our area of focus. If we are unable to successfully obtain rights to suitable product candidates or technologies, our business, financial condition and prospects could suffer.

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

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any products for which we obtain marketing approval.

For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the Affordable Care Act, or the ACA, was enacted to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. As implementation of the ACA is ongoing, the law appears likely to continue the downward pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs.

Moreover, the Drug Supply Chain Security Act imposes new obligations on manufacturers of pharmaceutical products related to product tracking and tracing. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We are not sure whether additional legislative changes will be enacted, or whether the current regulations, guidance or interpretations will be changed, or what the impact of such changes on our business, if any, may be.

Our operations and relationships with customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to penalties including criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with providers, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any product candidates for which we obtain marketing approval.

Restrictions under applicable U.S. federal and state healthcare laws and regulations may include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal healthcare programs such as Medicare and Medicaid;

- federal false claims laws, including the federal False Claims Act, imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

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

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health, or HITECH, Act and its implementing regulations, also imposes obligations, including mandatory contractual terms, on certain types of people and entities with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

- the federal Physician Payment Sunshine Act requires applicable manufacturers of covered 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 payments and other transfers of value to physicians and teaching hospitals, as well as certain ownership and investment interests held by physicians and their immediate family, which includes annual data collection and reporting obligations; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers.

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

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of product candidates from government-funded healthcare programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government-funded healthcare programs.

Risks Related to Manufacturing and Commercialization

Gene therapy products are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in our development or commercialization programs or otherwise harm our business.

The manufacturing processes used to produce our product candidates are complex, novel and have not been validated for commercial use. 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 candidates require processing steps that are more complex than those required for most small molecule drugs. Moreover, unlike small molecules, 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 is consistent from lot-to-lot or will perform in the intended manner. Accordingly, we employ multiple steps to control the manufacturing process to assure that the process works reproducibly and the 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 the FDA, the EMA or other applicable standards or specifications with consistent and acceptable production yields and costs.

In addition, the FDA, the EMA and other foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA or other foreign regulatory authorities may require that we not distribute a lot until the agency authorizes its release. 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. 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 scientific, quality-control and manufacturing personnel needed to operate our manufacturing processes, which could result in delays in production or difficulties in maintaining compliance with applicable regulatory requirements.

Any problems in our manufacturing process or facilities could result in delays in our planned clinical trials and increased costs, and could make us a less attractive collaborator for potential partners, including larger biotechnology companies and academic research institutions, which could limit our access to additional attractive development programs. It could also require us to find alternative manufacturing processes, which may be unavailable to us on attractive terms, or at all. Problems in our manufacturing process could restrict our ability to meet potential future market demand for our products.

We may rely on third-party manufacturers to produce some of our product candidates, but we have not entered into binding agreements with any such manufacturers to support commercialization. Additionally, these manufacturers do not have experience producing our product candidates at commercial levels and may not achieve the necessary regulatory approvals or produce our product candidates at the quality, quantities, locations and timing needed to support commercialization.

We have not yet secured manufacturing capabilities for commercial quantities of our product candidates. Although we intend to rely on third-party manufacturers for commercialization of certain of our product candidates if regulatory approval is achieved, we have only entered into agreements with such

manufacturers to support our clinical studies. We may be unable to negotiate binding agreements with the manufacturers to support our potential commercialization activities at commercially reasonable terms.

Manufacturers may not have the experience or ability to produce our product candidates at commercial levels. We may run into technical or scientific issues related to manufacturing or development that we may be unable to resolve in a timely manner or with available funds. We also have not completed all of the characterization and validation activities necessary for potential commercialization and regulatory approvals. If our manufacturing partners do not conduct all such necessary activities in accordance with applicable regulations, our commercialization efforts will be harmed.

Even if our third-party product manufacturers develop an acceptable manufacturing process, if such third-party manufacturers are unable to produce the necessary quantities of our product candidates, or in compliance with cGMP or other pertinent regulatory requirements, and within our planned timeframe and cost parameters, the development and sales of our products, if approved, may be materially harmed.

We and our collaborators, third-party manufacturers and suppliers use biological materials and may use hazardous materials, and any claims relating to improper handling, storage or disposal of these materials could be time consuming or costly.

We and our collaborators, third-party manufacturers and suppliers may use hazardous materials, including chemicals and biological agents and compounds that could be dangerous to human health and safety or the environment. Our operations and the operations of our third-party manufacturers and suppliers also produce hazardous waste products. Federal, state and local laws and regulations govern the use, generation, manufacture, storage, handling and disposal of these materials and wastes. Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our product development efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended.

Any contamination in our or our third parties' manufacturing process, shortages of raw materials or reagents or failure of any of our key suppliers to deliver necessary components of our platform could result in delays in our clinical development or marketing schedules.

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could materially adversely affect our or our third-party vendor's ability to produce our gene therapies on schedule and could therefore harm our results of operations and cause reputational damage.

The raw materials required in our and our third-party vendors manufacturing processes are derived from biological sources. We cannot assure you that we or our third-party vendors have, or will be able to obtain on commercially reasonable terms, or at all, sufficient rights to these materials derived from biological sources. Such raw materials are difficult to procure and may also be subject to contamination or recall. A material shortage, contamination, recall, or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the clinical and commercial manufacturing of our product candidates, which could materially and adversely affect our operating results and development timelines.

We rely on third-party suppliers for the supply and manufacture of certain components of our technology. Should our ability to procure these material components from our suppliers be compromised, our ability to continuously operate would be impaired until an alternative supplier is sourced, qualified and tested, which could limit our ability to produce a clinical and commercial supply of our product candidates and harm our business.

We currently rely on third parties to manufacture our product candidates and we do not have complete control over third-party manufacturers' compliance with cGMP regulations.

Before any of our collaborators, third-party manufacturers and suppliers can begin to commercially manufacture our product candidates, they must demonstrate to regulatory authorities that the planned chemistry, manufacturing and controls for our gene therapy product candidates meet certain requirements. Manufacturing of product candidates for clinical and commercial purposes must comply with the cGMP and applicable non-U.S. regulatory requirements. The cGMP requirements govern quality control and documentation policies and procedures. Complying with cGMP and non-U.S. regulatory requirements will require that we expend time, money and effort in production, recordkeeping and quality control to assure that our product candidates meet applicable specifications and other requirements. Our third-party manufacturers' also must demonstrate to the FDA that they can make the product candidate in accordance with the cGMP requirements as part of a pre-approval inspection prior to FDA approval of the product candidate. Failure to pass a pre-approval inspection might significantly delay FDA approval of our product candidates. If any of our third-party manufacturers fail to comply with these requirements, we would be subject to possible regulatory action, which could limit the jurisdictions in which we are permitted to sell our products. As a result, our business, financial condition and results of operations may be materially harmed.

In addition, our third-party manufacturers may fail to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates.

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

Ethical, social and legal concerns about gene therapy could result in additional regulations restricting or prohibiting our products. Even with the requisite approvals from FDA in the United States, the EMA in the European Union and other regulatory authorities internationally, the commercial success of our product candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and our product candidates in particular, as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of gene therapy products and, in particular, our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy, durability and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of product candidates over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA or the European Commission;
- the willingness of physicians to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;

- product labeling or product insert requirements of the FDA, EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party payor coverage and adequate reimbursement.

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

We face significant competition in an environment of rapid technological change and the possibility that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may harm our business and financial condition, and our ability to successfully market or commercialize our product candidates.

The biotechnology and pharmaceutical industries, including the gene therapy field, are characterized by rapidly changing technologies, competition and a strong emphasis on intellectual property. We are aware of several companies focused on developing gene therapies in various indications as well as several companies addressing other methods for modifying genes and regulating gene expression. We may also face competition from large and specialty pharmaceutical and biotechnology companies, academic research institutions, government agencies and public and private research institutions.

For the treatment of XLMTM, Valerion Therapeutics, LLC is studying VAL-0620, a fusion protein consisting of an antibody linked to MTM1. Preclinical evaluation of this approach in the MTM1 murine model demonstrated improvements in both muscle structure and function, as reported in a 2013 publication. This program has not been reported by Valerion Therapeutics, LLC to have progressed to clinical development.

For the treatment of Crigler-Najjar, the current standard of care is phototherapy, and upon disease progression, liver transplant. There are currently no products approved specifically for the treatment of Crigler-Najjar. Genethon, a French not-for-profit organization, is developing an AAV-UGT1A1 gene therapy for the treatment of Crigler-Najjar syndrome, and has announced plans to initiate clinical development by the end of 2016. Promethera has received orphan drug designation from the FDA and European Commission for the treatment of Crigler-Najjar syndrome for HepaStem, a product that comprises heterologous human adult liver progenitor cells. Promethera previously completed a Phase 1/2 study that enrolled patients with Crigler-Najjar syndrome or ornithine transcarbamylase deficiency. No further development in Crigler-Najjar syndrome has been announced for HepaStem. Additionally, Alexion recently announced that, in collaboration with Moderna, it is developing a messenger RNA product candidate for the treatment of Crigler-Najjar.

For the treatment of Pompe disease, the current standard of care is ERT with recombinant GAA protein. Genzyme Corporation currently markets MYOZYME and LUMIZYME, which are ERTs for the treatment of Pompe disease. Multiple companies, including Genzyme Corporation, Amicus Therapeutics, Inc., Valerion Therapeutics, LLC and Oxyrane UK Limited are currently reported to be developing next generation ERT to treat Pompe disease. The furthest advanced of these is neoGAA from Genzyme Corporation. In addition, there are currently multiple academic institutions and companies researching alternative gene therapy approaches to treating Pompe disease. We do not believe these approaches utilize AAV9 capsids and none are currently reported to be in clinical development.

For the treatment of CASQ2-CPVT, nadolol or propranolol are currently used as first-line treatment sometimes with the addition of a calcium channel blocker such as verapamil. The sodium channel blocker flecainide, and implantable cardioverter defibrillators are also currently used in the treatment of CASQ2-CPVT. Heart transplant is used infrequently as a last-line therapy in refractory cases of CPVT. Additionally, there are no known investigational therapies in development for CASQ2-CPVT.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources than we do, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of competitors. Our commercial opportunity could be reduced or eliminated if 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 product candidates that we may develop. Competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market, if ever. Additionally, new or advanced technologies developed by our competitors may render our current or future product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors.

To become and remain profitable, we must develop and eventually commercialize product candidates with significant market potential, which will require us to be successful in a range of challenging activities. These activities can include completing preclinical studies and initiating and completing clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products that are approved and satisfying any post marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never generate revenues that are significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

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

Our target indications, including XLMTM, Crigler-Najjar, Pompe disease and CASQ2-CPVT, are indications with small patient populations. In order for products that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such products must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved product candidate that accounts for the smaller potential market size. If we are unable to establish or sustain coverage and adequate reimbursement for any future product candidates from third-party payors, the adoption of those products and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product candidates, if approved.

We expect the cost of a single administration of gene therapy products, such as those we are developing, to be substantial when and if they achieve regulatory approval. Therefore, we expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of any of our product candidates will depend substantially, both domestically and internationally, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement for new products are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, since CMS decides whether and to what extent a new product will be covered and reimbursed under Medicare. Private payors tend to follow CMS to a substantial degree. However, one payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product. Further, a payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. It is difficult to predict what CMS will decide with respect to reimbursement for novel products such as ours since there is no body of established practices and precedents for these new products. Reimbursement agencies in Europe may be more conservative than CMS. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as our product candidates. In many countries, particularly the countries of the European Union, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, the prices of products under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our product candidates may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Moreover, increasing efforts by governmental and third-party payors, in the United States and internationally, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of certain third-party payors, such as health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products into the healthcare market.

In addition to CMS and private payors, professional organizations such as the American Medical Association, or the AMA, can influence decisions about reimbursement for new products by determining standards for care. In addition, many private payors contract with commercial vendors who sell software that provide guidelines that attempt to limit utilization of, and therefore reimbursement for, certain products deemed to provide limited benefit to existing alternatives. Such organizations may set guidelines that limit reimbursement or utilization of our product candidates. Even if favorable coverage and reimbursement status is attained for one or more products for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

If in the future we are unable to establish U.S. or global sales and marketing capabilities or enter into agreements with third parties to sell and market our product candidates, we may not be successful in commercializing our product candidates if they are approved and we may not be able to generate any revenue.

We currently do not have a marketing or sales team for the marketing, sales and distribution of any of our product candidates that are able to obtain regulatory approval. In order to commercialize any product

candidates after approval, we must build on a territory-by-territory basis marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services, and we may not be successful in doing so. If our product candidates receive regulatory approval, we may decide to establish an internal sales or marketing team with technical expertise and supporting distribution capabilities to commercialize our product candidates, which will be expensive and time-consuming and will require significant attention of our executive officers to manage. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of any of our product candidates that we obtain approval to market.

With respect to the commercialization of all or certain of our product candidates, we may choose to collaborate, either globally or on a territory-by-territory basis, with third parties that have direct sales forces and established distribution systems, either to augment our own sales force and distribution systems or in lieu of our own sales force and distribution systems. If we are unable to enter into such arrangements when needed on acceptable terms, or at all, we may not be able to successfully commercialize any of our product candidates that receive regulatory approval or any such commercialization may experience delays or limitations. If we are not successful in commercializing our product candidates, either on our own or through collaborations with one or more third parties, our future product revenue will suffer and we may incur significant additional losses.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

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

We may not be successful in finding strategic collaborators for continuing development of certain of our product candidates or successfully commercializing or competing in the market for certain indications.

In addition to our relationships with Genethon and the University of Pennsylvania, for some of our product candidates, we may decide to collaborate with additional pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates. We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing drugs, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. The terms of any additional collaborations or other arrangements that we may establish may not be favorable to us.

We may also be restricted under existing collaboration agreements from entering into future agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate additional collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

In addition, our collaborations with Genethon and the University of Pennsylvania, and any future collaborations that we enter into, may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration would adversely affect us financially and could harm our business reputation.

Risks Related to Our Financial Position

We have a history of operating losses, and we may not achieve or sustain profitability. We anticipate that we will continue to incur losses for the foreseeable future. If we fail to obtain additional funding to conduct our planned research and development effort, we could be forced to delay, reduce or eliminate our product development programs or commercial development efforts.

We are an early-stage biotechnology company with a limited operating history on which to base your investment decision. Biotechnology product development is a highly speculative undertaking and involves a substantial degree of risk. Our operations to date have been limited primarily to organizing and staffing our company, business planning, raising capital, acquiring and developing product and technology rights and conducting preclinical research and development activities for our product candidates. We have never generated any revenue from product sales. We have not obtained regulatory approvals for any of our product candidates, and have funded our operations to date through proceeds from sales of our preferred stock.

We have incurred net losses in each year since our inception. We incurred net losses of $10.8 million and $26.5 million for the years ended December 31, 2014 and 2015, respectively, and net losses of $4.1 million and $10.5 million for the three months ended March 31, 2015 and 2016, respectively. As of March 31, 2016, we had an accumulated deficit of $51.2 million. Substantially all of our operating losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We expect to continue to incur significant expenses and operating losses over the next several years and for the foreseeable future as we intend to continue to conduct research and development, clinical testing, regulatory compliance activities, manufacturing activities, and, if any of our product candidates is approved, sales and marketing activities that, together with anticipated general and administrative expenses, will likely result in us incurring significant losses for the foreseeable future. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' deficit and working capital.

Even if this offering is successful, we expect that we will need to raise additional funding before we can expect to become profitable from any potential future sales of our products. This additional financing may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit, or terminate our product development efforts or other operations.

We will require substantial future capital in order to complete planned and future preclinical and clinical development for AT132, AT342, AT982, AT307 and other future product candidates, if any, and potentially commercialize these product candidates. We expect our spending levels to increase in connection with our preclinical studies and planned clinical trials, if any, of our lead product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. Furthermore, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate certain of our licensing activities, our research and development programs or other operations.

Our operations have consumed significant amounts of cash since inception. As of March 31, 2016, our cash, cash equivalents and investments were $80.3 million. We estimate that the net proceeds from this offering will be approximately $66.1 million, based on an assumed initial public offering price of $15.00 per share, the midpoint of the estimated offering price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses. We expect that the net proceeds from this offering, together with our existing cash, cash equivalents and investments, will enable us to fund our operating expenses and capital expenditure requirements through 2018. See “Use of Proceeds” for more information.

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

- the costs associated with the scope, progress and results of discovery, preclinical development, laboratory testing and clinical trials for our product candidates;
- the costs associated with the development of our internal manufacturing facility and processes;
- the costs related to the extent to which we enter into partnerships or other arrangements with third parties in order to further develop our product candidates;
- the costs and fees associated with the discovery, acquisition or in-license of product candidates or technologies;
- our ability to establish collaborations on favorable terms, if at all;
- the costs of future commercialization activities, if any, including product sales, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval; and
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims.

Our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of product candidates that we do not expect to be commercially available for

many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives, which may not be available to us on acceptable terms, or at all.

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

We are a preclinical company formed in November 2012. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring our technology, identifying potential product candidates and undertaking research and preclinical studies of our product candidates and establishing licensing arrangements. We have not yet demonstrated the ability to complete and report preclinical or clinical trials of our product candidates, obtain marketing approvals, manufacture a commercial scale product or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a licensing and research focus to a company that is also capable of supporting clinical development and commercial activities. We may not be successful in such a transition.

Our ability to utilize our net operating loss carryforwards may be subject to limitation.

We have incurred substantial losses during our history and do not expect to become profitable in the near future and we may never achieve profitability. As of December 31, 2015, we had federal and state net operating loss carryforwards of $32.1 million and $34.4 million, respectively, both of which begin to expire in 2033. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset future taxable income, if any, until such unused losses expire. Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards, or NOLs, and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. We may experience ownership changes in the future as a result of subsequent shifts in our stock ownership. 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 be subject to limitations, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

Risks Related to Intellectual Property

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 commercial success depends in part on our ability to obtain and maintain proprietary or intellectual property protection in the United States and other countries for our current product candidates and future products, as well as our core technologies, including our manufacturing know-how. We strive to protect and enhance the proprietary technology, inventions and improvements that are commercially important to the development of our business by seeking, maintaining and defending our intellectual property, whether developed internally or licensed from third parties. We also rely on trade secrets, know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position in the field

of gene therapy. Additionally, we intend to rely on regulatory protection afforded through rare drug designations, data exclusivity and market exclusivity as well as patent term extensions, where available.

Our in-licensed patents and patent applications are directed to the compositions of matter and methods of use related to various aspects of our product candidates as well as certain aspects of our manufacturing capabilities. As of March 31, 2016, we had filed one U.S. provisional patent application directed to modified AAV vectors and methods of manufacturing the same. If granted, we expect this patent would expire in 2036. We have in-licensed patents and patent applications owned by the Trustees of the University of Pennsylvania, or the University of Pennsylvania, relating to various AAV vectors. These patents and patent applications are licensed or sublicensed to REGENXBIO and sublicensed to us. Our first sublicense is exclusive in the field of treatment of XLMTM and Pompe disease in humans by *in vivo* gene therapy using AAV8 and AAV9. Our second sublicense is exclusive in the field of treatment of CPVT in humans by *in vivo* gene therapy using AAV9. Our third sublicense is exclusive in the field of treatment of Crigler-Najjar syndrome in humans by *in vivo* gene therapy using AAV8. These sublicenses are subject to certain retained rights. We have also in-licensed a patent family owned by the Fondazione Salvatore Maugeri, or FSM, relating to gene therapy of recessive CPVT. We have also in-licensed certain patents, rights and know-how from the University of Florida Research Foundation for the treatment of Pompe, and certain intellectual property rights controlled by Genethon for the treatment of XLMTM. Additionally, since March 31, 2016, we have in-licensed a patent application from the University of Pennsylvania relating to AAV vectors containing codon-optimized UGT1A1 for the treatment of Crigler-Najjar. As described in "Business—Intellectual Property," the REGENXBIO patents will expire between 2022 and 2026, the FSM patents will expire by 2032 and the Genethon patent family will expire by 2034, and a patent obtained from the patent application filed by the University of Pennsylvania directed to UGT1A1 would be projected to expire in 2036 absent patent term adjustment or patent term extension. 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.

The degree of patent protection we require to successfully compete in the marketplace may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our licensed patents have, or that any of our pending licensed patent applications that mature into issued patents will include, claims with a scope sufficient to protect our current and future product candidates or otherwise provide any competitive advantage. The FSM and Genethon patent families were filed only in the United States, and therefore these patent families will not provide patent protection outside the United States. While other patent families include foreign counterparts, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. In addition, none of the patent applications licensed from the University of Florida Research Foundation relating to gene therapy for Pompe disease have matured into issued patents in the United States. Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally twenty years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. If any of our AT132, AT342, AT982 or AT307 product candidates are approved by the FDA as a biological product under a BLA in the United States, we believe the product would qualify for a 12-year period of exclusivity. For example, if our AT132 product was approved by the FDA as a biological product under a BLA in 2020, we believe it would qualify for a 12-year period of exclusivity, which would expire in 2032, or two years before the Genethon patent family will expire in the United States absent patent term adjustment or patent term extension. Similarly, if our AT307 product was approved by the FDA as a biological product under a BLA in 2020, we believe it would qualify for a 12-year period of exclusivity, which would expire in 2032, the same year the FSM patent family will expire in the United States absent patent term adjustment or patent term extension. Moreover, our exclusive license is subject to retained rights, which may adversely impact our competitive position. As a result, our licensed patent portfolio may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing products similar to our product candidates, including biosimilar versions of such products. In addition, the patent portfolio licensed to us is, or may be,

licensed to third parties, such as outside our field, and such third parties may have certain enforcement rights. Thus, patents licensed to us could be put at risk of being invalidated or interpreted narrowly in litigation filed by or against another licensee or in administrative proceedings brought by or against another licensee in response to such litigation or for other reasons.

Other parties have developed technologies that may be related or competitive to our own and such parties may have filed or may file patent applications, or may have received or may receive patents, claiming inventions that may overlap or conflict with those claimed in our own patent applications or issued patents. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and in other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether the inventors of our licensed patents and applications were the first to make the inventions claimed in those patents or pending patent applications, or that they were the first to file for patent protection of such inventions. Further, we cannot assure you that all of the potentially relevant prior art relating to our licensed patents and patent applications has been found. If such prior art exists, it can invalidate a patent or prevent a patent from issuing from a pending patent application. As a result, the issuance, scope, validity and commercial value of our patent rights cannot be predicted with any certainty.

In addition, the patent prosecution process is expensive and time-consuming, and we or our licensors may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we or our licensors will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We cannot provide any assurances that we will be able to pursue or obtain additional patent protection based on our research and development efforts, or that any such patents or other intellectual property we generate will provide any competitive advantage. Patent prosecution is a lengthy process and the scope of the claims initially submitted for examination may be significantly narrowed by the time they issue, if at all. Moreover, we do not have the right to control the preparation, filing and prosecution of patent applications, or to control the maintenance of the patents, covering technology that we license from third parties. Therefore, these patents and applications may not be filed, prosecuted or maintained in a manner consistent with the best interests of our business.

Even if we acquire patent protection that we expect should enable us to maintain competitive advantage, third parties, including competitors, may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. In litigation, a competitor could claim that our patents, if issued, are not valid for a number of reasons. If a court agrees, we would lose our rights to those challenged patents.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability and our licensed patents may be challenged in courts or patent offices in the United States and abroad. For example, we may be subject to a third-party submission of prior art to the U.S. Patent and Trademark Office, or USPTO, challenging the validity of one or more claims of our licensed patents. Such submissions may also be made prior to a patent's issuance, precluding the granting of a patent based on one of our pending licensed patent applications. We may become involved in opposition, derivation, reexamination, *inter partes* review, post-grant review, interference, or similar proceedings in the United States or abroad, challenging the patent rights of others from whom we have obtained licenses to such rights. Furthermore, our licensed patents may be challenged in district court. Competitors may claim that they invented the inventions claimed in such issued patents or patent applications prior to the inventors of our licensed patents, or may have filed patent applications before the inventors of our licensed patents did. A competitor may also claim that we are infringing its patents and that we therefore cannot practice our technology as claimed under our licensed patents, if issued. As a result, one or more claims of our licensed patents may be narrowed or invalidated.

Even if they are unchallenged, our licensed patents and pending patent applications, if issued, may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to

circumvent our licensed patents by developing similar or alternative technologies or therapeutics in a non-infringing manner. For example, even if we have a valid and enforceable patent, we may not be able to exclude others from practicing our invention if the other party can show that they used the invention in commerce before our filing date or the other party benefits from a compulsory license. Moreover, a third party may develop a competitive product that provides benefits similar to one or more of our product candidates but that uses a vector or an expression construct that falls outside the scope of our patent protection or license rights. If the patent protection provided by the patents and patent applications we hold or pursue with respect to our product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our product candidates could be negatively affected, which would harm our business. Although currently all of our patents and patent applications are in-licensed, similar risks would apply to any patents or patent applications that we may own or in-license in the future.

If we breach our license agreements it could have a material adverse effect on our commercialization efforts for our product candidates.

If we breach any of the agreements under which we license the use, development and commercialization rights to our product candidates or technology from third parties, we could lose license rights that are important to our business. We currently hold licenses or other rights for certain intellectual property from REGENXBIO relating to various AAV vectors, from Genethon related to XLMTM, from the University of Pennsylvania relating to Crigler-Najjar, from the University of Florida Research Foundation relating to Pompe disease and from the Fondazione Salvatore Maugeri relating to various nucleic acid sequences associated with single mutation arrhythmias related to CASQ2-CPVT.

Under our existing license agreements, we are subject to various obligations, including diligence obligations such as development and commercialization obligations, as well as potential royalty payments and other obligations. If we fail to comply with any of these obligations or otherwise breach our license agreements, our licensors may have the right to terminate the applicable license in whole or in part. Generally, the loss of any one of our current licenses, or any other license we may acquire in the future, could harm our business, prospects, financial condition and results of operations.

Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other intellectual property rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- whether and the extent to which inventors are able to contest the assignment of their rights to our licensors.

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. In addition, if disputes arise as to ownership of licensed intellectual property, our ability to pursue or enforce the licensed patent rights may be jeopardized. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize our products could suffer.

All of our current product candidates are licensed from or based upon licenses from third parties. If any of these license or sublicense agreements are terminated or interpreted to narrow our rights, our ability to advance our current product candidates or develop new product candidates based on these technologies will be materially adversely affected.

We now depend, and will continue to depend, on licenses and sublicenses from third parties and potentially on other strategic relationships with third parties for the research, development, manufacturing and commercialization of our current product candidates. If any of our licenses or relationships or any in-licenses on which our licenses are based are terminated or breached, we may:

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

Additionally, even if not terminated or breached, our intellectual property licenses or sublicenses 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.

If we experience any of the foregoing, it could have a materially adverse effect on our business and could force us to cease operations which could cause you to lose all of your investment.

We are required to pay certain royalties under our license agreements with third-party licensors, and we must meet certain milestones to maintain our license rights.

Under our license agreements with REGENXBIO, the University of Florida, the University of Pennsylvania and FSM, we will be required to pay royalties based on our net revenues from sales of our products utilizing the technologies and products. These royalty payments could adversely affect the overall profitability for us of any products that we may seek to commercialize. In order to maintain our license rights under these license agreements, we will need to meet certain specified milestones, subject to certain cure provisions, in the development of our product candidates and in the raising of funding. In addition, these agreements contain development obligations and we may not be successful in meeting all of the obligations in the future on a timely basis or at all. We may need to outsource and rely on third parties for many aspects of the clinical development, sales and marketing of our products covered under our license agreements. Delay or failure by any such third parties could adversely affect the continuation of our license agreements with third-party licensors. For example, our Exclusive License Agreement with Know-How with the University of Florida Research Foundation provides that the University of Florida Research Foundation has the right to terminate the agreement if we do not meet certain deadlines, such as submitting an IND or foreign equivalent in any country by October 31, 2016 or dosing the first patient in clinical trials in any country by March 31, 2017.

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

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and future products 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 frequent litigation regarding patents and other intellectual property rights. We may in the future become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates, future products and technology, including interference or *inter partes* review proceedings before the USPTO. Our competitors or other third parties may assert infringement or misappropriation claims against us, alleging that our therapeutics, manufacturing methods, formulations or administration methods are covered by their patents. For example, we do not know which processes we will use for commercial manufacture of our future products, or which technologies owned or controlled by third parties may prove important or essential to those processes. Given the vast number of patents in our field of technology, we cannot be certain or guarantee that we do not infringe existing patents or that we will not infringe patents that may be granted in the future. Many companies have filed, and continue to file, patent applications related to gene therapy and orphan diseases. Some of these patent applications have already been allowed or issued and others may issue in the future. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed and additional patents granted in the future, as well as additional research and development programs expected in the future. Furthermore, because patent applications can take many years to issue, may be confidential for 18 months or more after filing and can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use, sale or importation of our product candidates or future products. If a patent holder believes the manufacture, use, sale or importation of one of our product candidates or future products infringes its patent, the patent holder may sue us even if we have licensed other patent protection for our technology. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant product revenue and against whom our licensed patent portfolio may therefore have no deterrent effect.

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

Third parties may assert infringement claims against us based on existing intellectual property rights and intellectual property rights that may be granted in the future. If we were to challenge the validity of an issued U.S. patent in court, such as an issued U.S. patent of potential relevance to some of our product candidates or future products or manufacture or methods of use, we would need to overcome a statutory presumption of validity that attaches to every U.S. patent. This means that in order to prevail, we would have to present clear and convincing evidence as to the invalidity of the patent's claims. There is no assurance that a court would find in our favor on questions of infringement or validity.

Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcome is uncertain. If we are found, or believe there is a risk we may be found, to infringe a third

party's intellectual property rights, we could be required or may choose to obtain a license from such third party to continue developing and marketing our products and technology. However, we may not be able to obtain any such license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. Without such a license, we could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our future products or force us to cease some of our business operations, which could materially harm our business. Alternatively, we may need to redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. If we lose a foreign patent lawsuit, alleging our infringement of a competitor's patents, we could be prevented from marketing our therapeutics in one or more foreign countries and/or be required to pay monetary damages for infringement or royalties in order to continue marketing. Claims that we have misappropriated the confidential information, trade secrets or other intellectual property of third parties could have a similar negative impact on our business. Any of these outcomes would have a materially adverse effect on our business.

Even if we are successful in these proceedings, we may incur substantial costs and divert management time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, defend an infringement action or challenge the validity of the patents in court, or redesign our future products or processes. Patent litigation is costly and time-consuming, and some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. We may not have sufficient resources to bring these actions to a successful conclusion. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could delay our research and development efforts and limit our ability to continue our operations.

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

In addition to the protection afforded by patents, we rely upon unpatented trade secret protection, unpatented know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our contractors, collaborators, scientific advisors, employees and consultants and invention assignment agreements with our consultants and employees. We may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements, however, despite the existence generally of confidentiality agreements and other contractual restrictions. Monitoring unauthorized uses and disclosures is difficult and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the contractors, collaborators, scientific advisors, employees and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets. Enforcing a claim that a third party illegally obtained and is using our trade secrets, like patent litigation, is expensive and time consuming and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing or unwilling to protect trade secrets.

Our trade secrets could otherwise become known or be independently discovered by our competitors. Competitors could purchase our product candidates and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our protected technology or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If our trade secrets are not adequately protected or sufficient to provide an advantage over our competitors, our competitive position could be adversely affected, as could our

business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating our trade secrets.

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

The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In addition, periodic maintenance fees on issued patents often must be paid to the USPTO and foreign patent agencies over the lifetime of the patent. While an unintentional lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance 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. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, we may not be able to stop a competitor from marketing drugs that are the same as or similar to our product candidates, which would have a material adverse effect on our business.

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

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

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

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States

can be less extensive than those in the United States. The requirements for patentability may differ in certain countries, particularly in developing countries. Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. 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. Further, licensing partners may not prosecute patents in certain jurisdictions in which we may obtain commercial rights, thereby precluding the possibility of later obtaining patent protection in these countries. For example, the FSM and Genethon patent families were only filed in the United States, and therefore these patent families will not provide patent protection outside 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 may also export 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, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, 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.

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

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention. An adverse outcome in a litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors, and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings.

Changes to the patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Recent patent reform legislation in the United States and other countries, including the Leahy-Smith America Invents Act, or Leahy-Smith Act, signed into law in September 2011, could increase those uncertainties and costs. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. In addition, the Leahy-Smith Act has transformed the U.S. patent system into a "first to file" system. The first-to-file provisions, however, only became effective in March 2013. Accordingly, it is not yet 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 make it more difficult to obtain patent protection for our inventions and 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, results of operations and financial condition.

The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. For example, in Association for Molecular Pathology v. Myriad Genetics, Inc., the Supreme Court ruled that a "naturally occurring DNA segment is a product of nature and not patent eligible merely because it has been isolated," and invalidated Myriad Genetics's patents on the BRCA1 and BRCA2 genes. Certain claims of our licensed patents relate to isolated AAV vectors, capsid proteins, or nucleic acids. To the extent that such claims are deemed to be directed to natural products, or to lack an inventive concept above and beyond an isolated natural product, a court may decide the claims are invalid under Myriad. Additionally, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could impact our ability to obtain patent protection for our proprietary technology or our ability to enforce our proprietary technology. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We 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, and the employees, consultants or advisors of our licensors, are currently, or were previously, employed at or affiliated with universities, hospitals 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. Moreover, some of our and our licensors' employees, consultants or advisors are or have been affiliated with multiple institutions. There is not guarantee that such institutions will not challenge our or our licensors' intellectual property ownership rights. 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.

Risks Related to Employee Matters, Managing Growth and Other Risks Related to Our Business

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.

As of May 31, 2016, we had 75 full-time employees. As our development, manufacturing and commercialization plans and strategies develop, and as we transition into operating as a public company, we expect to need and are actively recruiting additional managerial, operational, sales, marketing, financial and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining and motivating additional employees;
- managing our internal development efforts effectively, including the clinical, FDA and international regulatory review process for our product candidates, while complying with our contractual obligations to contractors and other third parties; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to develop, manufacture and commercialize our product candidates will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert financial and other resources, and a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time, to managing these growth activities.

We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services, including substantially all aspects of regulatory approval, clinical management and manufacturing. We cannot assure you that the services of independent organizations, advisors and consultants will continue to be available to us on a timely basis when needed, or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval of our product candidates or otherwise advance our business. We cannot assure you that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to

further develop and commercialize our product candidates and, accordingly, may not achieve our research, development and commercialization goals.

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

We are highly dependent on the research and development, clinical and business development expertise of Matthew Patterson, our President and Chief Executive Officer, Dr. Suyash Prasad, our Chief Medical Officer, Natalie Holles, our Chief Operating Officer, and Thomas Soloway, our Chief Financial Officer, as well as the other principal members of our management, scientific and clinical team. Although we have entered into employment letter agreements or employment agreements with our executive officers, each of them may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and manufacturing strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

Recruiting and retaining qualified scientific, clinical, manufacturing and, if needed, sales and marketing personnel will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize drugs. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. Failure to succeed in clinical trials may make it more challenging to recruit and retain qualified scientific personnel.

If we fail to establish and maintain proper and effective internal control over financial reporting, our operating results and our ability to operate our business could be harmed.

Ensuring that we have adequate internal financial and accounting controls and procedures in place so that we can produce accurate financial statements on a timely basis is a costly and time-consuming effort that needs to be re-evaluated frequently. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with generally accepted accounting principles. In connection with this offering, we intend to begin the process of documenting, reviewing and improving our internal controls and procedures for compliance with Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, which will require annual management assessment of the effectiveness of our internal control over financial reporting beginning with the year ended December 31, 2017.

During the audit of our financial statements for the years ended December 31, 2014 and 2015 a material weakness was identified in our internal control over financial reporting. Under standards established by the Public Company Accounting Oversight Board, a material weakness is a deficiency or combination of deficiencies in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected and corrected on a timely basis. The material weakness that was identified related to a lack of sufficient accounting resources and personnel that limits our ability to adequately segregate duties, establish defined accounting policies and procedures and perform timely reviews of account reconciliations.

We have implemented and are continuing to implement measures designed to improve our internal control over financial reporting to address the underlying causes of this material weakness, including the hiring of our Chief Financial Officer and other accounting personnel and establishing new accounting and financial reporting procedures, policies and processes to have in place an appropriate level of internal control over financial reporting. However, we are still in the process of implementing these measures and cannot assure you that we will be successful in doing so or that these measures will significantly improve or remediate the material weakness described above. We, and our independent registered public accounting firm, were not required to perform an evaluation of our internal control over financial reporting as of December 31, 2015 in accordance with the provisions of the Sarbanes-Oxley Act. Accordingly, we cannot assure you that we have identified all, or that we will not in the future have additional, material weaknesses. Material weaknesses may still exist when we report on the effectiveness of our internal control over financial reporting as required by reporting requirements under Section 404 of the Sarbanes-Oxley Act after the completion of this offering. If we are unable to successfully remediate the existing material weakness in our internal control over financial reporting, the accuracy and timing of our financial reporting, and our stock price, may be adversely affected and we may be unable to maintain compliance with the applicable stock exchange listing requirements.

Implementing any appropriate changes to our internal controls may distract our officers and employees, entail substantial costs to modify our existing processes and take significant time to complete. These changes may not, however, be effective in maintaining the adequacy of our internal controls, and any failure to maintain that adequacy, or consequent inability to produce accurate financial statements on a timely basis, could increase our operating costs and harm our business. In addition, investors' perceptions that our internal controls are inadequate or that we are unable to produce accurate financial statements on a timely basis may harm our stock price and make it more difficult for us to effectively market and sell our service to new and existing customers.

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

As a public company, particularly after we are no longer an "emerging growth company," we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002 and rules subsequently implemented by the Securities and Exchange Commission and The NASDAQ Stock Market LLC, or NASDAQ, have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly.

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

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

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We will remain an emerging growth company until the earlier of (i) the last day of the fiscal year in which we have total annual gross revenue of $1 billion or more; (ii) the last day of the fiscal year following the fifth anniversary of the date of the completion of this offering; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the last business day of our most recently completed second fiscal quarter. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- being permitted to present only two years of audited financial statements in addition to any required unaudited interim financial statements with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure in this prospectus;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

We may choose to take advantage of some, but not all, of the available exemptions. We have taken advantage of reduced reporting burdens in this prospectus. In particular, we have provided only two years of audited financial statements and have not included all of the executive compensation information that would be required if we were not an emerging growth company. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Recent and future acquisitions or strategic alliances could disrupt our business and harm our financial condition and operating results.

We may acquire additional businesses or drugs, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business, including, for example our August 2015 acquisition of Cardiogen Sciences, Inc., or Cardiogen. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable

to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new drugs resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction. The risks we face in connection with acquisitions, including our recent acquisition of Cardiogen, include:

- diversion of management time and focus from operating our business to addressing acquisition integration challenges;
- coordination of research and development efforts;
- retention of key employees from the acquired company;
- changes in relationships with strategic partners as a result of product acquisitions or strategic positioning resulting from the acquisition;
- cultural challenges associated with integrating employees from the acquired company into our organization;
- the need to implement or improve controls, procedures, and policies at a business that prior to the acquisition may have lacked sufficiently effective controls, procedures and policies;
- liability for activities of the acquired company before the acquisition, including intellectual property infringement claims, violation of laws, commercial disputes, tax liabilities, and other known liabilities;
- unanticipated write-offs or charges; and
- litigation or other claims in connection with the acquired company, including claims from terminated employees, customers, former stockholders or other third parties.

Our failure to address these risks or other problems encountered in connection with our past or future acquisitions or strategic alliances could cause us to fail to realize the anticipated benefits of these transactions, cause us to incur unanticipated liabilities and harm the business generally. There is also a risk that future acquisitions will result in the incurrence of debt, contingent liabilities, amortization expenses or incremental operating expenses, any of which could harm our financial condition or operating results.

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

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. For example, the global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the global financial crisis, could result in a variety of risks to our business, including, weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

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

Natural disasters could severely disrupt our operations and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our manufacturing facilities, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business.

Our internal computer systems, or those of our third-party collaborators or other contractors, may fail or suffer security breaches, which could result in a material disruption of our development programs.

Our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations, 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. 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.

Our employees, principal investigators, CROs and consultants may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with the regulations of FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of clinical studies, which could result in regulatory sanctions and cause serious harm to our reputation. We intend to adopt a code of conduct applicable to all of our employees upon the completion of this offering, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

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

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

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

We currently maintain product liability insurance coverage of up to $5.0 million, which may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage when we begin clinical trials and if we successfully commercialize any product candidate. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, anti-corruption laws, and anti-money laundering laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic and international markets. We can face criminal liability and other serious consequences for violations which can harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, the U.S. Foreign Corrupt Practices Act of 1977, as amended, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, contractors, and other partners from authorizing, promising, offering, or providing, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may engage third parties for clinical trials outside of the United States, to sell our products abroad once we enter a commercialization phase, and/or to obtain necessary permits, licenses, patent registrations, and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors, and other partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences.

Risks Related to Our Common Stock and This Offering

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock in this offering.

Our stock price is likely to be volatile. The stock market in general and the market for biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the initial public offering price. The market price for our common stock may be influenced by many factors, including:

- the success of competitive drugs or technologies;
- results of preclinical studies or clinical trials of our product candidates or those of our competitors;
- unanticipated or serious safety concerns related to the use of any of our product candidates;
- adverse regulatory decisions, including failure to receive regulatory approval for any of our product candidates;
- regulatory or legal developments in the United States and other countries;
- the size and growth of our prospective patient populations;
- developments concerning our collaborators, our external manufacturers or in-house manufacturing capabilities;
- inability to obtain adequate product supply for any product candidate for preclinical studies, clinical trials or future commercial sale or inability to do so at acceptable prices;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or drugs;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the biotechnology sector;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section.

An active trading market for our common stock may not develop and you may not be able to resell your shares at or above the initial public offering price.

Prior to this offering, there has been no public market for shares of our common stock. Although we have applied to list our common stock on NASDAQ, an active trading market for our shares may never develop or be sustained following this offering. The initial public offering price of our common stock will be determined through negotiations between us and the underwriters. This initial public offering price may not be indicative of the market price of our common stock after this offering. In the absence of an active trading market for our common stock, investors may not be able to sell their common stock at or above the initial public offering price or at the time that they would like to sell.

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

The trading market for our common stock will rely in part on the research and reports that industry or financial analysts publish about us or our business. We may never obtain research coverage by industry or financial analysts. If no or few analysts commence coverage of us, the trading price of our stock would likely decrease. Even if we do obtain analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

Our executive officers, directors, principal stockholders and their affiliates will continue to exercise significant influence over our company after this offering, which will limit your ability to influence corporate matters and could delay or prevent a change in corporate control.

As of May 31, 2016, our executive officers, directors, holders of 5% or more of our capital stock and their respective affiliates beneficially owned approximately 75% of our capital stock and, upon the closing of this offering, that same group will hold approximately 57% of our outstanding capital stock (assuming no exercise of the underwriters' option to purchase additional shares, no exercise of outstanding options and no purchases of shares in this offering by any members of this group), in each case assuming the conversion of all outstanding shares of our convertible preferred stock into shares of our common stock immediately prior to the completion of this offering.

After this offering, this group of stockholders will have the ability to control us through this ownership position even if they do not purchase any additional shares in this offering. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents or approval of any merger, sale of assets or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders. The interests of this group of stockholders may not always coincide with your interests or the interests of other stockholders and they may act in a manner that advances their best interests and not necessarily those of other stockholders, including seeking a premium value for their common stock, and might affect the prevailing market price for our common stock.

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

We expect that significant additional capital may be needed in the future to continue our planned operations, including conducting our planned clinical trials, manufacturing and commercialization efforts, expanded research and development activities and costs associated with operating as a public company. To raise

capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. After this offering, we will have 21,020,378 shares of common stock outstanding based on the number of shares outstanding as of March 31, 2016. This includes the 5,000,000 shares that we sell in this offering, which may be resold in the public market immediately without restriction. The remaining 16,020,378 shares of our common stock will be subject to lock-up agreements with the underwriters of this offering and/or market standoff agreements that restrict the stockholders' ability to transfer shares of our common stock for 180 days from the date of this prospectus. Moreover, after this offering, holders of an aggregate of 15,375,756 shares of our common stock will have rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also intend to register all shares of common stock that we may issue under our equity incentive plans. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements described in the "Underwriting" section of this prospectus.

Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

We have never declared or paid any cash dividends on our common stock and do not currently intend to do so for the foreseeable future. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will be limited to the appreciation of stock. Therefore, the success of an investment in shares of our common stock will depend upon any future appreciation in value of the stock. We cannot guarantee you that shares of our common stock will appreciate in value or even maintain the price at which our stockholders have purchased their shares.

Our management will have broad discretion over the use of the proceeds we receive in this offering and might not apply the proceeds in ways that increase the value of your investment.

Our management will have broad discretion to use the net proceeds from this offering, including for any of the purposes described in the section entitled "Use of Proceeds," and you will be relying on the judgment of our management regarding the application of these proceeds. You will not have the opportunity to influence our decisions on how to use the proceeds, and we may not apply the net proceeds of this offering in ways that increase the value of your investment. Because of the number and variability of factors that will determine our use of the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. We expect to use the net proceeds from this offering to advance preclinical and clinical development of AT132, AT342, AT982 and AT307; to operate and expand our internal manufacturing facility; and for general corporate purposes, including working capital. We may also use a portion of the proceeds for the acquisition of, or investment in, technologies, intellectual property or businesses that complement our business, although we have no present commitments or agreements to this effect. The failure by our management to apply these funds effectively could harm our business. Pending their use, we intend to invest the net proceeds from this offering in marketable securities that may include investment-grade interest-bearing securities, money market accounts, certificates of deposit, commercial paper and guaranteed obligations of the U.S. government. These investments may not yield a favorable return to our stockholders. If we do not invest or apply the net proceeds from this offering in ways that enhance stockholder value, we may fail to achieve expected financial results, which could cause our stock price to decline.

If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the book value of your shares.

You will suffer immediate and substantial dilution in the net tangible book value of our common stock you purchase in this offering. Assuming an initial public offering price of $15.00 per share, the midpoint of the

estimated offering price range set forth on the cover page of this prospectus, purchasers of common stock in this offering will experience immediate dilution of $8.19 per share in net tangible book value of our common stock. In the past, we issued options and other securities to acquire common stock at prices below the initial public offering price. To the extent these outstanding securities are ultimately exercised, investors purchasing common stock in this offering will sustain further dilution. See "Dilution" for a more detailed description of the dilution to new investors in the offering.

Our amended and restated certificate of incorporation will designate the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, employees or agents.

Our amended and restated certificate of incorporation that will become effective prior to the completion of this offering will provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or agents to us or our stockholders, any action asserting a claim arising pursuant to any provision of the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws or any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein and the claim not being one which is vested in the exclusive jurisdiction of a court or forum other than the Court of Chancery or for which the Court of Chancery does not have subject matter jurisdiction. Any person purchasing or otherwise acquiring any interest in any shares of our capital stock shall be deemed to have notice of and to have consented to this provision of our amended and restated certificate of incorporation. This choice of forum provision may limit our stockholders' ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, employees or agents, which may discourage such lawsuits against us and our directors, officers, employees and agents even though an action, if successful, might benefit our stockholders. Stockholders who do bring a claim in the Court of Chancery could face additional litigation costs in pursuing any such claim, particularly if they do not reside in or near Delaware. The Court of Chancery may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments or results may be more favorable to us than to our stockholders. Alternatively, if a court were to find this provision of our amended and restated certificate of incorporation inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs associated with resolving such matters in other jurisdictions, which could have a material adverse effect on our business, financial condition or results of operations.

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

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

- establish a classified board of directors so that not all members of our board are elected at one time;

- permit only the board of directors to establish the number of directors and fill vacancies on the board;

- provide that directors may only be removed "for cause" and only with the approval of two-thirds of our stockholders;

- require super-majority voting to amend some provisions in our restated certificate of incorporation and restated bylaws;

- authorize the issuance of "blank check" preferred stock that our board could use to implement a stockholder rights plan, also known as a "poison pill";

- eliminate the ability of our stockholders to call special meetings of stockholders;

- prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders;

- prohibit cumulative voting; and

- establish advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at annual stockholder meetings.

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

Any of these provisions of our charter documents or Delaware law could, under certain circumstances, depress the market price of our common stock. See the section entitled "Description of Capital Stock."

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus, including the sections entitled "Prospectus Summary," "Risk Factors," "Use of Proceeds," "Management's Discussion and Analysis of Financial Condition and Results of Operations," and "Business" contains forward-looking statements. Forward-looking statements include all statements that are not historical facts and can be identified by the words "believe," "may," "will," "potentially," "estimate," "continue," "anticipate," "intend," "could," "would," "project," "plan," "expect," and similar expressions that convey uncertainty of future events or outcomes.

These forward-looking statements are subject to a number of risks, uncertainties, and assumptions, including those described in "Risk Factors" and elsewhere in this prospectus. Moreover, we operate in a very competitive and rapidly changing environment, and new risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties, and assumptions, the forward-looking events and circumstances discussed in this prospectus may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

You should not rely upon forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur. We undertake no obligation to update publicly any forward-looking statements for any reason after the date of this prospectus to conform these statements to actual results or to changes in our expectations, except as required by law.

You should read this prospectus, and the documents that we reference in this prospectus and have filed with the SEC as exhibits to the registration statement of which this prospectus is a part, with the understanding that our actual future results, levels of activity, performance and events and circumstances may be materially different from what we expect.

INDUSTRY AND MARKET DATA

Unless otherwise indicated, information contained in this prospectus concerning our industry and the markets in which we operate is based on information from various sources, including independent industry publications. In presenting this information, we have also made assumptions based on such data and other similar sources, and on our knowledge of, and our experience to date in, the potential markets for our product candidates. The industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of factors, including those described in the section entitled "Risk Factors." These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us.

USE OF PROCEEDS

We estimate that the net proceeds from our sale of 5,000,000 shares of our common stock in this offering at an assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses, will be approximately $66.1 million. If the underwriters' option to purchase additional shares is exercised in full, we estimate that we will receive additional net proceeds of $10.5 million.

A $1.00 increase (decrease) in the assumed initial public offering price would increase (decrease) the net proceeds to us from this offering by $4.7 million, assuming the number of shares offered by us remains the same and after deducting the estimated underwriting discounts and commissions. Similarly, each increase (decrease) of one million shares in the number of shares offered would increase (decrease) the net proceeds to us from this offering by approximately $14.0 million, assuming that the assumed initial public offering price remains the same and after deducting the estimated underwriting discounts and commissions.

We currently intend to use the net proceeds from this offering for the following purposes:

- approximately $18.0 to $20.0 million to advance AT132 for the treatment of XLMTM through preliminary results from a Phase 1/2 clinical trial expected in the fourth quarter of 2017;
- approximately $13.0 to $15.0 million to advance AT342 for the treatment of Crigler-Najjar through preliminary results from a Phase 1/2 clinical trial expected in the fourth quarter of 2017;
- approximately $3.0 to $5.0 million to advance preclinical development of AT982 for the treatment of Pompe disease through preliminary results from a Phase 1/2 clinical trial expected in the second half of 2017;
- approximately $3.0 to $5.0 million to advance preclinical development of AT307 for the treatment of CASQ2-CPVT through submission of an IND or CTA in 2017;
- approximately $7.0 to $10.0 million to operate and expand our internal manufacturing facility; and
- the remainder for working capital and other general corporate purposes, which will include funding for the hiring of additional personnel, capital expenditures and the costs of operating as a public company.

We estimate that our current cash, cash equivalents and investments, along with the net proceeds from this offering, will be sufficient for us to fund our operating expenses and capital expenditure requirements through 2018.

The expected use of the net proceeds from this offering represents our intentions based upon our current plans and business conditions. As of the date of this prospectus, we cannot predict with any certainty all of the particular uses for the net proceeds or the amounts that we will actually spend on the uses set forth above. We may use a portion of the net proceeds for the acquisition of, or investment in, technologies, intellectual property or businesses that complement our business, although we have no present commitments or agreements to this effect.

The amounts and timing of our future expenditures and the extent of product candidate development may vary significantly depending on numerous factors, including the status, results and timing of our current preclinical studies and clinical trials we may commence in the future, product approval process with the FDA and other regulatory agencies, our current collaborations and any new collaborations we may enter into with third parties and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering.

The expected net proceeds of this offering will not be sufficient for us to fund any of our product candidates through regulatory approval, and we will need to raise substantial additional capital to complete the development and commercialization of our product candidates.

Pending their use as described above, we intend to invest the net proceeds from this offering in marketable securities that may include investment-grade interest-bearing securities, money market accounts, certificates of deposit, commercial paper and guaranteed obligations of the U.S. government.

DIVIDEND POLICY

We have never declared or paid cash dividends on our capital stock. We do not expect to pay dividends on our common stock for the foreseeable future. Instead, we anticipate that all of our earnings, if any, will be used for the operation and growth of our business. Any future determination to declare cash dividends would be subject to the discretion of our board of directors and would depend upon various factors, including our results of operations, financial condition and capital requirements, restrictions that may be imposed by applicable law and our contracts and other factors deemed relevant by our board of directors.

CAPITALIZATION

The following table sets forth our cash, cash equivalents and investments and capitalization as of March 31, 2016 on:

- an actual basis;
- a pro forma basis to reflect (i) the automatic conversion of shares of our convertible preferred stock outstanding as of March 31, 2016 into 13,820,301 shares of our common stock effective immediately prior to the completion of this offering and (ii) the effectiveness of our restated certificate of incorporation as of immediately prior to the completion of this offering; and
- a pro forma as adjusted basis to reflect (i) the pro forma adjustments set forth above and (ii) the sale and issuance of 5,000,000 shares of our common stock in this offering, at an assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses.

The pro forma as adjusted information set forth in the table below is illustrative only and will be adjusted based on the actual initial public offering price and other terms of this offering determined at pricing.

You should read this table together with the section entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements and related notes included elsewhere in this prospectus.

	As of March 31, 2016		
	Actual	Pro Forma	Pro Forma As Adjusted[1]
	(in thousands, except share and per share amounts) (unaudited)		
Cash, cash equivalents and investments	$ 80,272	$ 80,272	$ 146,322
Stockholders' equity:			
Convertible preferred stock, $0.00001 par value: 30,855,031 shares authorized, 13,820,301 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma or pro forma as adjusted	$ 135,750	$ —	$ —
Preferred stock, $0.00001 par value: no shares authorized, issued and outstanding, actual; 10,000,000 shares authorized, no shares issued or outstanding, pro forma and pro forma as adjusted	—	—	—
Common stock, $0.00001 par value: 50,000,000 shares authorized, 2,200,077 shares issued and outstanding, actual; 300,000,000 shares authorized, 16,020,378 shares issued and outstanding, pro forma; 21,020,378 shares issued and outstanding, pro forma as adjusted	—	—	—
Additional paid-in capital	7,095	142,845	208,895
Accumulated deficit	(51,207)	(51,207)	(51,207)
Accumulated other comprehensive income	4	4	4
Total stockholders' equity	91,642	91,642	157,692
Total capitalization	$ 91,642	$ 91,642	$ 157,692

(1) A $1.00 increase (decrease) in the assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, would increase

(decrease) each of cash, cash equivalents and investments, additional paid-in capital, total stockholders' equity and total capitalization by approximately $4.7 million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting the estimated underwriting discounts and commissions. Similarly, each increase (decrease) of one million shares in the number of shares offered would increase (decrease), cash, cash equivalents and investments, additional paid-in capital, total stockholders' equity and total capitalization by approximately $14.0 million, assuming the assumed initial public offering price remains the same and after deducting the estimated underwriting discounts and commissions.

The table above excludes the following shares:

- 2,279,086 shares of our common stock issuable upon the exercise of outstanding options as of March 31, 2016, with a weighted-average exercise price of approximately $3.99 per share;
- 82,739 shares of our common stock issuable upon the exercise of outstanding options granted after March 31, 2016, with an exercise price of $7.54 per share;
- 101,127 shares of our common stock issuable upon the exercise of options that we expect to grant on the date of this prospectus, with an exercise price equal to the initial public offering price of our common stock; and
- 2,208,646 shares of common stock reserved for future issuance under our stock-based compensation plans as of March 31, 2016, consisting of (i) 599,773 shares of common stock reserved for future issuance under our 2012 Equity Incentive Plan as of March 31, 2016 (consisting of 682,512 shares reserved as of March 31, 2016, reduced by 82,739 shares underlying stock options granted after March 31, 2016), (ii) 1,398,873 shares of common stock reserved for future issuance under our 2016 Equity Incentive Plan (consisting of 1,500,000 shares, reduced by 101,127 shares underlying stock options that we expect to grant on the date of this prospectus), which will become effective on the date immediately prior to the date of this prospectus and (iii) 210,000 shares of common stock reserved for future issuance under our 2016 Employee Stock Purchase Plan, which will become effective on the date of this prospectus.

DILUTION

If you invest in our common stock, your ownership interest will be diluted to the extent of the difference between the initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock immediately after this offering.

As of March 31, 2016, our pro forma net tangible book value was approximately $77.1 million, or $4.81 per share of common stock. Pro forma net tangible book value per share represents the amount of our tangible assets less our liabilities divided by the total number of shares of our common stock outstanding as of March 31, 2016, after giving effect to the automatic conversion of shares of our convertible preferred stock outstanding as of March 31, 2016 into 13,820,301 shares of our common stock effective immediately prior to the completion of this offering.

After giving effect to (i) the pro forma adjustment set forth above and (ii) the sale and issuance of 5,000,000 shares of our common stock at an assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses, our pro forma net tangible book value as of March 31, 2016 would have been approximately $143.1 million, or $6.81 per share of our common stock. This represents an immediate increase in pro forma net tangible book value of $2.00 per share to our existing stockholders and an immediate dilution of $8.19 per share to investors purchasing shares in this offering, as follows:

Assumed initial public offering price per share		$15.00
Pro forma net tangible book value per share as of March 31, 2016	$4.81	
Increase in pro forma net tangible book value per share attributable to new investors in this offering	2.00	
Pro forma as adjusted net tangible book value per share after this offering		6.81
Dilution in net tangible book value per share to investors in this offering		$ 8.19

A $1.00 increase (decrease) in the assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, would increase (decrease) our pro forma as adjusted net tangible book value per share after this offering by $0.22, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting the estimated underwriting discounts and commissions. Similarly, each increase (decrease) of one million shares in the number of shares offered would increase (decrease) the dilution to new investors by $0.32 per share or $(0.36) per share, respectively, assuming the assumed initial public offering price remains the same and after deducting the estimated underwriting discounts and commissions.

If the underwriters exercise their option to purchase additional shares in full, our pro forma as adjusted net tangible book value per share after this offering would be $7.05 per share, and the dilution in pro forma net tangible book value per share to new investors in this offering would be $7.95 per share.

The following table summarizes, on a pro forma as adjusted basis as of March 31, 2016, the differences between the number of shares of common stock purchased from us, the total cash consideration and the average price per share paid to us by existing stockholders and by new investors purchasing shares in this offering, at an assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, before deducting estimated underwriting discounts and commissions and estimated offering expenses:

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders	16,020,378	76.2%	$141,291,176	65.3%	$ 8.82
New public investors	5,000,000	23.8	75,000,000	34.7	$15.00
Total	21,020,378	100%	$216,291,176	100%	

A $1.00 increase (decrease) in the assumed initial public offering price of $15.00 per share of our common stock, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus, would increase (decrease) the total consideration paid by new investors by $5.0 million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and before deducting the estimated underwriting discounts and commissions. Similarly, each increase (decrease) of one million shares in the number of shares offered would increase (decrease) the total consideration paid by new investors by $15.0 million, assuming the assumed initial public offering price remains the same and before deducting the estimated underwriting discounts and commissions and estimated offering expenses.

If the underwriters exercise their option to purchase additional shares in full, the number of shares of common stock held by existing stockholders will be reduced to 73.6% of the total number of shares of common stock to be outstanding after this offering, and the number of shares of common stock held by investors participating in this offering will be further increased to 26.4% of the total number of shares of common stock to be outstanding after this offering.

Our existing institutional investors associated with our board have indicated an interest in purchasing shares of common stock in this offering with an aggregate value of approximately $30.0 million at the initial public offering price. As these indications of interest are non-binding, the foregoing discussion and table do not reflect the potential purchase of any shares in this offering by these parties.

The number of shares of our common stock to be outstanding after this offering excludes:

- 2,279,086 shares of our common stock issuable upon the exercise of outstanding options as of March 31, 2016, with a weighted-average exercise price of approximately $3.99 per share;
- 82,739 shares of our common stock issuable upon the exercise of outstanding options granted after March 31, 2016, with an exercise price of $7.54 per share;
- 101,127 shares of our common stock issuable upon the exercise of options that we expect to grant on the date of this prospectus, with an exercise price equal to the initial public offering price of our common stock; and
- 2,208,646 shares of common stock reserved for future issuance under our stock-based compensation plans as of March 31, 2016, consisting of (i) 599,773 shares of common stock reserved for future issuance under our 2012 Equity Incentive Plan as of March 31, 2016 (consisting of 682,512 shares reserved as of March 31, 2016, reduced by 82,739 shares underlying stock options granted after March 31, 2016), (ii) 1,398,873 shares of common stock reserved for future issuance under our 2016 Equity Incentive Plan (consisting of 1,500,000 shares, reduced by 101,127 shares underlying stock options that we expect to grant on the date of this prospectus), which will become effective on the date immediately prior to the date of this prospectus and (iii) 210,000 shares of common stock reserved for future issuance under our 2016 Employee Stock Purchase Plan, which will become effective on the date of this prospectus.

SELECTED CONSOLIDATED FINANCIAL DATA

We derived our selected consolidated statements of operations data for the years ended December 31, 2014 and 2015 and the consolidated balance sheet data as of December 31, 2014 and 2015 from our audited consolidated financial statements included elsewhere in this prospectus. We derived our selected consolidated statements of operations data for the three months ended March 31, 2015 and 2016 and our consolidated balance sheet data as of March 31, 2016 from our unaudited interim condensed consolidated financial statements included elsewhere in this prospectus. The unaudited interim condensed consolidated financial statements were prepared on a basis consistent with our audited financial statements and include, in management's opinion, all adjustments, consisting only of normal recurring adjustments that we consider necessary for a fair presentation of the financial information set forth in those statements. Our historical results are not necessarily indicative of the results that may be expected in any future period and the results for the three months ended March 31, 2016 are not necessarily indicative of results to be expected for the full year ending December 31, 2016 or any other period.

The selected consolidated financial data below should be read in conjunction with the section entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements and related notes included elsewhere in this prospectus.

	Year Ended December 31,		Three Months Ended March 31,	
	2014	2015	2015	2016
	(in thousands, except share and per share amounts)		(unaudited)	
Consolidated Statements of Operations Data:				
Operating expenses:				
Research and development	$ 9,280	$ 20,235	$ 3,080	$ 7,906
General and administrative	1,670	6,491	1,083	2,632
Total operating expenses	10,950	26,726	4,163	10,538
Loss from operations	(10,950)	(26,726)	(4,163)	(10,538)
Interest income	6	245	61	97
Other income (expense), net	125	23	47	(23)
Net loss	$ (10,819)	$ (26,458)	$ (4,055)	$ (10,464)
Net loss per share, basic and diluted[(1)]	$ (21.56)	$ (23.03)	$ (6.63)	$ (4.85)
Shares used in computing net loss per share, basic and diluted[(1)]	501,707	1,148,827	612,039	2,159,081
Pro forma net loss per share, basic and diluted (unaudited)[(1)]		$ (2.28)		$ (0.65)
Shares used in computing pro forma net loss per share, basic and diluted (unaudited)[(1)]		11,621,249		15,979,382

(1) See Notes 2 and 13 to our audited consolidated financial statements and Note 8 to our unaudited interim condensed consolidated financial statements included elsewhere in this prospectus for an explanation of the calculations of our basic and diluted net loss per share, basic and diluted pro forma net loss per share and the shares used in computing basic and diluted net loss per share and basic and diluted pro forma net loss per share.

	December 31,		March 31,
	2014	2015	2016
		(in thousands)	(unaudited)
Consolidated Balance Sheet Data:			
Cash, cash equivalents and investments	$ 62,148	$ 95,227	$ 80,272
Working capital	57,830	91,916	76,150
Total assets	63,009	117,469	110,555
Convertible preferred stock	72,403	135,750	135,750
Accumulated deficit	(14,285)	(40,743)	(51,207)
Total stockholders' equity	59,864	101,689	91,642

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with the section entitled "Selected Consolidated Financial Data" and our consolidated financial statements and related notes included elsewhere in this prospectus. This discussion and other parts of this prospectus contain forward-looking statements that involve risks and uncertainties, such as our plans, objectives, expectations, intentions and beliefs. Our actual results could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below and those discussed in the section entitled "Risk Factors" included elsewhere in this prospectus.

Overview

We are a biotechnology company focused on developing and commercializing gene therapy products for patients suffering from serious, life-threatening rare diseases caused by single gene defects. We believe that gene therapy has powerful potential to treat these diseases through delivery of a functional copy of the affected gene to cells, resulting in production of the normal protein. Our vision is to become a fully integrated biotechnology company. In pursuit of this goal, we are executing on our core strategic initiatives, which include the development of proprietary in-house manufacturing capabilities and the expansion of our pipeline. We have assembled a world-class team with expertise in gene therapy, rare disease drug development and commercialization, and biologics manufacturing.

We have built a compelling portfolio of product candidates, including AT132 for the treatment of X-Linked Myotubular Myopathy, or XLMTM, AT342 for the treatment of Crigler-Najjar Syndrome, or Crigler-Najjar, AT982 for the treatment of Pompe disease and AT307 for the treatment of the CASQ2 subtype of Catecholaminergic Polymorphic Ventricular Tachycardia, or CASQ2-CPVT. We plan to submit Investigational New Drug applications, or INDs, or Clinical Trial Authorisations, or CTAs, for AT982 in the third quarter of 2016, for AT342 in the fourth quarter of 2016 and for AT132 in the first quarter of 2017, and expect to have preliminary data from all three programs in the second half of 2017. Given the available clinical and regulatory pathways, we believe that the rarity and severity of the diseases we target may provide advantages for drug development, including the potential for expedited development and regulatory review, and market exclusivity. We maintain full global rights to all of our product candidates.

We have built our portfolio of product candidates in part by engaging in strategic transactions with third parties. In July 2013, we entered into a license agreement with REGENXBIO Inc., or REGENXBIO, pursuant to which we obtained intellectual property rights related to AT132 and AT982. In January 2014, we entered into a collaborative development agreement with Genethon, pursuant to which we acquired intellectual property rights related to AT132 in exchange for granting Genethon the exclusive right to manufacture materials for preclinical and early clinical development, subject to Genethon's ability to supply required quantities in accordance with applicable timelines, and the funding for certain research and development activities related to AT132. In July 2015, we entered into a license with the University of Florida Research Foundation, or UFRF, pursuant to which we obtained intellectual property rights related to AT982. In August 2015, in connection with our acquisition of Cardiogen Sciences, Inc., or Cardiogen, we acquired a license agreement with Fondazione Salvatore Maugeri, or FSM, pursuant to which we obtained a license to FSM's intellectual property rights related to AT307 and certain other products that we may develop related to the treatment of several additional inherited arrhythmias. In November 2015, we entered into two additional license agreements with REGENXBIO, pursuant to which we obtained intellectual property rights related to AT307 and AT342. In May 2016, we entered into a license and collaboration agreement with the The Trustees of the University of Pennsylvania, or the University of Pennsylvania, pursuant to which we obtained a license to develop and commercialize a gene therapy product for Crigler-Najjar.

Upon execution of the license and collaboration agreement with the University of Pennsylvania, we met the conditions of a contractual milestone under our Crigler-Najjar license agreement with REGENXBIO, and made the required payment of $0.4 million to REGENXBIO. We also paid the University of Pennsylvania an upfront fee of $0.5 million, as well as $3.0 million for certain preclinical development activities. We may be required to make additional milestone payments and pay royalties and other amounts to third parties pursuant to our license and collaboration agreements as we further develop and commercialize our product candidates.

Since our inception, we have devoted substantially all of our resources to: identifying, acquiring, and developing our product candidate portfolio; organizing and staffing our company; raising capital; developing our manufacturing capabilities; and providing general and administrative support for these operations. We have never generated revenue and have incurred significant net losses since inception. We do not expect to receive any revenue from any product candidates that we develop until we obtain regulatory approval and commercialize our product candidates or enter into collaborative agreements with third parties. Our net losses were $10.8 million and $26.5 million for the years ended December 31, 2014 and 2015, respectively, and $4.1 million and $10.5 million for the three months ended March 31, 2015 and 2016, respectively. As of March 31, 2016, we had an accumulated deficit of $51.2 million. We expect to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially as we:

- invest significantly to further develop and seek regulatory approval for our existing product candidates;
- further expand our pipeline of potential product candidates;
- continue to develop our proprietary in-house manufacturing facility and capabilities;
- hire additional clinical, scientific, management and administrative personnel;
- seek regulatory and marketing approvals for any product candidates that we may develop;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any drugs for which we may obtain marketing approval;
- maintain, expand and protect our intellectual property portfolio;
- acquire or in-license other assets and technologies; and
- add additional operational, financial and management information systems and processes to support our ongoing development efforts, any future manufacturing or commercialization efforts and our transition to operating as a public company

We have funded our operations to date primarily from the issuance and sale of our convertible preferred stock. As of March 31, 2016, we had cash, cash equivalents and investments of $80.3 million.

To fund our current operating plans, we will need additional capital, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements. The amount and timing of our future funding requirements will depend on many factors, including the pace and results of our preclinical and clinical development efforts. We cannot assure you that we will ever be profitable or generate positive cash flow from operating activities.

Financial Operations Overview

Research and Development Expenses

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

- expenses incurred under agreements with consultants, third-party contract organizations and investigative clinical trial sites that conduct research and development activities on our behalf;
- laboratory and vendor expenses related to the execution of preclinical studies and clinical trials;
- costs related to production of preclinical and clinical materials, including fees paid to contract manufacturers; and
- costs related to in-licensing of rights to develop and commercialize our product candidate portfolio.

Internal costs are associated with activities performed by our research and development organization and generally benefit multiple programs. These costs are not separately allocated by product candidate. Unallocated, internal research and development costs consist primarily of:

- personnel costs, which include salaries, benefits and stock-based compensation expense;
- facilities and other expenses, which include expenses for rent and maintenance of facilities, depreciation and amortization expense;
- lab supplies and equipment used for internal research and development activities; and
- the change in fair value of contingent consideration payable.

We expense all research and development costs in the periods in which they are incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors, collaborators and third-party service providers. Nonrefundable advance payments for goods or services to be received in future periods for use in research and development activities are deferred and capitalized. The capitalized amounts are then expensed as the related goods are delivered and as services are performed.

The largest component of our operating expenses has historically been our investment in research and development activities. However, we do not allocate internal research and development costs, such as salaries, benefits, stock-based compensation expense and indirect costs to product candidates on a program-specific basis.

The following table summarizes our research and development expenses incurred during the respective periods:

	Years Ended December 31,		Three Months Ended March 31,	
	2014	2015	2015	2016
	(in thousands)		(unaudited)	
AT132 external program costs	$ 4,802	$ 5,909	$ 1,238	$ 1,571
AT982 external program costs	700	2,162	253	593
AT342 external program costs	—	600	—	548
AT307 external program costs	—	1,805	—	177
Other external program costs	—	—	—	201
Internal research and development costs	3,778	9,759	1,589	4,816
Total research and development expenses	$ 9,280	$20,235	$ 3,080	$ 7,906

We expect our research and development expenses to increase substantially for the foreseeable future as we continue to invest in research and development activities related to developing our product candidates, including investments in manufacturing, as our programs advance into later stages of development and as we begin to conduct clinical trials. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of our product candidates is highly uncertain. As a result, we are unable to determine the duration and completion costs of our research and development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel costs, facilities costs, including rent and maintenance of facilities, depreciation and amortization expense and other expenses for outside professional services, including legal, human resources, audit and accounting services. Personnel costs consist of salaries, benefits and stock-based compensation expense. In addition, we incurred acquisition costs, which were primarily legal and accounting fees, in connection with our acquisition of Cardiogen in 2015. There was no comparable expense in 2014 or the three months ended March 31, 2016. We expect our general and administrative expenses to increase for the foreseeable future due to anticipated increases in headcount to advance our product candidates and as a result of operating as a public company, including expenses related to compliance with the rules and regulations of the Securities and Exchange Commission, The NASDAQ Global Market, additional insurance expense, investor relations activities and other administration and professional services.

Interest Income

Interest income consists of interest earned on our cash, cash equivalents and investments.

Other Income (Expense), net

Other income (expense), net consists primarily of foreign currency transaction gains and losses incurred during the period.

Critical Accounting Polices and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with United States generally accepted accounting principles, or U.S. GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenue generated and expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

Business Combinations

We allocate the purchase price of acquired businesses to the tangible and intangible assets acquired and liabilities assumed based upon their estimated fair values on the acquisition date. The purchase price allocation process requires us to make estimates and assumptions, notably at the acquisition date with respect to intangible assets and in-process research and development.

Contingent Consideration Payable

We determine the fair value of contingent consideration payable on the acquisition date using a probability-based income approach utilizing an appropriate discount rate. Each reporting period thereafter, we revalue these obligations and record increases or decreases in their fair value as adjustments to research and development expense. Changes in the fair value of contingent consideration payable can result from adjustments to the estimated probability and assumed timing of achieving the underlying milestones, as well as from changes to estimated discount rates.

Accrued Research and Development Costs

We record accrued expenses for estimated costs of our research and development activities conducted by third-party service providers, which include the conduct of research, preclinical studies, regulatory consulting, clinical trials and contract manufacturing activities. We record the estimated costs of research and development activities based upon the estimated amount of services provided but not yet invoiced, and include these costs in accrued liabilities in the balance sheet and within research and development expense in the statement of operations and comprehensive loss. These costs are a significant component of our research and development expenses. We record accrued expenses for these costs based on the estimated amount of work completed and in accordance with agreements established with various third parties.

We estimate the amount of work completed through discussions with internal personnel and external service providers as to the progress or stage of completion of the services and the agreed-upon fees to be paid for such services. We make significant judgments and estimates in determining the accrued balance in each reporting period. As actual costs become known, we adjust our accrued estimates. Our accrued expenses are dependent, in part, upon the receipt of timely and accurate reporting from clinical research organizations and other third-party service providers. To date, there have been no material differences from our accrued expenses to actual expenses.

Stock-Based Compensation Expense

We recognize compensation costs related to stock-based awards granted to employees, including stock options, based on the estimated fair value of the awards on the date of grant, net of estimated forfeitures. We estimate the grant date fair value, and the resulting stock-based compensation expense, using the Black-Scholes option-pricing model. The grant date fair value of the stock-based awards is generally recognized on a straight-line basis over the requisite service period, which is generally the vesting period of the respective awards.

We account for stock-based compensation arrangements with non-employees using a fair value approach. The fair value of these options is measured using the Black-Scholes option pricing model reflecting the same assumptions as applied to employee options in each of the reported periods, other than the expected life, which is assumed to be the remaining contractual life of the option. The fair value of the unvested options under these arrangements is subject to remeasurement over the vesting terms as earned. Expense is recognized over the vesting period which is generally the same as the service period.

The Black-Scholes option-pricing model requires the use of highly subjective assumptions to determine the fair value of stock-based awards. These assumptions include:

- *Expected Term*—Our expected term represents the period that our stock-based awards are expected to be outstanding and is determined using the simplified method (based on the mid-point between the vesting date and the end of the contractual term for employee options and based on the contractual term for non-employee options).

- *Expected Volatility*—Since we are privately held and do not have any trading history for our common stock, the expected volatility was estimated based on the average volatility for comparable

publicly traded biotechnology companies over a period equal to the expected term of the stock option grants. The comparable companies were chosen based on their similar size, stage in the life cycle, or area of specialty.

- *Risk-Free Interest Rate*—The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of option.

- *Expected Dividend*—We have never paid dividends on our common stock and have no plans to pay dividends on our common stock. Therefore, we used an expected dividend yield of zero.

In addition to the Black-Scholes assumptions, we estimate our forfeiture rate based on an analysis of our actual forfeitures and will continue to evaluate the adequacy of the forfeiture rate based on actual forfeiture experience, analysis of employee turnover behavior and other factors. The impact from any forfeiture rate adjustment would be recognized in full in the period of adjustment and if the actual number of future forfeitures materially differs from our estimates, we will be required to record adjustments to stock-based compensation expense in future periods.

For the years ended December 31, 2014 and 2015, stock-based compensation expense was $0.5 million and $1.3 million respectively. For the three months ended March 31, 2015 and 2016, stock-based compensation expense was $0.1 million and $0.4 million, respectively. As of March 31, 2016, we had $4.8 million of total unrecognized stock-based compensation costs, net of estimated forfeitures, which we expect to recognize over a weighted-average period of 3.16 years.

Historically, for all periods prior to this initial public offering, the fair value of the shares of common stock underlying our share-based awards were estimated on each grant date by our board of directors. In order to determine the fair value of our common stock underlying option grants, our board of directors considered, among other things, contemporaneous valuations of our common stock prepared by an unrelated third-party valuation firm in accordance with the guidance provide by the American Institute of Certified Public Accountants Practice Guide, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*. Given the absence of a public trading market for our common stock, our board of directors exercised their judgment and considered a number of objective and subjective factors to determine the best estimate of the fair value of our common stock, including our stage of development; progress of our research and development efforts; the rights, preferences and privileges of our preferred stock relative to those of our common stock; equity market conditions affecting comparable public companies; the lack of marketability of our common stock; and valuations obtained from sales of our preferred stock to unrelated parties.

For valuations after the completion of this offering, our board of directors will determine the fair value of each share of underlying common stock based on the closing price of our common stock as reported on the date of grant.

The intrinsic value of all outstanding options as of March 31, 2016 was $25.0 million based on the estimated fair value of our common stock of $15.00 per share, the midpoint of the estimated offering price range set forth on the cover page of this prospectus.

Income Taxes

We use the asset and liability method of accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on the differences between the financial reporting and the tax bases of assets and liabilities and are measured using the enacted tax rates and laws that will be in effect when the differences are expected to reverse. We assess the likelihood that the resulting deferred tax assets will be realized. A valuation allowance is provided when it is more likely than not that some portion or all of a deferred tax asset will not be realized.

As of December 31, 2015, our total gross deferred tax assets were $19.4 million. Due to our lack of earnings history and uncertainties surrounding our ability to generate future taxable income, the net deferred tax assets have been fully offset by a valuation allowance. The deferred tax assets were primarily comprised of federal and state tax net operating loss and tax credit carryforwards.

Utilization of the net operating loss carryforwards may be subject to a substantial annual limitation due to ownership changes that may have occurred or that could occur in the future, as required by Section 382 of the Internal Revenue Code of 1986, as amended, or the Code, and similar state provisions. These ownership change limitations may limit the amount of net operating loss carryforwards and other tax attributes that can be utilized annually to offset future taxable income and tax, respectively. In general, an "ownership change" as defined by Section 382 of the Code results from a transaction or series of transactions over a three-year period resulting in an ownership change of more than 50 percentage points (by value) of the outstanding stock of a company by certain stockholders. Since our formation, we have raised capital through the issuance of capital stock on several occasions, which separately or combined with the purchasing stockholders' subsequent disposition of those shares, may have resulted in such ownership changes, or could result in ownership changes in the future.

We have not completed an analysis to assess whether an ownership change has occurred. If we have experienced an ownership change as defined in the Code at any time since our formation, utilization of our net operating loss carryforwards would be subject to an annual limitation under Section 382 of the Code. Any limitation may result in expiration of a portion of the net operating loss carryforwards before utilization. Further, until a study is completed and any limitation known, no amounts are being considered as an uncertain tax position or disclosed as an unrecognized tax benefit. Due to the existence of the valuation allowance, future changes in our unrecognized tax benefits will not impact our effective tax rate. Any carryforwards that will expire prior to utilization as a result of such limitations will be removed from deferred tax assets, with a corresponding reduction of the valuation allowance.

Results of Operations

Comparison of the three months ended March 31, 2015 and 2016

	Three Months Ended March 31,		
	2015	2016	Change
		(in thousands)	
Operating expenses:			
Research and development	$ 3,080	$ 7,906	$ 4,826
General and administrative.	1,083	2,632	1,549
Total operating expenses	4,163	10,538	6,375
Loss from operations	(4,163)	(10,538)	(6,375)
Interest income	61	97	36
Other income (expense), net	47	(23)	(70)
Net loss	$(4,055)	$(10,464)	$(6,409)

Research and Development

Research and development expenses increased by $4.8 million, or 157%, from $3.1 million for the three months ended March 31, 2015 to $7.9 million for the three months ended March 31, 2016. The increase was primarily due to a $1.4 million increase in personnel costs, a $0.7 million increase in lab supplies and consulting expenses for internal research activities, a $0.5 million increase in facilities costs as we expanded our research and development headcount and internal operations and a $0.1 million increase in stock-based compensation expense. There was also a $0.7 million increase in expenses for our AT132 and AT982 programs related to preclinical studies, increased manufacturing of study materials and increased consulting and initiation costs in

preparation for future anticipated clinical trials. In addition, during the three months ended March 31, 2016, we incurred $0.7 million of expenses associated with the launch of the AT342 and AT307 programs late in 2015 and $0.2 million of expenses in connection with other external research and development, with no comparable expenses in the same period in 2015.

General and Administrative

General and administrative expenses increased by $1.5 million, or 143%, from $1.1 million for the three months ended March 31, 2015 to $2.6 million for the three months ended March 31, 2016. The increase was primarily due to a $0.6 million increase in personnel and consulting costs due to increased headcount, a $0.2 million increase in facilities costs, a $0.4 million increase in legal and accounting fees to support general corporate, in-licensing and patent-related activities and a $0.2 million increase in stock-based compensation expense.

Interest Income

Interest income increased by $36,000, from $61,000 for the three months ended March 31, 2015 to $97,000 for the three months ended March 31, 2016, as we invested the funds we received from our Series C preferred stock financing into short duration fixed-income securities.

Other Income (Expense), Net

Other income (expense), net changed from income of $47,000 for the three months ended March 31, 2015 to a charge of $23,000 for the three months ended March 31, 2016. The change was primarily due to a reduction in foreign currency gains resulting from Euro-based invoices settled in U.S. dollars.

Comparison of the years ended December 31, 2014 and 2015

	Year ended December 31,		
	2014	2015	Change
		(in thousands)	
Operating expenses:			
Research and development	$ 9,280	$ 20,235	$ 10,955
General and administrative.	1,670	6,491	4,821
Total operating expenses	10,950	26,726	15,776
Loss from operations	(10,950)	(26,726)	(15,776)
Interest income	6	245	239
Other income (expense), net	125	23	(102)
Net loss	$(10,819)	$(26,458)	$(15,639)

Research and Development

Research and development expenses increased by $11.0 million, or 118%, from $9.3 million for the year ended December 31, 2014 to $20.2 million for the year ended December 31, 2015. The increase was primarily due to a $3.8 million increase in personnel costs and a $0.9 million increase in facilities costs due to an increase in our research and development headcount. In addition, there was a $2.6 million increase in expenses related to our AT132 and AT982 programs, as we conducted additional preclinical studies, increased manufacturing of study materials and incurred consulting and initiation costs in preparation for future clinical trials. In addition, we launched the AT342 and AT307 programs in 2015, incurring total costs of $2.4 million in 2015, with no comparable costs in 2014. There was also an increase of $1.2 million for lab supplies and $0.6 million for other expenses related to expanded research and development activities in support of preclinical activities and manufacturing process development. In addition, the increase in the fair value of the acquisition contingent consideration payable was $0.1 million, with no comparable expense in 2014.

General and Administrative

General and administrative expenses increased by $4.8 million, or 282%, from $1.7 million for the year ended December 31, 2014 to $6.5 million for the year ended December 31, 2015. The increase was primarily due to a $2.7 million increase in personnel and consulting costs, a $0.5 million increase in facilities costs due to increased headcount and a $1.0 million increase for legal fees in support of general corporate, in-licensing and patent related activities. Additionally, we incurred $0.4 million in transaction costs associated with the acquisition of Cardiogen in 2015, with no comparable expense in 2014.

Interest Income

Interest income increased by $239,000 from $6,000 for the year ended December 31, 2014 to $245,000 for the year ended December 31, 2015 as we invested the funds we received from our preferred stock financings into short duration fixed-income securities.

Other Income, Net

Other income, net decreased by $102,000 from $125,000 for the year ended December 31, 2014 to $23,000 for the year ended December 31, 2015. The decrease was due to a reduction in foreign currency gains resulting from Euro-based invoices settled in U.S. dollars.

Liquidity, Capital Resources and Plan of Operations

Since our inception in 2012 through March 31, 2016, our operations have been financed solely by net proceeds of $135.8 million from the sale of shares of our convertible preferred stock. As of March 31, 2016, we had $80.3 million in cash, cash equivalents and investments and an accumulated deficit of $51.2 million.

Our primary use of cash is to fund operating expenses, which consist of research and development expenditures and general and administrative expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

We believe that our existing cash, cash equivalents and investments will be sufficient to meet our anticipated cash and capital expenditure requirements for at least the next 12 months. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. We will continue to require additional financing to advance our current product candidates through clinical development, to develop, acquire or in-license other potential product candidates and to fund operations for the foreseeable future. We will continue to seek funds through equity or debt financings, collaborative or other arrangements with corporate sources, or through other sources of financing. Adequate additional funding may not be available to us on acceptable terms, or at all. Any failure to raise capital as and when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies.

Further, our operating plans may change, and we may need additional funds to meet operational needs and capital requirements for clinical trials and other research and development activities. We currently have no credit facility or committed sources of capital. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated product development programs.

If we need to raise additional capital to fund our operations, funding may not be available to us on acceptable terms, or at all. If we are unable to obtain adequate financing when needed, we may have to delay,

reduce the scope of or suspend one or more of our clinical trials, research and development programs or commercialization efforts. We may seek to raise any necessary additional capital through a combination of public or private equity offerings, debt financings, and collaborations or licensing arrangements. If we do raise additional capital through public or private equity offerings, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. If we raise additional capital through debt financing, we may be subject to covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If additional funding is required, there can be no assurance that additional funds will be available to us on acceptable terms on a timely basis, if at all. If we are unable to raise capital, we will need to curtail planned activities to reduce costs. Doing so will likely have an unfavorable effect on our ability to execute our business plans.

The following table summarizes our cash flows for the periods indicated:

	Years Ended December 31,		Three Months Ended March 31,	
	2014	2015	2015	2016
	(in thousands)			
Cash used in operating activities	$ (8,069)	$(27,515)	$ (4,849)	$(12,849)
Cash (used in) provided by investing activities	(16,664)	(8,876)	(24,158)	428
Cash provided by financing activities	57,386	62,850	—	76
Net increase (decrease) in cash and cash equivalents	$ 32,653	$ 26,459	$(29,007)	$(12,345)

Cash Flows from Operating Activities

Cash used in operating activities for the three months ended March 31, 2016 was $12.8 million. Our net loss was $10.5 million, which included noncash charges of $0.7 million, consisting primarily of $0.3 million of stock-based compensation expense and $0.2 million of depreciation and amortization expense. In addition, our prepaid expenses and other current assets increased by $1.4 million, our accounts payable and accrued liabilities decreased by $2.8 million and our facility lease obligations increased by $1.0 million, which resulted in a net use of cash.

Cash used in operating activities for the three months ended March 31, 2015 was $4.8 million. Our net loss was $4.1 million, which included noncash charges of $0.2 million, consisting primarily of $0.1 million of stock-based compensation expense. In addition, our prepaid expenses and other current assets increased by $0.3 million and our accounts payable and accrued liabilities decreased by $0.6 million, which resulted in a use of cash.

Cash used in operating activities for the year ended December 31, 2015 was $27.5 million. Our net loss was $26.5 million, which included noncash charges of $2.4 million, consisting primarily of $1.3 million of stock-based compensation expense and $0.8 million of depreciation and amortization expense. In addition, our prepaid expenses and other current assets increased by $7.0 million primarily due to prepayments for our research and development activities, which was partially offset by a net reduction in our use of cash due to a $3.3 million increase in our accounts payable and accrued liabilities and a $0.3 million increase in facility lease obligations.

Cash used in operating activities for the year ended December 31, 2014 was $8.1 million. Our net loss was $10.8 million, which included noncash charges of $0.5 million, primarily for stock-based compensation expense. In addition, our prepaid expenses and other current assets increased by $0.3 million and our accounts payable and accrued liabilities increased by $2.5 million, which resulted in a net reduction in our use of cash.

Cash Flows from Investing Activities

Cash provided by investing activities was $0.4 million for the three months ended March 31, 2016, primarily from the maturity or sale of marketable securities of $17.2 million, partially offset by the purchases of property and equipment of $2.1 million and the purchases of marketable securities of $14.7 million.

Cash used for investing activities was $24.1 million for the three months ended March 31, 2015, primarily for the purchase of marketable securities of $26.1 million, partially offset by the maturity or sales of marketable securities of $2.0 million.

Cash used for investing activities was $8.9 million for the year ended December 31, 2015 and was related to the purchase of marketable securities for $40.1 million and purchases of property and equipment for $1.7 million, partially offset by the maturity or sale of marketable securities of $32.8 million and cash received in the acquisition of Cardiogen of $0.1 million.

Cash used for investing activities for the year ended December 31, 2014 was $16.7 million related to the purchase of marketable securities for $16.5 million and purchases of property and equipment for $0.1 million.

Cash Flows from Financing Activities

Cash provided by financing activities for the three months ended March 31, 2016 was related to proceeds from the exercise of stock options of $0.1 million. There were no financing activities in the three months ended March 31, 2015.

Cash provided by financing activities for the year ended December 31, 2015 was related to net proceeds from the issuance of convertible preferred stock of $62.8 million and proceeds from the exercise of stock options of $0.1 million.

Cash provided by financing activities for the year ended December 31, 2014 was primarily related to net proceeds from the issuance of convertible preferred stock of $57.4 million.

Contractual Obligations and Other Commitments

The following table summarizes our contractual obligations as of December 31, 2015:

	Payments Due by Period						
	Less than 1 year	2 years	3 years	4 years	5 years	More than 5 years	Total
	(in thousands)						
Operating lease obligations:	$1,603	$1,794	$1,643	$1,691	$1,740	$2,591	$11,062
Total contractual obligations	$1,603	$1,794	$1,643	$1,691	$1,740	$2,591	$11,062

Lease Agreements

Beginning in January 2015, we entered into a series of amendments to our prior Janssen facility lease to increase the office and lab space, which resulted in a quarterly lease expense of approximately $0.1 million and an increase in the aggregate security deposit to approximately $0.1 million. The Janssen facility lease was terminated in June 2016. In July 2015, we entered into a sub-lease agreement for approximately 22,000 square feet of manufacturing space in South San Francisco for an initial term that expires in May 2017 with total minimum lease payments due of $0.9 million. In November 2015, we purchased an option to enter into a ten-year lease for the existing 22,000 square feet plus approximately 17,000 additional square feet of manufacturing space, which we exercised in May 2016; the ten-year lease will become effective in June 2017.

In September 2015, we entered into a lease agreement for approximately 22,000 square feet of office space in San Francisco, which serves as our headquarters location. The initial term commenced in February 2016 and expires in June 2022 with total payments due of $10.2 million.

Cardiogen Acquisition

In August 2015, we acquired Cardiogen, a biotechnology company focused on the discovery and development of AAV gene therapy products for rare, inherited arrhythmogenic diseases. As consideration for the acquisition, we issued 1,293,058 shares of common stock and 46,969 shares of Series B preferred stock. Additionally, upon the first dosing of a patient in a human clinical study involving AT307, we are obligated to pay to former Cardiogen stockholders $4.2 million in common stock plus an additional $5.8 million in either cash or common stock, at our election. We have not included this potential contingent payment obligation in the table above as the timing and likelihood of such payment is uncertain.

License and Collaboration Agreements

Under various license agreements, we will be required to make milestone payments and pay royalties and other amounts to third parties. Under the 2013 license agreement with REGENXBIO related to AT132 and AT982, we are required to pay REGENXBIO (i) an annual maintenance fee; (ii) up to $8.85 million in combined milestone fees per licensed product related to XLMTM and up to $8.85 million in combined milestone fees per licensed product related to Pompe disease, a small portion of which may be paid in the form of shares of our common stock; (iii) mid to high single digit royalty percentages on net sales of licensed products and (iv) mid-single digit to low twenties royalty percentages of any sublicense fees we receive from sublicensees for the licensed patent rights.

Under the 2015 license agreement with REGENXBIO regarding intellectual property rights related to AT307, we are required to pay REGENXBIO (i) an annual maintenance fee for each covered indication; (ii) up to $8.8 million in combined development and regulatory milestone fees for each indication and each licensed product; (iii) up to $45.0 million in combined commercial milestone fees based on various annual aggregate net sales thresholds; (iv) mid-single digit to low teens royalty percentages on net sales of licensed products sold by us, our affiliates and sublicensees and (v) a low twenties percentage of any sublicense fees we receive from sublicensees for the licensed products and certain fees we receive from the sale or transfer of specified rights related to a licensed product.

Under the 2015 license agreement with REGENXBIO regarding intellectual property rights related to AT342, we are required to pay REGENXBIO (i) an annual maintenance fee; (ii) up to $7.6 million in combined development and regulatory milestone fees per licensed product; (iii) mid-single digit to low teens royalty percentages on net sales of licensed products sold by us, our affiliates and sublicensees and (iv) a low twenties percentage of certain sublicense fees we receive from sublicensees for the licensed products and certain fees we receive from the sale or transfer of specified rights related to a licensed product.

Under the 2015 license agreement with UFRF, we are required to pay UFRF (i) an annual maintenance fee; (ii) up to $1.2 million in combined development and regulatory milestone payments; (iii) low-single digit royalty percentages on net sales of AT982 and certain other product candidates that we may develop in the future related to Pompe disease, subject to minimum annual royalty payments of up to $0.2 million per year following the first commercial sale; and (iv) certain percentages of sublicense fees we receive from sublicensees of the licensed patent rights.

Under the license agreement with FSM that we acquired in connection with the 2015 Cardiogen acquisition, we are required to pay FSM low-single digit royalties on net sales of AT307 and certain other product candidates that we may develop in the future related to the treatment of CASQ2-CPVT and several additional inherited arrhythmias.

Under a 2014 collaborative development agreement with Genethon, we are also committed to reimbursing Genethon for mutually agreed manufacturing costs and research and development activities related to AT132. We have not included these potential payment obligations in the table above as the amount and timing of such payments are not known.

Under a 2016 license and collaboration agreement with the University of Pennsylvania, we are obligated to pay the University of Pennsylvania (i) up to an aggregate of $6.0 million for preclinical development activities agreed upon by both parties, subject to adjustment based on the work plan, which amount includes the $3.0 million already paid in May 2016, (ii) up to an aggregate of $13.7 million in development, regulatory and net sales milestone payments for the first licensed product; (iii) low to mid-single digit royalty percentages on tiered annual net sales of the licensed products and (iv) mid-single digit to low double-digit percentages of any sublicense fees we receive from third parties for the grant of sublicenses to any licensed patent rights.

As of March 31, 2016, we had not developed a commercial product using the licensed technologies and no milestones had been achieved under these agreements, except for a $0.4 million payment to REGENXBIO that was due upon our entry into the license and collaboration agreement with the University of Pennsylvania. We have not included any contingent payment obligations, such as milestones or royalties, in the table above as the amount, timing and likelihood of such payments are not known.

For further information about our license and collaboration agreements, see the section entitled "Business—License and Collaboration Agreements."

Other Contracts

We also enter into contracts in the normal course of business with various third parties for preclinical research studies, clinical trials, testing and other services. These contracts generally provide for termination upon notice, and therefore we believe that our noncancelable obligations under these agreements are not material.

Internal Control over Financial Reporting

During the audit of our financial statements for the years ended December 31, 2014 and 2015, a material weakness was identified in our internal control over financial reporting. Under standards established by the Public Company Accounting Oversight Board, a material weakness is a deficiency or combination of deficiencies in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected and corrected on a timely basis. The material weakness that was identified related to a lack of sufficient accounting resources and personnel that limits our ability to adequately segregate duties, establish defined accounting policies and procedures and perform timely reviews of account reconciliations.

We have implemented and are continuing to implement measures designed to improve our internal control over financial reporting to address the underlying causes of this material weakness, including the hiring of our Chief Financial Officer and other accounting personnel and establishing new accounting and financial reporting procedures, policies and processes to have in place an appropriate level of internal control over financial reporting. We, and our independent registered public accounting firm, were not required to perform an evaluation of our internal control over financial reporting as of December 31, 2015 in accordance with the provisions of the Sarbanes-Oxley Act. Accordingly, we cannot assure you that we have identified all, or that we will not in the future have additional, material weaknesses. Material weaknesses may still exist when we report on the effectiveness of our internal control over financial reporting as required by reporting requirements under Section 404 of the Sarbanes-Oxley Act after the completion of this offering.

Off-Balance Sheet Arrangements

We have not entered into any off-balance sheet arrangements and do not have any holdings in variable interest entities.

Quantitative and Qualitative Disclosures about Market Risk

We are exposed to market risks in the ordinary course of our business. These risks primarily include interest rate sensitivities. We had cash, cash equivalents and investments of $95.2 million and $80.3 million as of December 31, 2015 and March 31, 2016, respectively, which consist of bank deposits, money market funds, and marketable securities. Such interest-earning instruments carry a degree of interest rate risk; however, historical fluctuations in interest income have not been significant for us. We had no debt outstanding as of December 31, 2015 and March 31, 2016.

JOBS Act Accounting Election

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards, and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Recent Accounting Pronouncements

In August 2014, the FASB issued ASU 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*, or ASU 2014-15, which requires management to evaluate whether there is substantial doubt that we are able to continue operating as a going concern within one year after the date the financial statements are issued or available to be issued. If there is substantial doubt, additional disclosure is required, including the principal condition or event that raised the substantial doubt, our evaluation of the condition or event in relation to our ability to meet our obligations and our plan to alleviate (or, which is intended to alleviate) the substantial doubt. ASU 2014-15 is effective for interim and annual reporting periods beginning after December 15, 2016. Early adoption is permitted. We are currently assessing what impact, if any, the adoption of this ASU will have on our consolidated financial statements and related disclosure.

In January 2016, the FASB issued ASU No. 2016-01, *Financial Instruments - Overall* (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities, or ASU 2016-01. ASU 2016-01 addresses certain aspects of recognition, measurement, presentation and disclosure of financial instruments. ASU 20196-01 is effective for fiscal years, and interim periods within those years, beginning after December 15, 2017, which for us is January 1, 2018. We are currently assessing what impact, if any, the adoption of this ASU will have on our consolidated financial statements and related disclosure.

In February 2016, the FASB issued ASU No. 2016-02, *Leases* (Topic 842), or ASU 2016-02. Under the new guidance, (with the exception of short-term leases) at the commencement date, lessees will be required to recognize a lease liability and a right-of-use asset. Lessor accounting is largely unchanged, while lessees will no longer be provided with a source of off-balance sheet financing. Public business entities should apply the amendments in ASU 2016-02 for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years (January 1, 2019, for us). Early application is permitted. Lessees (for capital and operating leases) must apply a modified retrospective transition approach for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements. The modified retrospective approach would not require any transition accounting for leases that expired before the earliest comparative period presented. We are currently evaluating the impact that the standard will have on our consolidated financial statements and related disclosure.

In March 2016, the FASB Issued ASU No. 2016-09, *Compensation-Stock Compensation: Improvements to Employee Share-Based Payment Accounting*, or ASU 2016-09. The updated guidance changes how companies account for certain aspects of share-based payment awards to employees, including the accounting for income taxes, forfeitures, and statutory tax withholding requirements, as well as classification in the statement of cash flows. The update to the standard is effective for fiscal years and interim periods within those years beginning after December 15, 2016, with early application permitted. We are currently assessing what impact, if any, the adoption of this ASU will have on our consolidated financial statements and related disclosure.

BUSINESS

"Audentes" from the Latin verb *audeo*: Those who have courage; those who have boldness, daring.

Courageous Patients. Bold Effort.

Overview

We are a biotechnology company focused on developing and commercializing gene therapy products for patients suffering from serious, life-threatening rare diseases caused by single gene defects. We believe that gene therapy has powerful potential to treat these diseases through delivery of a functional copy of the affected gene to cells, resulting in production of the normal protein. We have built a compelling portfolio of product candidates, including AT132 for the treatment of X-Linked Myotubular Myopathy, or XLMTM, AT342 for the treatment of Crigler-Najjar Syndrome, or Crigler-Najjar, AT982 for the treatment of Pompe disease and AT307 for the treatment of the CASQ2 subtype of Catecholaminergic Polymorphic Ventricular Tachycardia, or CASQ2-CPVT. We plan to submit Investigational New Drug applications, or INDs, or Clinical Trial Authorisations, or CTAs, for AT982 in the third quarter of 2016, for AT342 in the fourth quarter of 2016 and for AT132 in the first quarter of 2017, and expect to have preliminary data from all three programs in the second half of 2017. We maintain full global rights to all of our product candidates.

Our vision is to become a fully integrated biotechnology company. In pursuit of this goal, we are executing on our core strategic initiatives, which include the development of proprietary in-house manufacturing capabilities and the expansion of our pipeline. We have assembled a world-class team with expertise in gene therapy, rare disease drug development and commercialization, and biologics manufacturing.

Our mission is to dramatically and positively transform the lives of patients suffering from serious, life-threatening rare diseases with limited or no treatment options. For example, we are developing AT132 to treat XLMTM, a disease for which there are no approved therapies and from which approximately 50% of affected children die in the first 18 months of life. We believe our product candidates have the potential to provide long-lasting benefits, changing the lives of patients with these devastating diseases. Given the available clinical and regulatory pathways, we believe that the rarity and severity of the diseases we target may provide advantages for drug development, including the potential for expedited development and regulatory review, and market exclusivity.

We focus on the treatment of rare diseases caused by single gene, or monogenic, defects in DNA that we believe can be effectively addressed using gene therapy. Conventional approaches such as protein therapeutics attempt to replace the deficient protein, but they do not correct the underlying genetic defect causing the disease. In addition, protein therapeutics often require frequent administration by injection or infusion and often result in sub-optimal safety and efficacy. We believe gene therapy is an ideal treatment modality for diseases caused by monogenic defects. Our portfolio of product candidates employs the use of adeno-associated virus, or AAV, a small, non-pathogenic virus that is genetically engineered to function as a delivery vehicle, or vector, and is administered to a patient to introduce a healthy copy of a mutated gene to the body. AAV gene therapy vectors are modified such that they will not cause an infection like a normal virus, but are capable of delivering therapeutic genes into patients' cells. Vectors derived from AAV have a well-established safety profile in humans and have been shown to effectively deliver genes to the liver, eye, muscle and brain. Preclinical and clinical data demonstrate that AAV vectors are capable of providing durable efficacy with a favorable adverse event profile due at least in part to AAV's low immunogenic potential. AAV vectors can be described by the serotype, or strain, of the original virus isolate that was used to form the outer shell, or capsid, of the vector. We selected AAV8 and AAV9 as our in-licensed vector capsid serotypes, based on their biological properties, which we believe will translate into positive clinical effect in our target indications. For example, we believe AAV8 is advantageous for the treatment of Crigler-Najjar given its ability to penetrate the liver, the primary organ implicated in this disease pathology.

Our business model is to develop and commercialize a broad portfolio of gene therapy product candidates to treat rare diseases. We use a focused set of criteria to select product candidates that we believe have the best chance of success. These criteria include:

- serious, life-threatening rare diseases;
- monogenic diseases with well-understood biology;
- disease characteristics well-suited for treatment with AAV gene therapy technology;
- high potential for meaningful clinical benefit;
- compelling preclinical data;
- clear measures for evaluation in clinical trials; and
- opportunities for expedited development through established regulatory pathways.

We have built a portfolio of gene therapy product candidates and we intend to further expand our portfolio over time. Set forth below is a table summarizing our development programs.

Product Candidate *(Indication)*	Stage of Development				Planned IND/ CTA Submission	Preliminary Data Expected	Commercial Rights
	Discovery	Lead Optimization	IND-Enabling	Phase 1/2			
AT132 *(XLMTM)*					Q1 2017	Q4 2017	AUDENTES THERAPEUTICS
AT342 *(Crigler-Najjar)*					Q4 2016	Q4 2017	AUDENTES THERAPEUTICS
AT982 *(Pompe Disease)*					Q3 2016	H2 2017	AUDENTES THERAPEUTICS
AT307 *(CASQ2-CPVT)*					2017		AUDENTES THERAPEUTICS

AT132. XLMTM is characterized by extreme muscle weakness, respiratory failure and early death with an estimated 50% mortality rate in the first 18 months of life. The disease is the result of mutations in the MTM1 gene that affect the production of myotubularin, an enzyme required for normal development and function of skeletal muscle. The incidence of XLMTM is estimated to be one in 50,000 male births. Currently, only supportive treatment options, such as ventilator use or a feeding tube, are available. We are developing AT132, an AAV8 vector containing a functional copy of the MTM1 gene, for the treatment of XLMTM. We believe AT132 may provide patients with significantly improved outcomes based on the ability of AAV8 to preferentially treat skeletal muscle. Preclinical study results in both canine and murine models of the disease demonstrated dramatic improvements in all outcomes, including histology, muscle strength, respiratory function and survival. Our goal is to achieve these same benefits in XLMTM patients following a single intravenous administration of AT132.

AT342. Crigler-Najjar is a rare, congenital autosomal recessive monogenic disease characterized by severely high levels of bilirubin in the blood and risk of irreversible neurological damage and death. Average life expectancy is reported as being 30 years of age with phototherapy. Crigler-Najjar is estimated to affect approximately one in 1,000,000 newborns. Infants with Crigler-Najjar develop severe jaundice shortly after birth

resulting in rapid presentation and diagnosis. Crigler-Najjar is caused by mutations in the gene encoding the UGT1A1 (uridine-diphosphate (UDP)-glucuronosyltransferase (UGT) 1A1) enzyme resulting in an inability to convert unconjugated bilirubin to a water-soluble form that can be excreted from the body. Clinical diagnosis is confirmed via genetic testing of the UGT1A1 gene. The current standard of care for Crigler-Najjar is aggressive management of high bilirubin levels with persistent, daily phototherapy, usually for longer than 12 hours per day using intense fluorescent light focused on the bare skin, while the eyes are shielded. Phototherapy speeds bilirubin decomposition and excretion, lowering serum bilirubin levels. Phototherapy wanes in effectiveness beginning around age four due to thickening of the skin and a reduction in surface area to body mass ratio, and a liver transplant may be required for survival.

We are developing AT342, an AAV8 vector containing a functional version of the UGT1A1 gene. Preclinical data in murine models of the disease demonstrate AAV8-UGT1A1 significantly reduces bilirubin levels, even at UGT1A1 liver expression levels of just five to eight percent of normal. We are advancing AT342 with the goal of administering a single dose that results in a significant, durable reduction in serum bilirubin, a reduction in or elimination of lengthy daily phototherapy, and elimination of the need for a liver transplant. We believe that serum bilirubin levels will be a clinically relevant endpoint and that determination of efficacy of AT342 will be straightforward due to the ease and reliability of measurement.

AT982. Pompe disease is a serious, progressive genetic disease characterized by severe muscle weakness, respiratory failure leading to ventilator dependence and, in infants, increased cardiac mass and heart failure. In untreated infants, the disease is often fatal due to cardio-respiratory failure within the first year of life. Pompe disease is caused by mutations in the gene encoding the lysosomal enzyme alpha-glucosidase, or GAA, which results in a deficiency of GAA protein and leads to the accumulation of glycogen. The incidence of Pompe disease is approximately one in 40,000 births. The only approved treatment for Pompe disease is enzyme replacement therapy, or ERT, which is a chronic treatment delivered in bi-weekly intravenous infusions. Despite the availability of ERT, significant medical need still persists, which is primarily due to the inability of ERT to penetrate key tissues affected by the disease and immunogenicity of ERT treatment. We believe our approach with AT982, which uses an AAV serotype 9 capsid vector containing a functional copy of the GAA gene, can overcome the limitations of ERT and provide long-term improvement in patient symptoms. Further, we believe AT982 may provide patients with superior outcomes based on the ability of AAV9 to penetrate key cells and tissues affected by the disease, such as motoneurons, which are not effectively treated with ERT. Preclinical data in a murine model achieved statistically significant improvements in weight gain, ventilation parameters, glycogen deposition and cardiac left ventricle mass. We believe intracellular production of the therapeutic protein may improve efficacy, reduce immunogenicity and deliver a durable therapeutic effect with a single intravenous administration.

AT307. CASQ2-CPVT is a rare monogenic disease that is characterized by life-threatening arrhythmias that may lead to sudden cardiac death. There are currently only limited treatment options with variable efficacy for patients suffering from CPVT, including beta-blockers and a sodium channel blocker. The autosomal recessive form of the disease is caused by mutations in the calsequestrin 2 gene, or CASQ2 gene, and is characterized by stress-induced heartbeat rhythm changes in an otherwise structurally normal heart. It is estimated that CPVT occurs in one in 10,000 people, with approximately 2% to 5% due to mutations in the CASQ2 gene. This equates to an approximate prevalence of 6,000 affected people in North America, Europe and other addressable markets. Despite treatment with anti-arrhythmia therapies, sympathectomy and implantable cardiac defibrillators, a significant portion of the patients remain symptomatic. We are developing AT307, an AAV9 vector containing a functional version of the CASQ2 gene. We believe AT307 may provide patients with improved outcomes based on the ability of AAV9 to preferentially treat cardiac muscle. Preclinical data in murine models of the disease demonstrated an ability to prevent ventricular tachycardia through restoration of CASQ2 protein expression. We are advancing AT307 with the goal of providing a single administration of AT307 that results in a significant reduction in life-threatening arrhythmic events and a major improvement in quality of life.

Although we believe our product candidates have the potential to provide long-term improvement in patient symptoms with a single administration, we will need to complete additional preclinical studies and clinical trials to determine the safety and efficacy of our product candidates. The results of these future studies and trials may be different than the results of our earlier studies and trials. We have not received regulatory approval for any of our product candidates, and in order to obtain regulatory approval and commercialize our product candidates, the U.S. Food and Drug Administration, or FDA, or foreign regulatory agencies will need to determine that our product candidates are safe and effective. To date, no gene therapy products have been approved in the United States and two have been approved in Europe.

We believe that our proprietary manufacturing capabilities provide a core strategic advantage. We lease a manufacturing facility in South San Francisco that has been used for commercial manufacture of biologic drug products in the past, and have improved the facility to support our desired research, process development and manufacturing capabilities in accordance with current Good Manufacturing Practices, or cGMP, requirements. We plan to initiate cGMP manufacturing of our products in our facility in the second half of 2016. We have made and will continue to make significant investments to further optimize our manufacturing capabilities to cost-effectively produce high-quality AAV vectors at both clinical and commercial scale. We believe that our manufacturing processes, methods, expertise and facilities will give us a comprehensive manufacturing platform for production of our AAV product candidates at commercial scale.

We have a focused, passionate team with collective expertise in gene therapy, rare disease drug development and commercialization, and biologics manufacturing. Matthew Patterson, our President, Chief Executive Officer and Co-Founder, is a biotechnology leader with over 20 years of experience at Genzyme Corporation, BioMarin Pharmaceutical, Amicus Therapeutics and our company. We are backed by a group of leading life science institutional investors, including 5AM Ventures, Cormorant Asset Management LLC, Cowen Private Investments, Deerfield Management Company, Foresite Capital, OrbiMed, RA Capital Management, Redmile Group, Rock Springs Capital Management LP, Sofinnova Ventures, Venrock and Versant Ventures.

Our Strategy

Our strategy is to leverage the expertise of our team and the transformative potential of gene therapy technology to develop treatments that improve outcomes for patients with serious, life-threatening rare diseases. Key elements of our strategy are:

- ***Constantly focus on serving patients.*** We take pride in our efforts to harness the transformative potential of gene therapy to improve the lives of patients suffering from devastating rare diseases. We intend to continue to engage with patient advocacy groups to better understand the burden of disease and align our efforts with the needs of patients and caregivers.

- ***Advance our four lead product candidates through clinical development.*** We plan to submit INDs or CTAs for our product candidates as follows: AT982 for the treatment of Pompe disease in the third quarter of 2016, AT342 for the treatment of Crigler-Najjar in the fourth quarter of 2016, AT132 for the treatment of XLMTM in the first quarter of 2017 and AT307 for the treatment of CASQ2-CPVT in 2017.

- ***Continue to expand our pipeline with additional gene therapy product candidates targeting serious, life-threatening rare diseases.*** We intend to continue leveraging our expertise and focused selection criteria to expand our pipeline of product candidates. Our relationships with leading academic institutions and other rare disease companies are an important component of our strategy for sourcing additional product candidates.

- ***Continue to build our proprietary manufacturing capabilities and invest in a state-of-the-art cGMP facility.*** We believe the quality, reliability and scalability of our gene therapy manufacturing approach will be a core competitive advantage crucial to our long-term success. We intend to be capable of internal cGMP manufacturing in the second half of 2016.

Our Strengths

We believe our leadership position is based on our following strengths:

- ***Rare disease expertise.*** Led by a management team with over 100 years of combined experience in rare diseases, we are building a fully integrated and industry-leading biotechnology company. Leveraging recent developments in gene therapy, we aim to provide durable and meaningful treatment options to patients suffering from rare monogenic diseases.

- ***Highly focused selection criteria for development programs.*** We employ a disciplined approach to select and expand our pipeline of product candidates. We believe the application of our selection criteria enables the efficient, cost-effective and successful development of our product candidates.

- ***Promising product candidate pipeline.*** On the basis of rigorous preclinical investigation, we are preparing to advance our four lead product candidates into the clinic: AT132 for the treatment of XLMTM, AT342 for the treatment of Crigler-Najjar, AT982 for the treatment of Pompe disease and AT307 for the treatment of CASQ2-CPVT.

- ***Proprietary know-how and capabilities.*** Our proprietary manufacturing capabilities provide a major core strategic advantage, including better control over the cost and timelines of developing our product candidates, superior protection of novel inventions and intellectual property, and expanded possibilities for new programs and partnerships.

- ***Broad network.*** We believe our strong relationships with key opinion leaders and patient advocacy groups will support our product development efforts and our potential for future commercial success. Leveraging our collaborations with these parties allows us to better understand the diseases we target and optimize our research, clinical development and commercial plans.

Gene Therapy Background

Genes are composed of sequences of deoxyribonucleic acid, or DNA, which code for proteins that perform a broad range of physiologic functions within all living organisms. DNA is a large, highly charged molecule that is difficult to transport across a cell membrane and deliver to the nucleus, where it can be transcribed and translated into protein.

Gene therapy is a therapeutic approach to treating genetic diseases caused by mutations in DNA. For gene therapy to work, an isolated gene sequence or segment of DNA needs to be delivered efficiently to the desired target tissues and cell types. The treatment involves the administration of a functional gene to produce normal protein within a patient's cells, offering the potential for durable therapeutic benefit. To achieve these goals, scientists have designed and developed a variety of viral vectors to facilitate gene delivery in cells.

Our Approach

The AAV gene therapy vectors we utilize are capable of transducing a wide range of tissues with generally little or no toxicity and only mild immune response. Functionally, AAV packages a single-stranded linear DNA genome that can be engineered to contain a therapeutic gene in place of all the virus genes. AAV vectors have a well-established safety profile and do not naturally propagate by themselves in the absence of another viral infection, reducing the likelihood of inappropriate viral spread following administration. As a result, AAV vectors are emerging as the preferred delivery vehicle for gene therapy.

Our vector design strategy includes careful selection of the vector capsid (the outer protein shell) and sophisticated engineering of the vector genome (the therapeutic DNA expression cassette) to target the correct tissues and improve the potential to provide patients with meaningful and durable outcomes. Optimal selection of capsids can reduce immune responses that attenuate the function of AAV vectors, and enable more robust

trafficking to the specific tissues we care about for each disease. The vector genome is composed of multiple structural elements, including the gene coding sequence and the promoter, which drives expression of the gene. We use the latest available technology to engineer the vector genome to direct the target cells to make the desired protein at the appropriate level necessary to achieve a therapeutic effect for the longest period possible. We believe the product candidates we have created offer distinct advantages for our indications due to their selectivity for target tissue types and focused expression of the desired protein.

Our Product Candidate and Target Indication Selection Criteria

Our business model is to develop and commercialize a broad portfolio of gene therapy product candidates to treat rare diseases. We use a focused set of criteria to select product candidates that we believe have the best chance of success. These criteria include:

- ***Serious, life-threatening rare diseases with high unmet medical need.*** We target orphan indications where there are limitations with existing therapeutic options or no such options exist, particularly with an opportunity to bring products with high value to patients and their caregivers.
- ***Monogenic diseases with well-understood biology.*** Gene therapy is particularly effective when applied to replace a single gene producing a single protein, the function of which is well understood.
- ***High potential for meaningful clinical benefit.*** We focus on diseases with the potential to demonstrate a meaningful therapeutic effect with only moderate levels of expression of the deficient protein.
- ***Well suited for AAV gene therapy.*** We select target indications and product candidates where we believe AAV technology can be used effectively.
- ***Compelling preclinical data.*** We look for product candidates that have positive results from preclinical studies in animal models of disease that provide increased confidence in the potential for positive human results.
- ***Clear measures for evaluation in clinical trials.*** We prioritize diseases that we believe have the potential for straightforward clinical endpoints to demonstrate efficacy.
- ***Opportunities for expedited development through established regulatory pathways.*** We believe our product candidates may be eligible for expedited regulatory review, including Breakthrough Therapy and Fast Track designations.

Our AAV Product Candidates

AT132 for the Treatment of X-Linked Myotubular Myopathy

Overview of XLMTM

XLMTM is a rare, severe, congenital muscle disease with an estimated incidence of one in 50,000 male births. The disease is caused by mutations in the MTM1 gene, which encodes a protein called myotubularin. Myotubularin is an enzyme involved in the development, maturation, maintenance and function of skeletal muscle cells. Mutations in the MTM1 gene result in production of too little or no functional protein. Importantly, we believe that even a modest increase of functional protein may have a significant therapeutic benefit for XLMTM patients.

Infants with XLMTM are typically born with severe muscle weakness and the majority require chronic mechanical ventilation from birth. Approximately 50% of patients die in the first 18 months of life. There is no approved treatment for XLMTM and disease management is primarily supportive. Of the patients that survive the

infantile period, most are severely incapacitated and do not have a life expectancy beyond early adolescence. Diagnosis of XLMTM is generally based on recognition of clinical symptoms at birth, typically followed by muscle biopsy and confirmation with genetic testing. Like many rare diseases, we believe XLMTM is under diagnosed and that approval of treatment would increase disease awareness, screening and diagnosis.

AT132 Description

AT132 is an AAV8 vector that delivers an MTM1 gene expression cassette containing a desmin promoter, which is a regulatory element that drives gene transcription in muscle tissue. The MTM1 cassette is capable of increasing myotubularin expression in targeted tissues. AT132 was designed with these elements because AAV8 is known to effectively penetrate skeletal muscle and the desmin promoter is primarily active in muscle. We believe AT132 has the potential to provide long-term clinical benefit to XLMTM patients through persistent expression of the functional protein following a single intravenous administration.

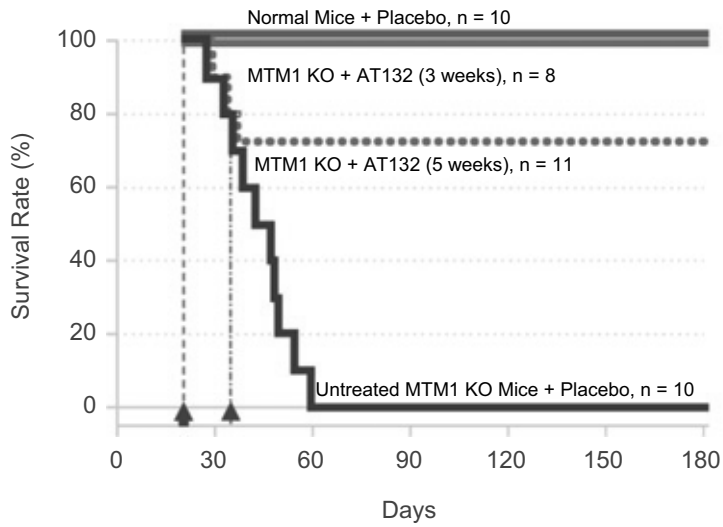
Preclinical Proof-of-Concept for AT132

We have two robust animal models of XLMTM, a murine model consisting of mice engineered to knock out the functional MTM1 gene, or MTM1 KO mice, and a naturally occurring canine model. Preclinical studies in these models have used an AT132 construct engineered to include the species-specific MTM1 transgene. Both models present with disease symptoms similar to that of humans including severe muscle weakness, respiratory failure and early death. We believe that in this indication the canine model, as with many large animal models, is particularly valuable given similarities to humans with XLMTM in size, weight and physiology.

Murine Model

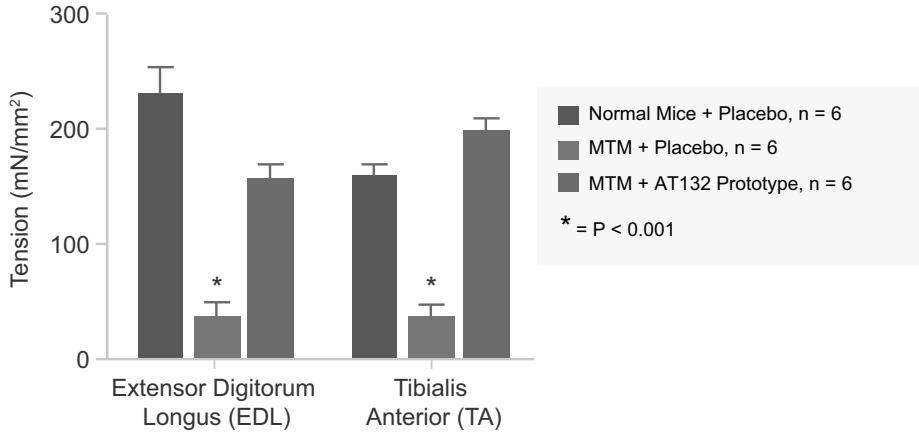
In the murine model, symptom onset occurs at approximately two to three weeks of age, and there is rapid progression of the disease leading to death at approximately seven to eight weeks of age. Through multiple studies in this murine model, treatment with AT132 has been shown to significantly improve disease symptoms when compared to untreated controls. Specifically, the administration of a single intravenous dose of AT132 (3×10^{14} vg/kg, or vector genomes per kilogram) to eight mice at three weeks of age resulted in improved muscle strength, nearly normal growth and long-term survival in MTM1 KO mice. In order to evaluate the potential benefit of treatment of mice at a later stage of disease, the same dose was administered to 11 severely affected MTM1 KO mice at five weeks of age, when 20% of the animals in the treatment group had already died, and a robust effect on survival was again observed. The figure below summarizes the effects of AT132 on survival.

AT132 Improves Survival in MTM1 Knockout Mice



In an additional study in the murine model, muscle strength was evaluated. MTM1 KO mice were treated by intramuscular injection of an AT132 prototype. As a control, normal mice were treated with a placebo. Contractile strength of the muscles in the extensor digitorum longus, or EDL, and tibialis anterior, or TA, muscles were measured four weeks post dose. The effects of the AT132 prototype are shown in the figure below.

AT132-Prototype Restores Muscle Contractility in MTM1 Knockout Mice



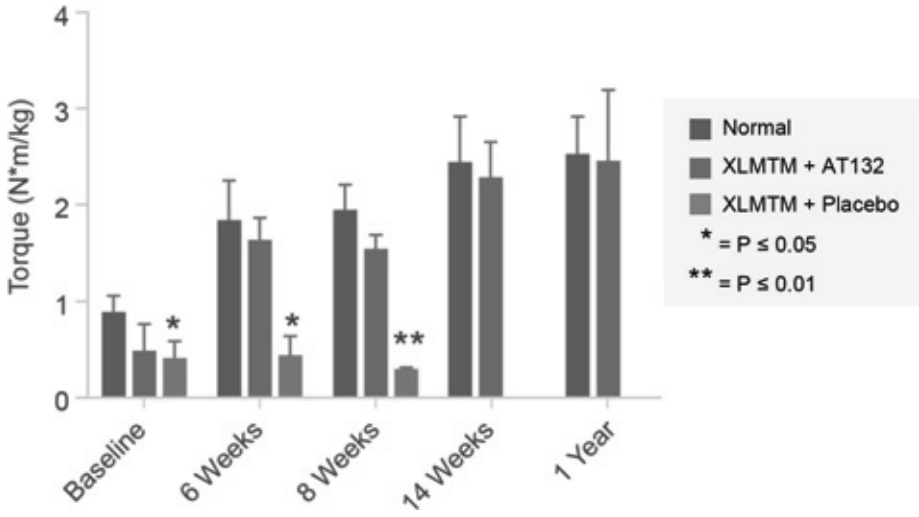
Statistical significance is important and when used herein is denoted by p-values. The p-value is the probability that the reported result was achieved purely by chance (for example, a p-value < 0.001 means that there is a less than 0.1% chance that the observed change was purely due to chance). Generally, a p-value less than 0.05 is considered to be statistically significant.

Canine Model

In the naturally occurring Labrador Retriever model, symptom onset occurs at nine to ten weeks of age, and disease progression leads to death at approximately 18 weeks of age. Multiple studies in this model have demonstrated that a single administration of AT132 significantly improves all disease symptoms and survival rates. In two dogs treated in one of the earliest studies, these effects have lasted over three and a half years to date and the dogs continue to thrive.

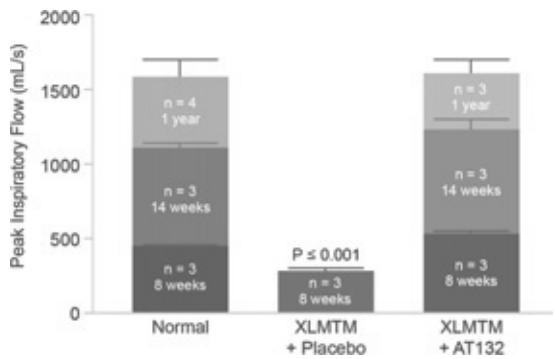
The first canine study was designed as a proof-of-concept to determine whether AT132 could improve muscle strength and organ function in comparison to an XLMTM dog treated with a placebo. Administration of a single dose of AT132 (2.5 x 10^{14} vg/kg) to three dogs at nine weeks of age resulted in maintenance of muscle strength, respiratory function and survival comparable to normal dogs. The evaluation of the muscle strength of these dogs is illustrated in the figure below. The XLMTM dog dosed with placebo died before the 14-week measurement.

AT132 Improves Muscle Strength in XLMTM Dogs



The evaluation of respiratory function as measured by the fastest flow rate measured during inhalation, or peak inspiratory flow, is illustrated in the figure below.

AT132 Improves Respiratory Function in XLMTM Dogs



Most importantly, all treated dogs achieved a statistically significant improvement in survival, which extended far beyond the critical 18-week time point, when all untreated XLMTM dogs could no longer ambulate. All three of the treated dogs survived for the one-year duration of the study. One of these dogs was euthanized for study purposes, but the other two are now approximately three and a half years of age and remain indistinguishable from normal dogs.

The second canine study was designed to compare the effects of three different doses of AT132 delivered by systemic administration versus untreated XLMTM and normal dogs. The three doses, a low dose (5.0 x 10^{13} vg/kg), a mid-dose (2.5 x 10^{14} vg/kg) and a high dose (8.0 x 10^{14} vg/kg), were administered to

XLMTM dogs at ten weeks of age and the dogs were evaluated for 45 weeks. Three dogs were treated at each dose. In this study, the low dose was deemed to be the minimally effective dose, meaning that it produced somewhat extended survival and some improvement in functional parameters, including muscle strength, but not optimal restoration of function. Dosing at both the mid and high doses resulted in dramatically superior efficacy outcomes as compared to untreated XLMTM dogs, including muscle strength, respiratory function and 100% survival. In addition, biodistribution analyses of both cohorts revealed encouraging expression levels of myotubularin. Specifically, the mid dose resulted in a range of 10% to 40% of normal myotubularin levels, and the high dose resulted in approximately 100% of normal myotubularin levels as measured in a wide range of skeletal muscle.

We intend to conduct additional preclinical studies of AT132, primarily related to safety assessments, prior to the submission of our IND or CTA.

Planned Clinical Development of AT132

The clinical development plan for AT132 currently consists of three studies to evaluate AT132 in children with XLMTM and to characterize the natural history of the disease. We plan to submit an IND or CTA in the first quarter of 2017 and initiate ASPIRO, a Phase 1/2 interventional clinical trial thereafter. We anticipate preliminary data from ASPIRO to be available in the fourth quarter of 2017. We are currently conducting a retrospective chart review and a prospective natural history and run-in study.

- *RECENSUS Study (Ongoing)—Retrospective Medical Chart Review:* The RECENSUS study is an international, non-interventional, retrospective medical chart review of approximately 120 living and deceased XLMTM patients. The purpose of this study is to further characterize the clinical manifestations and natural history of XLMTM. In addition, this study may serve as a historical control for the planned Phase 1/2 ASPIRO trial. However, because the patient population, clinical management and/or other factors may be different than those used in the ASPIRO trial, we may be unable to use the RECENSUS study to demonstrate statistical significance of results in the planned ASPIRO trial.

- *INCEPTUS Study (Ongoing)—Prospective Natural History and Run-In Study:* The INCEPTUS study is an international, non-interventional clinical assessment study of approximately 12 patients, ages three years or younger, with XLMTM. The primary objective of this study is to characterize the disease course and natural history of children with XLMTM, with a specific focus on respiratory and neuromuscular measurements. In addition, the study will assess the burden of disease on XLMTM subjects and caregivers. The study is evaluating subjects over a three to 12-month period prior to potential enrollment in ASPIRO, the Phase 1/2 interventional study of AT132.

- *ASPIRO Study (Planned)—Phase 1/2 Interventional Study:* The ASPIRO study is planned as a Phase 1/2 multicenter, multinational, open-label, dose escalation study evaluating the safety and preliminary efficacy of AT132 in approximately nine XLMTM patients up to four years of age. Primary safety endpoints include an assessment of the rate of adverse events and certain laboratory parameters, and primary efficacy endpoints include assessments of neuromuscular and respiratory function. Secondary endpoints include the burden of disease and health related quality of life, and muscle tissue histology and biomarkers. The primary analysis is expected to be conducted at 12 months, with interim evaluations expected to be conducted at earlier time points. After the primary 12-month assessment, patients are expected to continue on for another four years to assess long term safety, durability of effect and developmental progression.

Regulatory Interactions

We have met with and are planning additional meetings with regulatory authorities regarding our planned IND and CTA submissions. In addition, both the FDA and European Medicines Agency, or EMA, have granted orphan drug designation for AT132.

AT342 for the Treatment of Crigler-Najjar

Overview of Crigler-Najjar

Crigler-Najjar is a rare, congenital autosomal recessive monogenic disease characterized by severely high levels of bilirubin in the blood and risk of irreversible neurological damage and death. Crigler-Najjar is caused by mutations in the gene encoding the UGT1A1 (uridine-diphosphate (UDP)-glucuronosyltransferase (UGT) 1A1) enzyme resulting in an inability to convert unconjugated bilirubin to a water-soluble form that can be excreted from the body. Unconjugated bilirubin can cross the blood brain barrier, and the accumulation of unconjugated bilirubin in the central nervous system can lead to irreversible neurological damage and death.

Infants with Crigler-Najjar develop severe jaundice shortly after birth resulting in rapid presentation and diagnosis. Clinical diagnosis can be confirmed via genetic testing of the UGT1A1 gene. The current standard of care for Crigler-Najjar is aggressive management of high bilirubin levels with persistent, daily phototherapy, usually for longer than 12 hours per day, for the rest of a patient's life. Exchange transfusion or plasmapheresis are sometimes required in order to lower bilirubin levels rapidly. Phototherapy becomes less effective as a child ages, beginning around the age of four years. Average life expectancy is reported as being 30 years of age with phototherapy, and there is an ongoing lifelong risk of a catastrophic cerebral event. Crigler-Najjar is estimated to affect approximately one in 1,000,000 newborns.

Limitations of Current Therapy for Crigler-Najjar

There are currently no products approved specifically for the treatment of Crigler-Najjar. The current standard of care for Crigler-Najjar is aggressive management of high bilirubin levels, with persistent, daily phototherapy, usually for longer than 12 hours per day using intense fluorescent light focused on the bare skin, while the eyes are shielded. The impact on quality of life is substantial. Phototherapy speeds bilirubin decomposition and excretion, lowering serum bilirubin levels. However, the effectiveness of phototherapy typically wanes beginning around four years of age due to thickening of the skin and a reduction in the surface area to body mass ratio. As children get older, compliance with phototherapy becomes challenging. As Crigler-Najjar infants and children begin to experience progression of neurological symptoms and increasing risk of a catastrophic cerebral event, a liver transplant is often required for survival. However, limited donor organ availability, the risks associated with the transplant procedure itself and potential for organ rejection limit the utility of a transplant as a widespread treatment modality for Crigler-Najjar.

AT342 Description

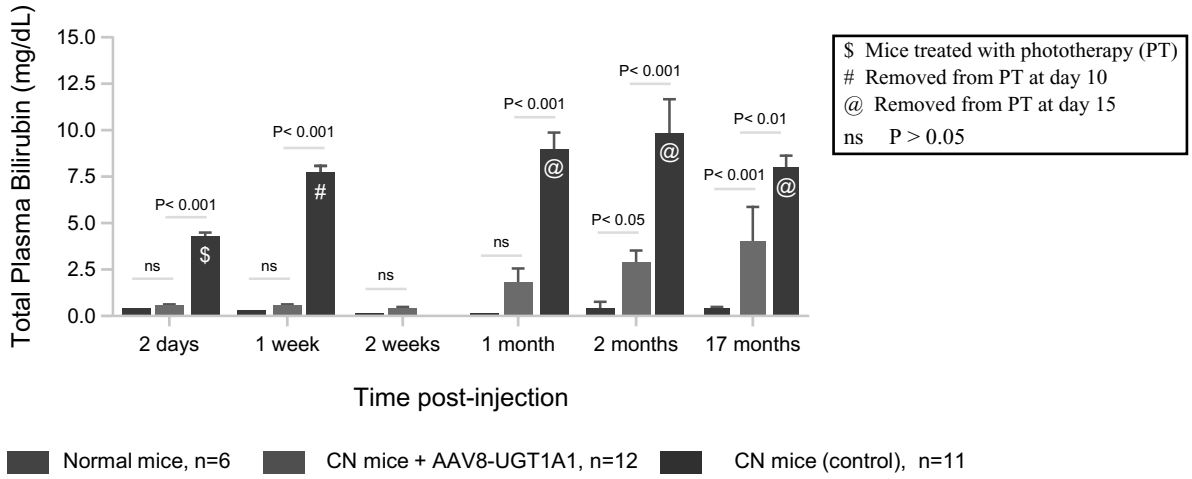
AT342 consists of an AAV8 vector capsid designed to deliver a functional UGT1A1 gene and increase UGT1A1 protein expression in the liver and other tissues. Importantly, AAV8 has high affinity for liver cells allowing for the efficient introduction of therapeutic genes into liver cells. We believe that, if approved and determined by the FDA to be safe and effective, AT342 has the potential to provide long-term clinical benefit to Crigler-Najjar patients through persistent expression of the protein following a single administration, resulting in significant reduction in bilirubin levels, reduction or elimination of the need for lengthy daily phototherapy treatment and elimination of the need for a liver transplant.

Preclinical Proof-of-Concept for AT342

Preclinical proof-of-concept study results have been reported using AAV8-UGT1A1 in a murine model of Crigler-Najjar syndrome. Data demonstrate that a single administration of AAV8-UGT1A1 resulted in a rapid

and significant reduction in total bilirubin levels of 12 mice as compared to 11 mice that received only phototherapy. The administration of AAV8-UGT1A1 also proved durable, lasting the entire 17-month duration of the study. Bilirubin levels at 17 months were over 50% lower in AAV8-UGT1A1 treated mice versus control mice that received only phototherapy. Furthermore, bilirubin levels remained below the level at which neurological damage is observed in this model for the duration of the study.

AAV8-UGT1A1 Reduces Total Bilirubin Levels in a Crigler-Najjar Syndrome Mouse Model



We plan to complete dose selection and toxicology studies with AT342 prior to initiating clinical development.

Planned Clinical Development of AT342

We plan to begin clinical development of AT342 with a natural history and run-in study, followed by a Phase 1/2 clinical trial, in which we plan to evaluate the safety of AT342 in Crigler-Najjar patients, as well as assess efficacy measures including bilirubin levels and time on phototherapy.

We plan to submit an IND or CTA for AT342 in the fourth quarter of 2016, and expect preliminary data from the Phase 1/2 clinical trial in the fourth quarter of 2017.

- *Prospective Natural History and Run-In Study:* The prospective natural history study is planned as an international, non-interventional clinical assessment study of approximately 12 to 15 patients with Crigler-Najjar. The primary objective of this study is to characterize the disease course, natural history, bilirubin variability and phototherapy usage of patients with Crigler-Najjar, with a specific focus on serum bilirubin levels and time on phototherapy. In addition, the study is expected to assess the burden of disease on Crigler-Najjar subjects and caregivers. The study is also expected to identify patients for potential enrollment in the Phase 1/2 study and as a control for the Phase 1/2 study.

- *Phase 1/2 Study:* The Phase 1/2 study of AT342 is planned as a multicenter, multinational, open-label, ascending dose trial to evaluate the safety and efficacy of AT342 in approximately nine Crigler-Najjar patients greater than or equal to two years of age. Planned efficacy measures include serum bilirubin levels and time on phototherapy. The primary analysis is expected to be conducted at 12 months, with interim evaluations expected to be conducted at earlier time points. Other endpoints include the percent of patients receiving phototherapy, quality of life, auditory function and bilirubin kinetics.

Regulatory Interactions

The FDA has granted orphan drug designation for AT342, and we are planning pre-IND and CTA meetings with the FDA and several European Union country health authorities to discuss the planned Phase 1/2 trial of AT342.

AT982 for the Treatment of Pompe Disease

Overview of Pompe Disease

Pompe disease is a rare, severe, progressive, congenital neuromuscular disease. The overall incidence is estimated to be approximately one in 40,000 people although frequency and disease progression varies with age of onset, ethnicity and geography. The disease is characterized by mutations in the gene that encodes the enzyme acid alpha-glucosidase, or GAA. GAA is responsible for degrading glycogen within the lysosome, and dysfunction or absence of functional GAA results in toxic accumulation of glycogen in cells. Tissues and cells most affected by the disease are predominantly skeletal muscle, cardiac muscle and motoneurons.

The severity of Pompe disease symptoms and rate of progression is highly variable and correlated with age of symptom onset and the degree of enzyme deficiency. Infantile or early onset disease, the most severe form of Pompe disease, accounts for approximately one quarter of all affected patients. Those with early-onset disease are usually diagnosed in the first few months of life due to the severe presentation associated with total or near-total absence of GAA activity, and confirmatory diagnosis is made through genetic testing. These infants usually present with feeding difficulties, failure to thrive, muscular hypotonia, progressive weakness, respiratory distress, severe enlargement of the tongue and thickening of the heart muscle. If left untreated, these children usually die in the first year of life. Those with late-onset disease typically have enzyme levels at 1% to 40% of normal and usually have symptoms such as reduced mobility and respiratory problems. Late-onset patients experience progressive difficulty walking and respiratory decline, and although life expectancy can vary, it is a life-limiting disease and death generally occurs due to complications from respiratory failure. Newborn-screening programs can successfully identify Pompe disease in the newborn period, but such programs have not yet been widely implemented worldwide.

Limitations of Current Therapy for Pompe Disease

The only approved treatment for Pompe disease is ERT. Although ERT is the current standard of care for the disease, it has a number of recognized limitations:

- Currently approved versions of ERT are administered every two weeks, and in some cases more frequently.
- Large doses of ERT have to be delivered systemically in order to achieve potentially therapeutic levels in the target tissues, and as a result approximately 93% of patients develop antibodies against the therapy. Such antibody responses may impact both the safety and efficacy of ERT. Currently approved ERT products carry a black box warning related to the risk of life-threatening anaphylaxis, and severe allergic and immune mediated reactions.
- While initial studies of ERT demonstrated that treatment improved survival and ventilator-free survival of patients with early-onset disease, long-term follow-up of these patients indicates substantial disease progression. Subsequent analyses of the effectiveness of ERT have identified variations in outcomes, with most infants exhibiting declines in motor and respiratory function and reduced survival despite treatment.
- ERT is unable to cross the blood-brain barrier and thus cannot reduce the accumulation of glycogen in the brain, spinal cord and peripheral nervous system. It is believed that glycogen accumulation is particularly detrimental to the function of the cells of the peripheral nervous system in Pompe,

especially motoneurons, and thus the inability of ERT to reach these cells may lead to incomplete treatment of the underlying pathology and account for the failure of ERT to halt disease progression and reverse functional decline.

- Chronic therapy with ERT is costly. Experts in health technology assessment have projected the lifetime costs of ERT to be in excess of $7 million for patients with infantile onset Pompe. Due to the requirement of dosing by body weight, the cost for infantile patients increases year-over-year as these patients grow.

AT982 Description

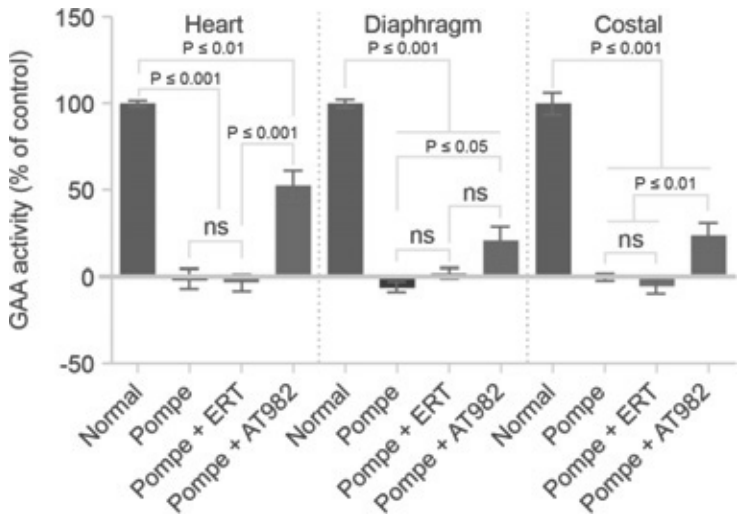
AT982 consists of an AAV9 vector that delivers a GAA gene expression cassette containing a desmin promoter capable of increasing GAA activity in targeted tissues. AT982 was designed with these elements because AAV9 is known to effectively penetrate the heart, muscle and motoneurons and the desmin promoter is known to increase gene expression primarily in muscle but also in motoneurons. We believe AT982 has the potential to provide long-term clinical benefit to patients with Pompe disease through persistent expression of the GAA protein following a single intravenous administration.

Preclinical Proof-of-Concept for AT982

Preclinical studies of AT982 have been conducted in a robust and well established genetically modified murine model of Pompe disease. In these studies, treatment resulted in improvement in several measures of efficacy, including enzyme activity, glycogen clearance and skeletal muscle, cardiac and respiratory function.

A recent study evaluated systemic administration of AT982 to six mice at a dose level of approximately 5 x 10^{12} vg/kg and compared outcomes versus untreated Pompe mice, normal mice and mice treated with multiple doses of ERT. Treatment with AT982, measured at three months following treatment, significantly increased GAA activity in heart, diaphragm and costal muscle versus both untreated mice and mice treated with ERT, as shown in the figure below.

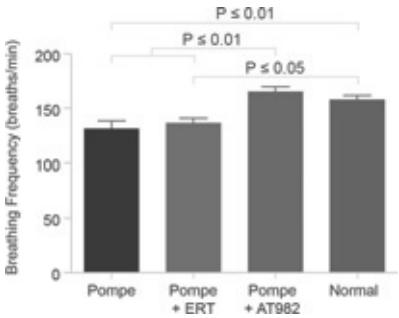
AT982 Significantly Increases GAA Activity Compared to ERT in Multiple Muscle Tissues



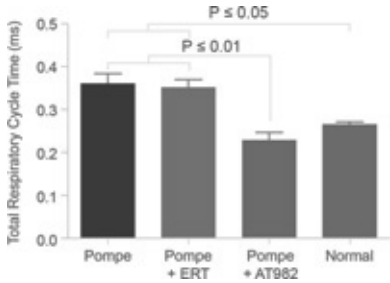
At three months following administration, both the AT982 and ERT-treated groups showed significant improvements in body mass, cardiac function and diaphragm function. However, AT982 also resulted in a statistically significant increase in breathing frequency, a decrease in expiratory time and an increase in timing of the total respiratory cycle as compared with both ERT and control, which resulted in outcomes comparable to those in normal mice. These data are shown in the figure below. The enhanced respiratory function on these parameters compared to ERT may result from increased GAA activity in motoneurons, specifically the phrenic nerve that innervates the diaphragm. For three of the six measures studied, no significant differences were detected between all three groups at this age. Separate studies in the murine model of Pompe have demonstrated the ability of AT982 to enter motoneurons and increase GAA activity.

AT982 Restores Several Respiratory Parameters

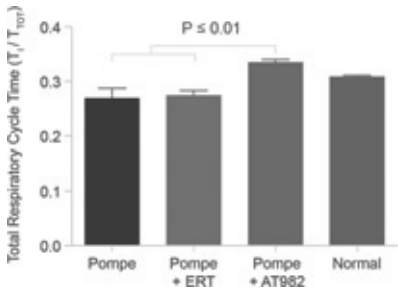
Breathing Frequency



Expiratory Time



Total Respiratory Cycle Time



Planned Clinical Development of AT982

We plan to submit an IND for AT982 in the third quarter of 2016 and initiate the Phase 1/2 proof-of-concept trial in adults with Pompe disease shortly thereafter. We expect preliminary data from the Phase 1/2 proof-of-concept trial to be available in the second half of 2017.

- *Phase 1/2 Proof-of-Concept Study:* The Phase 1/2 proof-of-concept study is planned as a double-blind, randomized trial in approximately eight adult patients with Pompe disease who are currently on enzyme replacement therapy, the current standard of care in Pompe disease. The study is expected to evaluate safety and GAA protein expression after administration of AT982 injected into the TA muscle of the leg, and also after readministration of AT982 into the TA muscle of the contralateral leg. A well-characterized immune modulation strategy is expected to be employed prior to the initial exposure to AT982 in one leg and to the subsequent exposure of AT982 to the contralateral leg after four months. At each dosing of AT982, the contralateral leg is expected to receive placebo. Patients will act as their own control and two different doses are expected to be used to explore a dose response.

- *Potential Future development of AT982:* After the completion of the intra-muscular Phase 1/2 proof-of-concept study, we plan to evaluate different routes of administration of AT982, including intravenous and potentially intrathecal administration.

Regulatory Interactions

A pre-IND meeting has been held with the FDA and a pre-CTA meeting has been held with the EMA. The Phase 1/2 proof of concept study protocol has been reviewed by the National Institutes of Health, or NIH, Recombinant DNA Advisory Committee, or RAC. Both the FDA and EMA have granted orphan drug designation for AT982.

AT307 for the Treatment of CASQ2-Catecholaminergic Polymorphic Ventricular Tachycardia

Overview of CASQ2-CPVT

CASQ2-CPVT is a life-threatening, autosomal recessive, inherited cardiac disease caused by mutations in the CASQ2 gene that encodes the protein called calsequestrin 2. The CASQ2 protein plays a key role in the release of calcium within the cardiac muscle cell, which is necessary for normal cardiac contractile function to maintain normal heart rhythm. It is estimated that CPVT occurs in one in 10,000 people, with approximately 2% to 5% due to mutations in the CASQ2 gene. This equates to an estimated prevalence of 6,000 affected people in North America, Europe and other addressable markets. The number of identified cases is likely to increase with the advent of more accessible genetic testing.

CPVT is characterized by the sudden occurrence of severe ventricular arrhythmia that can cause dizziness and fainting, and can progress rapidly to cardiac arrest and sudden cardiac death. These arrhythmias are triggered during exercise or in response to a sudden stressful occurrence. It is estimated that 30% of people with CASQ2-CPVT will have had a cardiac event by the age of ten, and 79% will have had an event by the age of 40. Untreated, mortality is reported to be in the range of 30% to 50% by the age of 30. In addition, a high proportion of sudden infant death is also thought to be due to severe arrhythmia-related events such as CPVT. Due to the association between exercise, stress and the onset of symptoms, there is a significant impact on the activities of daily living of patients, their families and their caregivers, as any stressful event or activity may trigger an episode, creating considerable anxiety for the patients and their family members. Despite major electrophysiological abnormality, patients with CPVT have a structurally normal heart and a normal baseline electrocardiogram. However, during a cardiac stress test, such as an exercise test on a treadmill, patients with CVPT display a distinct “polymorphic” electrocardiogram that makes clinical diagnosis straightforward.

Limitations of Current Therapy for CPVT

Despite available therapies to treat CPVT, which include beta-blockers and the sodium channel blocker flecainide, it is estimated that 30% to 40% of patients still experience significant cardiac events. Patients unresponsive to available therapies may be candidates for implantation of cardiac defibrillators, though their safety and effectiveness is considerably more limited in young patients. Due to the limitations of existing therapies, there remains a significant unmet medical need for patients with CPVT.

AT307 Description

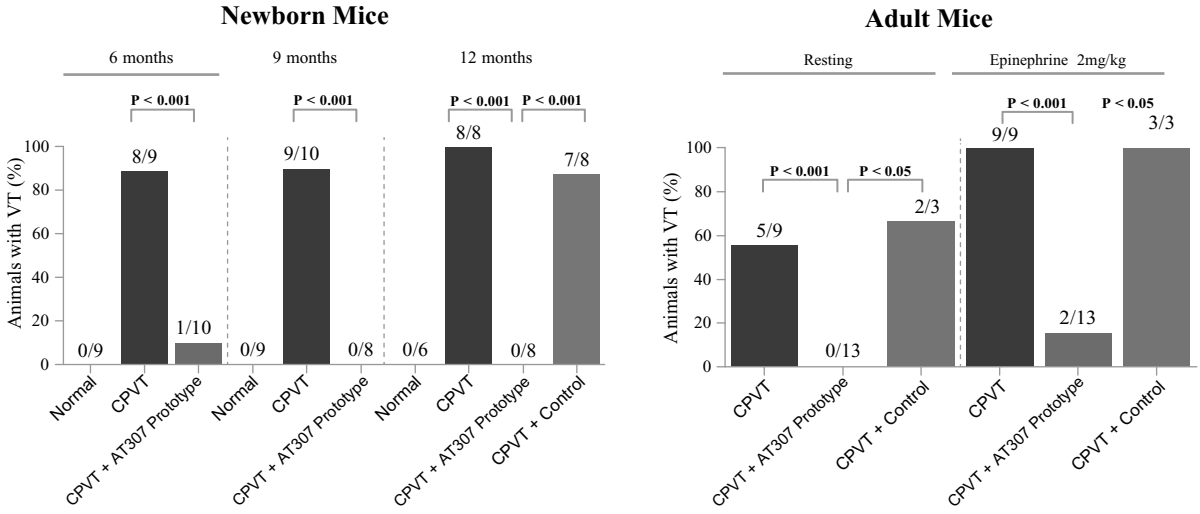
AT307 consists of an AAV9 vector that is designed to deliver a functional CASQ2 gene and to increase CASQ2 protein expression in targeted tissues. We are utilizing AAV9 because it is known to effectively penetrate heart tissue. We are evaluating a number of different promoters and other proprietary vector structural elements to optimize AT307 for transgene expression and product quality. We believe AT307 has the potential to provide long-term clinical benefit to CASQ2-CPVT patients through persistent expression of the protein following a single administration, resulting in a significant reduction in life-threatening arrhythmic events and other disease symptoms.

Preclinical Proof-of-Concept for AT307

Initial preclinical proof-of-concept studies were conducted using an AT307 prototype product candidate in a genetically engineered murine model of CASQ2-CPVT. This mouse manifests stress-induced arrhythmias upon epinephrine administration, as well as cellular and molecular manifestations of the disease. In this model, a single administration of the AT307 prototype to nine mice resulted in a significant improvement in CASQ2 protein expression to a level approaching that of normal animals. Cardiomyocytes isolated from animals with a CASQ2 mutation show abnormal electrophysiology, as demonstrated by pre-arrhythmic events such as increased delayed after depolarizations and triggered activity. Cardiomyocytes isolated from the affected mice treated with the AT307 prototype had electrophysiology indistinguishable from that of normal mice.

Additionally, the efficacy of the AT307 prototype was evaluated in studies in both newborn and adult affected mice. In both studies treatment resulted in significant reductions in ventricular tachycardia versus untreated controls when challenged with epinephrine. The effect of this single treatment lasted for the one-year duration of the studies.

AT307 Prototype Improves Ventricular Tachycardia in Newborn and Adult Mice



We are conducting a large-animal study to determine the optimal route of administration of AT307, and are conducting additional studies in the murine model to select our development candidate and to determine an appropriate dose for our planned clinical trial.

Planned Clinical Development of AT307

We plan to submit an IND or CTA for AT307 in 2017 and initiate a Phase 1/2 study thereafter. In this study, we plan to determine the safety of AT307 in patients with CASQ2-CPVT and to use the clear efficacy endpoint of an exercise electrocardiogram as a means to evaluate therapeutic benefit.

Regulatory Interactions

Both the FDA and EMA have granted orphan drug designation for AT307, and we plan to discuss our development plans with the FDA and several European Union country health authorities.

Manufacturing

We believe it is important to our business to ensure reliable, high quality clinical and commercial supply that is produced cost effectively. For these reasons, we are building strong scientific AAV process development

and manufacturing teams and are investing in a state-of-the-art cGMP manufacturing facility in South San Francisco to develop and implement novel in-house production technologies. We view the development of internal manufacturing capacity as a key competitive advantage as it allows for better control over product development timelines, costs and intellectual property, such as trade secrets, novel inventions and proprietary knowledge. Our process development and manufacturing teams are composed of a combination of industry veterans and established key opinion leaders in the field of AAV manufacturing.

Process development research is currently ongoing in our internal laboratories. Our new manufacturing facility is also supporting our development programs with production of AAV vectors for preclinical studies, and is expected to be available for cGMP manufacturing of our product candidates in the second half of 2016. We anticipate this facility will be capable of providing cGMP supply at scale suitable for commercial production by 2018.

Our manufacturing strategy focuses on utilizing mammalian cells as the substrate for AAV-based product candidates. Mammalian cells are the natural host for AAV, and so provide a cellular environment most closely mimicking that in which the virus normally replicates. We believe that matching the production host cell to the vector in this way best preserves the quality of the replication complexes responsible for synthesizing viral vector genomes and creating, assembling and filling viral vector capsids with those genomes. Our early phase product candidates are manufactured using transient transfection, in which genetic components for vector production are supplied to cells during each manufacturing run. We are evaluating a future transition to a stable cell line system, in which at least some genetic components are permanently integrated into the host cell genome before manufacturing occurs.

Our current production process utilizes HEK293 cells, which are the most commonly used host cell for AAV vector production. These cells are familiar to regulatory authorities and commercial cell culture media manufacturers, and take up foreign DNA robustly to produce high transient vector titers. Our early clinical stage production platform utilizes serum-free suspension cell culture of HEK293 cells and transient transfection of plasmids to produce clinical grade AAV vectors in a scalable process. We believe this approach maximizes speed of development, product quality and regulatory compliance. Further, our analytical team utilizes the latest technologies for characterization of biological molecules to enable the creation of strict standards of quality and potency that we believe will differentiate our products from others in the field.

Our Plans for Clinical and Commercial Scale-Up

As our products progress through clinical development, we plan to transition their production to newer mammalian cell processes that maximize vector product yield while maintaining the high quality derived from the current processes. This may include transitioning to a stable cell producer system, which our team is currently evaluating in our research facilities. As large scale gene therapy manufacturing remains a new discipline, we view our investment in the capacity to develop, manufacture and analyze AAV vectors as strategically important, and we expect it to yield intellectual property and know-how that benefits both our internal programs and the broader gene therapy field.

Current Status of Manufacturing

We have established relationships with research facilities, contract manufacturing organizations, or CMOs, and our collaborators to manufacture and supply our product candidates for preclinical and clinical studies. We plan to manufacture AT132 in our own facilities, and anticipate that AT342 will be initially manufactured by a CMO. AT982 is currently manufactured by the University of Florida in a facility that we believe complies with cGMPs. As we establish and scale our internal manufacturing capabilities, we plan to transition all process development and manufacturing activities to our own facilities.

Intellectual Property

We have licensed numerous patents and patent applications and possess substantial proprietary know-how and trade secrets relating to our development programs and manufacturing capabilities. We strive to protect and enhance the proprietary technology, inventions and improvements that are commercially important to the development of our business by seeking, maintaining and defending our intellectual property, whether developed internally or licensed from third parties. We also rely on trade secrets, know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position in the field of gene therapy. Additionally, we intend to rely on regulatory protection afforded through rare drug designations, data exclusivity and market exclusivity as well as patent term extensions, where available.

We are heavily dependent on patented or proprietary technologies that we license from third parties. For additional information regarding these license agreements, see "—License and Collaboration Agreements." We anticipate that we will require additional licenses to third-party intellectual property rights relating to our development programs in the future, which may not be available on commercially reasonable terms, if at all.

Our in-licensed patents and patent applications are directed to the compositions of matter and methods of use related to various aspects of our product candidates as well as certain aspects of our manufacturing capabilities. As of March 31, 2016, we had filed one U.S. provisional patent application directed to modified AAV vectors and methods of manufacturing the same. If granted, we expect this patent would expire in 2036. Our in-licensed patent portfolio as it relates to one or more of our product candidates includes:

- one U.S. patent relating to AT132, expiring in 2034, as well as one U.S. patent application, comprising claims directed to recombinant AAV for use in treating XLMTM and AAV constructs containing the MTM gene under control of the desmin promoter and uses thereof;

- one U.S. patent application relating to AT342, which, if granted, would be projected to expire in 2036, comprising claims directed to recombinant AAV for use in treating Crigler-Najjar and AAV constructs containing a codon-optimized UGT1A1 gene;

- four U.S. patents, expiring between 2022 and 2024, and one U.S. patent application as well as corresponding patents and patent applications internationally, relating to recombinant AAV vectors having an AAV8 capsid utilized in AT132 and AT342;

- two U.S. patent applications projected to expire between 2028 and 2032, as well as corresponding patent applications internationally relating to AT982, comprising claims directed to recombinant AAV having an AAV9 capsid for use in treating Pompe disease and AAV constructs containing the GAA gene under control of the desmin promoter and uses thereof;

- one U.S. patent relating to AT307, expiring in 2032, as well as one U.S. patent application, each with claims directed to methods of treating recessive CPVT by CASQ2 gene therapy; and

- one U.S. patent, expiring in 2026, and one U.S. patent application as well as corresponding patents and patent applications internationally, relating to recombinant AAV vectors having an AAV9 capsid utilized in AT982 and AT307.

The term of individual patents may vary based on the countries in which they are obtained. Generally, patents issued for applications filed in the United States are effective for 20 years from the earliest effective non-provisional filing date. In addition, in certain instances, a patent term can be extended to recapture a portion of the term effectively lost as a result of FDA regulatory review period. The restoration period cannot be longer than five years and the total patent term, including the restoration period, must not exceed 14 years following FDA approval. The duration of patents outside of the United States varies in accordance with provisions of applicable local law, but typically is also 20 years from the earliest effective filing date.

In addition to patents and patent applications that we license, we rely on trade secrets and know-how to develop and maintain our competitive position. For example, significant aspects of our AAV manufacturing capabilities and gene therapy technology are based upon trade secrets and know-how. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, and obtain and maintain ownership of certain technologies, in part, through confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and commercial partners. We also seek to preserve the integrity and confidentiality of our data, trade secrets and know-how, including by implementing measures intended to maintain the physical security of our premises and the physical and electronic security of our information technology systems.

Our future commercial success depends, in part, on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business; defend and enforce our patents; preserve the confidentiality of our trade secrets; and operate without infringing the valid enforceable patents and proprietary rights of third parties. Our ability to stop third parties from making, using, selling, offering to sell or importing our products may depend on the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities. With respect to our licensed intellectual property, we cannot be sure that patents will issue with respect to any of the pending patent applications to which we license rights or with respect to any patent applications that we or our licensors may file in the future, nor can we be sure that any of our licensed patents or any patents that may be issued in the future to us or our licensors will be commercially useful in protecting our product candidates and methods of manufacturing the same. Moreover, we may be unable to obtain patent protection for certain of our product candidates generally, as well as with respect to certain indications. See the section entitled "Risk Factors—Risks Related to Our Intellectual Property" for a more comprehensive description of risks related to our intellectual property.

License and Collaboration Agreements

We have built our portfolio of product candidates in part by engaging in strategic transactions with third parties. We intend to continue to collaborate with additional third parties to expand our pipeline of product candidates, as well as to deepen our existing relationships with our collaborators and licensors. We intend to leverage these relationships to continue to advance the scientific understanding of the indications we target. We have in the past supported investigator-sponsored preclinical studies and clinical trials, and may do so in the future with our current and future collaborators.

REGENXBIO License Agreement (XLMTM/Pompe)

In July 2013, we entered into an exclusive license agreement with REGENXBIO Inc. (formerly ReGenX Biosciences, LLC), or REGENXBIO. Under the agreement, REGENXBIO granted us an exclusive worldwide license under certain patent rights to make, have made, use, import, sell and offer for sale licensed products in the treatment of both XLMTM and Pompe disease using both AAV8 and AAV9.

As consideration for the licensed rights, we paid REGENXBIO an initial fee of $0.3 million and 50,228 shares of our common stock. We will also owe REGENXBIO (i) an annual maintenance fee; (ii) up to $8.85 million in combined milestone fees per licensed product related to XLMTM and up to $8.85 million in combined milestone fees per licensed product related to Pompe disease, a small portion of which may be paid in the form of shares of our common stock; (iii) mid to high single digit royalty percentages on net sales of licensed products and (iv) mid-single digit to low twenties royalty percentages of any sublicense fees we receive from sublicensees for the licensed patent rights.

We are obligated to achieve certain development milestones, including submission to the FDA and subsequent effectiveness of an IND for each indication within a specified time period, which we may extend for additional time for a specified number of extensions upon the payment of a fee.

The agreement will expire upon the expiration, lapse, abandonment or invalidation of the last claim of the licensed patent rights to expire, lapse or become abandoned or unenforceable in all countries worldwide. We may terminate the agreement upon prior written notice. REGENXBIO may terminate the agreement immediately if we or our affiliates become insolvent, if we are late by a specified number of days in paying money due under the agreement or if we or our affiliates commence any action against REGENXBIO or its licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate the agreement for material breach that is not cured within a specified number of days.

REGENXBIO License Agreement (Crigler-Najjar Syndrome)

In November 2015, we entered into a second license agreement with REGENXBIO. Under the agreement, REGENXBIO granted us an exclusive worldwide license under certain patent rights to make, have made, use, import, sell and offer for sale licensed products for the treatment of Crigler-Najjar syndrome in humans using AAV8.

As consideration for the licensed rights, we paid REGENXBIO an upfront fee of $0.2 million and an additional $0.4 million upon our entry into the license and collaboration agreement with the University of Pennsylvania. We will also owe REGENXBIO (i) an annual maintenance fee; (ii) up to $7.6 million in combined development and regulatory milestone fees per licensed product; (iii) mid-single digit to low teens royalty percentages on net sales of licensed products sold by us, our affiliates and sublicensees and (iv) a low twenties percentage of certain sublicense fees we receive from sublicensees for the licensed products and certain fees we receive from the sale or transfer of specified rights related to a licensed product.

Under the agreement we are obligated to diligently use commercially reasonable efforts to develop, commercialize, market, promote and sell licensed products. We are also obligated to achieve certain development milestones, including submission to the FDA and subsequent effectiveness of an IND application, or acceptance by the European Medicines Agency of an equivalent application, within a specified time period, which we may extend for a specified number of extensions upon the payment of certain fees.

The agreement will continue on a country-by-country and licensed product-by-licensed product basis and expire upon the later of the expiration, lapse, abandonment or invalidation of the last claim of the licensed patent rights to expire, lapse or become abandoned or unenforceable in such country, or ten years after first commercial sale of such licensed product in such country. We may terminate the agreement upon prior written notice. REGENXBIO may terminate the agreement immediately in case of our bankruptcy, or other similar events, if we are late in paying money due under the agreement and do not pay in full within a specified number of days after receiving written notice, or if we or our affiliates commence any action against REGENXBIO or its licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate the agreement for material breach that is not cured within a specified number of days.

REGENXBIO License Agreement (CPVT)

Also in November 2015, we entered into a third license agreement with REGENXBIO. Under the agreement, REGENXBIO granted us an exclusive worldwide license under certain patent rights to make, have made, use, import, sell and offer for sale licensed products for the treatment of CPVT in humans using AAV9. Within a specified time and upon written notice we may elect to substitute for, or add to, CPVT certain other inherited arrhythmias.

As consideration for the licensed rights, we paid REGENXBIO an upfront fee of $1.0 million. For each additional indication we may elect to pursue under the licensed rights, we agreed to pay REGENXBIO a fee of $0.5 million upon such election. We will also owe REGENXBIO (i) an annual maintenance fee for each covered indication; (ii) up to $8.8 million in combined development and regulatory milestone fees for each indication and each licensed product; (iii) up to $45.0 million in combined commercial milestone fees based on various annual aggregate net sales thresholds; (iv) mid-single digit to low teens royalty percentages on net sales of licensed

products sold by us, our affiliates and sublicensees and (v) a low twenties percentage of any sublicense fees we receive from sublicensees for the licensed products and certain fees we receive from the sale or transfer of specified rights related to a licensed product.

Under the agreement, we are obligated to use commercially reasonable efforts to develop, commercialize, market, promote and sell licensed products for each indication. We are also obligated to achieve certain development milestones for each indication, including submission to the FDA and subsequent effectiveness of an IND application, or acceptance by the European Medicines Agency of an equivalent application, within a specified time period, which we may extend for additional time and for a specified number of extensions upon the payment of certain fees.

The agreement will continue on a country-by-country and licensed product-by-licensed product basis and expire upon the later of the expiration, lapse, abandonment or invalidation of the last claim of the licensed patent rights to expire, lapse or become abandoned or unenforceable in such country, or ten years after first commercial sale of such licensed product in such country. We may terminate the agreement in its entirety or for each elected disease indication upon prior written notice. REGENXBIO may terminate the agreement immediately in case of our bankruptcy, or other similar events, if we are late in paying money due under the agreement and do not pay in full within a specified number of days after receiving written notice, or if we or our affiliates commence any action against REGENXBIO or its licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate the agreement for material breach that is not cured within a specified number of days.

Genethon Collaborative Development Agreement

In January 2014, we entered into a collaborative development agreement with Genethon, a French not-for-profit organization. Subject to certain limitations on patents that are co-owned or in-licensed by us, Genethon granted us a royalty-free, exclusive, worldwide license under certain background intellectual property rights controlled by Genethon to research, develop, make and commercialize certain products for the treatment of XLMTM. In addition, the collaboration agreement provides that new intellectual property arising from the performance of the development plan will be owned jointly by both parties and Genethon granted us a royalty-free, exclusive, worldwide license to Genethon's interest in such new intellectual property to research, develop, make and commercialize certain products for the treatment of XLMTM. Genethon also granted us a right of first negotiation to negotiate rights to other internal research programs conducted by Genethon to research, develop, manufacture or commercialize other products for the treatment of XLMTM that are not already included within the scope of this agreement.

In connection with the entry into the collaborative development agreement, we issued 262,396 shares of our common stock to Genethon, of which 87,465 shares vested immediately, 87,465 shares vested in January 2015 and 87,466 shares vested in January 2016. Unvested shares are subject to a repurchase option at our election in the event of any termination of the agreement. Unvested shares will become fully vested in the event we undergo a change in control or an initial public offering. Genethon also received certain registration rights and information rights, similar to those held by our preferred stockholders.

The agreement provides Genethon with the exclusive right to manufacture licensed product for preclinical and clinical purposes, subject to Genethon's ability to supply required quantities in accordance with applicable timelines. Manufacturing costs will be paid by us. Under the agreement, we are obligated to fund Genethon's research and development activities related to AT132.

Unless earlier terminated, the agreement will stay in effect until completion of the research program and our license grants will survive any expiration of the agreement. Either party may terminate the agreement for the other party's uncured material breach of the agreement or for the other party's bankruptcy. We may terminate the agreement for convenience upon prior written notice. Genethon may terminate the agreement upon raising an

objection to continued development on grounds of a safety or efficacy issue and upon prior written notice of such objection.

University of Florida License Agreement

Effective July 2015, we entered into a license agreement with the University of Florida Research Foundation, or UFRF, which was amended in June 2016. Under the agreement, UFRF granted us an exclusive, worldwide license under certain patent rights and a non-exclusive license to certain related know-how for the treatment of Pompe. We agreed to pay an upfront license fee, an annual maintenance fee until first commercial sale of a licensed product, up to $1.2 million in combined development and regulatory milestone payments, and a low single digit royalty on net sales of licensed products sold by us and our sublicensees, subject to minimum annual royalty payments following the first commercial sale of a licensed product. We are obligated to pay royalties on a country-by-country basis until the later of expiration of the last valid claim within the licensed patent rights in such country and ten years after first commercial sale of a licensed product in such country. We also agreed to pay to UFRF certain percentages of sublicense fees we receive from sublicensees of the licensed patent rights based on the stage of development at the time the sublicense is executed.

Under the agreement, we are obligated to diligently perform a specified development plan and to use commercially reasonable efforts to market and commercialize at least one licensed product which has obtained regulatory approval. We are also obligated to achieve a number of diligence milestones, including the achievement of first commercial sale within a specific time period. If we fail to meet any of these diligence milestones and the deadlines are not extended in accordance with the terms of the agreement, then UFRF may terminate the agreement.

We may terminate the agreement for convenience upon prior written notice. UFRF may terminate the agreement upon prior written notice for breach of the agreement by us, including specific listed breaches, our violation of laws or regulations in the development or commercialization of licensed products or our bankruptcy or liquidation. In addition, UFRF may terminate the agreement immediately if we or our affiliates challenge the validity, patentability or enforceability of the licensed patents rights. If the challenge is brought by a sublicensee, UFRF may request that we terminate the sublicense.

FSM License Agreement

In August 2015, we acquired Cardiogen Sciences, Inc., or Cardiogen. Through this transaction, we acquired a license agreement previously entered into by Cardiogen with the Fondazione Salvatore Maugeri, or FSM an Italian non-profit organization. Under the license agreement, we obtained an exclusive worldwide license to certain intellectual property to develop, use and commercialize products related to recessive CPVT, as well as to several additional inherited arrhythmias. Under the agreement we are obligated to use commercially reasonable efforts to develop and, after receiving regulatory approval for products in a given country, commercialize such products in such country.

As consideration for the license, Cardiogen issued 425,000 shares of Cardiogen common stock to FSM. In connection with our acquisition of Cardiogen, the Cardiogen shares held by FSM were cancelled and converted into 51,968 shares of our common stock. We also agreed to pay FSM low single digital royalties on net sales of licensed products for as long as such product is covered by a valid claim of the licensed patents in the applicable country.

We may terminate the agreement for convenience upon prior written notice. Either party may terminate the agreement upon prior written notice for the uncured material breach of the agreement by the other party or the other party’s bankruptcy or liquidation.

University of Pennsylvania License and Collaboration Agreement

In May 2016, we entered into a license and collaboration agreement with The Trustees of the University of Pennsylvania, or the University of Pennsylvania. Under the agreement, the University of Pennsylvania granted us an exclusive worldwide license under certain patent rights to research, develop, use, sell, offer for sale, have sold, make, have made and import licensed products for the treatment of Crigler-Najjar.

As consideration for the licensed rights, we paid the University of Pennsylvania an upfront fee of $0.5 million, as well as $3.0 million for certain preclinical development activities. We are obligated to pay the University of Pennsylvania (i) up to an aggregate of $6.0 million for preclinical development activities agreed upon by both parties, subject to adjustment based on the work plan, which amount includes the $3.0 million already paid in May 2016, (ii) up to an aggregate of $13.7 million in development, regulatory and net sales milestone payments for the first licensed product; (iii) low to mid single-digit royalty percentages on tiered annual net sales of the licensed products sold by us, our affiliates or sublicensees and (iv) mid single-digit to low double-digit percentages of any sublicense fees we receive from third parties for the grant of sublicenses to any licensed patent rights.

Under the agreement, we are obligated to use commercially reasonable efforts to develop, pursue regulatory approval for, market and commercialize at least one licensed product. The University of Pennsylvania will be responsible for conducting preclinical development activities according to a work plan, including all IND-enabling non-clinical studies and research grade manufacturing. We will be responsible for regulatory strategy and operations, clinical development, GMP manufacture and commercialization of the licensed products.

The agreement will continue on a country-by-country basis and expire upon the later of the expiration of the last valid claim of the licensed patent rights that covers the exploitation of such licensed patent rights in such country, or ten years after first commercial sale of such licensed product in such country. We may terminate the agreement upon 60 days' prior written notice. Either party may terminate the agreement for material breach that is not cured within a specified number of days.

Competition

The biotechnology and pharmaceutical industries, including the gene therapy field, are characterized by rapidly changing technologies, competition and a strong emphasis on intellectual property. We are aware of several companies focused on developing gene therapies in various indications as well as several companies addressing other methods for modifying genes and regulating gene expression. We may also face competition from large and specialty pharmaceutical and biotechnology companies, academic research institutions, government agencies and public and private research institutions. The key competitive factors affecting the success of any approved product will include the efficacy, safety profile, method of administration, cost and level of promotional activity.

For our product candidates, we are aware of the following competing efforts:

- ***AT132 for XLMTM.*** Valerion Therapeutics, LLC is studying VAL-0620, a fusion protein consisting of an antibody linked to the MTM1 protein. Preclinical evaluation of this approach in an XLMTM murine model demonstrated improvements in both muscle structure and function, as reported in a 2013 publication. This program has not been reported by Valerion Therapeutics, LLC to have progressed to clinical development.

- ***AT342*** for ***Crigler-Najjar.*** The current standard of care for the treatment of Crigler-Najjar is phototherapy, and when urgent treatment is needed to avoid neurological damage, aggressive intravenous fluid hydration, management of glucose levels, albumin administration and plasma exchange may be provided. Upon disease progression, liver transplant may be required for survival.

There are currently no products approved for the treatment of Crigler-Najjar. Genethon, a French not-for-profit organization, is developing an AAV-UGT1A1 gene therapy for the treatment of Crigler-Najjar syndrome, and has announced plans to initiate clinical development by the end of 2016. Promethera has received orphan designation from the FDA and European Commission for the treatment of Crigler-Najjar syndrome for HepaStem, a product that comprises heterologous human adult liver progenitor cells. Promethera previously completed a Phase 1/2 study that enrolled patients with Crigler-Najjar syndrome or ornithine transcarbamylase deficiency. No further development in Crigler-Najjar syndrome has been announced for HepaStem. Additionally, Alexion recently announced that, in collaboration with Moderna, it is developing a messenger RNA product candidate for the treatment of Crigler-Najjar.

- ***AT982 for Pompe Disease.*** The current standard of care for the treatment of Pompe disease is ERT with recombinant GAA protein. Genzyme Corporation currently markets MYOZYME and LUMIZYME, which are ERTs for the treatment of Pompe disease. Multiple companies, including Genzyme Corporation, Amicus Therapeutics, Inc., Valerion Therapeutics, LLC and Oxyrane UK Limited are currently reported to be developing next generation ERT to treat Pompe disease. The furthest advanced of these is neoGAA from Genzyme Corporation. In addition, there are currently multiple academic institutions and companies researching alternative gene therapy approaches to treating Pompe disease. We do not believe these approaches utilize AAV9 capsids for motoneuron targeting and none are currently reported to be in clinical development.

- ***AT307 for CASQ2-CPVT.*** To date, no therapies have been approved specifically for the treatment of CASQ2-CPVT. Beta-blockers, including nadolol or propranolol, are currently used as first line treatment, sometimes with the addition of a calcium channel blocker such as verapamil. The sodium channel blocker flecainide, and implantable cardioverter defibrillators are also currently used in the treatment of CASQ2-CPVT. Heart transplant is used infrequently as a last-line therapy in refractory cases of CPVT. Additionally, there are no known investigational therapies in development for CASQ2-CPVT.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources than we do, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of competitors. Our commercial opportunity could be reduced or eliminated if 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 product candidates that we may develop. Competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market, if ever. Additionally, new or advanced technologies developed by our competitors may render our current or future product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors.

Government Regulation and Product Approval

Government authorities in the United States, at the federal, state and local level, and in other countries and jurisdictions, including the European Union, extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting, and import and export of pharmaceutical products. The processes for obtaining regulatory approvals in the United States and in foreign countries and jurisdictions, along with subsequent compliance with applicable statutes and regulations and other regulatory authorities, require the expenditure of substantial time and financial resources.

FDA Approval Process

In the United States, pharmaceutical products are subject to extensive regulation by the FDA. The Federal Food, Drug, and Cosmetic Act, or the FDC Act, and other federal and state statutes and regulations, govern, among other things, the research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling, and import and export of pharmaceutical products. Biological products used for the prevention, treatment, or cure of a disease or condition of a human being are subject to regulation under the FDC Act, except the section of the FDC Act which governs the approval of New Drug Applications, or NDAs. Biological products, such as our gene therapy products, are approved for marketing under provisions of the Public Health Service Act, or PHSA, via a Biologics License Application, or BLA. However, the application process and requirements for approval of BLAs are very similar to those for NDAs, and biologics are associated with similar approval risks and costs as drugs. Failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as clinical hold, FDA refusal to approve pending NDAs or BLAs, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties, and criminal prosecution.

Biological product development for a new product or certain changes to an approved product in the United States typically involves preclinical laboratory and animal tests, the submission to the FDA of an investigational new drug application, or IND, which must become effective before clinical testing may commence, and adequate and well-controlled clinical trials to establish the safety and effectiveness of the drug for each indication for which FDA approval is sought. Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity, and novelty of the product or disease.

Preclinical tests include laboratory evaluation of product chemistry, formulation, and toxicity, as well as animal trials to assess the characteristics and potential safety and efficacy of the product. The conduct of the preclinical tests must comply with federal regulations and requirements, including Good Laboratory Practices. The results of preclinical testing are submitted to the FDA as part of an IND along with other information, including information about product chemistry, manufacturing and controls, and a proposed clinical trial protocol. Long-term preclinical tests, such as tests of reproductive toxicity and carcinogenicity in animals, may continue after the IND is submitted. A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has neither commented on nor questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin. Clinical trials involve the administration of the investigational biologic to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted: (i) in compliance with federal regulations; (ii) in compliance with Good Clinical Practice, or GCP, an international standard meant to protect the rights and health of patients and to define the roles of clinical trial sponsors, administrators, and monitors; as well as (iii) under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. Each protocol involving testing on U.S. patients and subsequent protocol amendments must be submitted to the FDA as part of the IND.

The FDA may order the temporary or permanent discontinuation of a clinical trial at any time, or impose other sanctions, if it believes that the clinical trial either is not being conducted in accordance with FDA regulations or presents an unacceptable risk to the clinical trial patients. The trial protocol and informed consent information for patients in clinical trials must also be submitted to an institutional review board, or IRB, for approval. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements, or may impose other conditions if it believes that the patients are subject to unacceptable risk.

Clinical trials to support BLAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In Phase 1, the initial introduction of the biologic into healthy human subjects or patients, the product is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with drug exposure, and to obtain early evidence of a treatment effect if possible. Phase 2

usually involves trials in a limited patient population to determine the effectiveness of the drug or biologic for a particular indication, determine optimal dose and regimen, and to identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 trials are undertaken to obtain additional information about clinical effects and confirm efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit the FDA to evaluate the overall benefit-risk relationship of the drug or biologic and to provide adequate information for the labeling of the product. In most cases, the FDA requires two adequate and well-controlled Phase 3 clinical trials to demonstrate the safety and efficacy of the drug or biologic. In rare instances, a single Phase 3 trial with other confirmatory evidence may be sufficient where there is a large multicenter trial demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity or prevention of a disease with a potentially serious outcome and confirmation of the result in a second trial would be practically or ethically impossible.

After completion of the required clinical testing, a BLA is prepared and submitted to the FDA. FDA approval of the BLA is required before marketing and distribution of the product may begin in the United States. The BLA must include the results of all preclinical, clinical, and other testing and a compilation of data relating to the product's pharmacology, chemistry, manufacture, and controls. The cost of preparing and submitting a BLA is substantial. The submission of most BLAs is additionally subject to a substantial application user fee, currently exceeding $2,374,000 for Fiscal Year 2016. Under an approved BLA, the applicant is also subject to annual product and establishment user fees, currently exceeding $114,000 per product and $585,000 per establishment for Fiscal Year 2016. These fees are typically increased annually. The FDA has 60 days from its receipt of a BLA to determine whether the application will be accepted for filing based on the Agency's determination that it is adequately organized and sufficiently complete to permit substantive review. Once the submission is accepted for filing, the FDA begins an in-depth review. The FDA has agreed to certain performance goals to complete the review of BLAs. Most applications are classified as Standard Review products that are reviewed within ten months of the date the FDA accepts the BLA for filing; applications classified as Priority Review are reviewed within six months of the date the FDA accepts the BLA for filing. A BLA can be classified for Priority Review when the FDA determines the biologic product has the potential to treat a serious or life-threatening condition and, if approved, would be a significant improvement in safety or effectiveness compared to available therapies. The review process for both standard and priority reviews may be extended by the FDA for three or more additional months to consider certain late-submitted information, or information intended to clarify information already provided in the BLA submission.

The FDA may also refer applications for novel biologic products, or biologic products that present difficult questions of safety or efficacy, to be reviewed by an advisory committee—typically a panel that includes clinicians, statisticians and other experts—for review, evaluation, and a recommendation as to whether the BLA should be approved. The FDA is not bound by the recommendation of an advisory committee, but generally follows such recommendations. Before approving a BLA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. Additionally, the FDA will inspect the facility or the facilities at which the biologic product is manufactured. The FDA will not approve the product unless compliance with cGMP is satisfactory and the BLA contains data that provide substantial evidence that the biologic is safe, pure, potent and effective in the claimed indication.

After the FDA evaluates the BLA and completes any clinical and manufacturing site inspections, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the BLA submission and may require substantial additional testing, or information, in order for the FDA to reconsider the application for approval. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the BLA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included. An approval letter authorizes commercial marketing and distribution of the biologic with specific prescribing information for specific indications. As a condition of BLA approval, the FDA may require a risk evaluation and mitigation strategy, or REMS, to help ensure that the benefits of the biologic outweigh the potential risks to

patients. A REMS can include medication guides, communication plans for healthcare professionals, and elements to assure a product's safe use, or ETASU. An ETASU can include, but is not limited to, special training or certification for prescribing or dispensing the product, dispensing the product only under certain circumstances, special monitoring, and the use of patient-specific registries. The requirement for a REMS can materially affect the potential market and profitability of the product. Moreover, the FDA may require substantial post-approval testing and surveillance to monitor the product's safety or efficacy.

Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing. Changes to some of the conditions established in an approved BLA, including changes in indications, product labeling, manufacturing processes or facilities, require submission and FDA approval of a new BLA or BLA supplement before the change can be implemented. A BLA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing BLA supplements as it does in reviewing BLAs.

Additional Regulation for Gene Therapy Products

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. FDA has issued various guidance documents regarding gene therapies, which outline additional factors that 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 potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. For instance, FDA usually recommends that sponsors observe all surviving subjects who receive treatment using gene therapies in clinical trials for potential gene therapy-related delayed adverse events for a minimum 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. FDA does not require the long-term tracking to be complete prior to its review of the BLA.

In addition, if a gene therapy trial is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, a protocol and related documentation must be submitted to, and the study registered with, the NIH Office of Biotechnology Activities, or OBA, pursuant to the NIH Guidelines for Research Involving Recombinant DNA Molecules, prior to the submission of an IND to the FDA. In addition, many companies and other institutions not subject to the NIH Guidelines voluntarily follow them. The NIH convenes the RAC, a federal advisory committee, to discuss selected protocols and informed consent documents that raise novel or particularly important scientific, safety or ethical considerations at one of its quarterly public meetings. The OBA notifies 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 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.

Fast Track Designation and Accelerated Approval

The FDA is required to facilitate the development, and expedite the review, of biologics that are intended for the treatment of a serious or life-threatening disease or condition for which there is no effective treatment and which demonstrate the potential to address unmet medical needs for the condition. Under the Fast Track program, the sponsor of a new biologic product candidate may request that the FDA designate the product for a specific indication for Fast Track status concurrent with, or after, the filing of the IND. The FDA must determine if the biologic product candidate qualifies for Fast Track designation within 60 days of receipt of the sponsor's request. Under the Fast Track program and FDA's accelerated approval regulations, the FDA may approve a biologic product for a serious or life-threatening illness or condition that provides meaningful

therapeutic benefit to patients over existing treatments based upon a surrogate endpoint. A surrogate endpoint is an endpoint that is reasonably likely to predict clinical benefit, or is a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A biologic product candidate approved using a surrogate endpoint is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the beneficial effect on a clinical endpoint. Failure to conduct required post-approval trials, or to confirm a clinical benefit during post-marketing trials, will allow the FDA to withdraw the approved biologic product from the market on an expedited basis. All promotional materials for biologic products approved under accelerated regulations are subject to prior review by the FDA.

In addition to other benefits such as the ability to use surrogate endpoints and engage in more frequent interactions with the FDA, the FDA may initiate review of sections of BLA with Fast Track designation before the application is complete. This is termed "rolling review" and is available if the applicant provides, and the FDA approves, a schedule for the submission of the outstanding BLA information and the applicant pays the applicable user fees. However, the FDA's performance goal for reviewing a BLA does not begin until the last section of the BLA is submitted. Additionally, the Fast Track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Breakthrough Therapy Designation

The FDA is also required to expedite the development and review of biological products that are intended to treat a serious or life-threatening disease or condition where preliminary clinical evidence indicates that the biologic product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. The sponsor of a new biologic product candidate may request that the FDA designate the candidate for a specific indication as a Breakthrough Therapy concurrent with, or after, the filing of the IND for the biologic product candidate. The FDA must determine if the biological product qualifies for Breakthrough Therapy designation within 60 days of receipt of the sponsor's request.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan drug designation to biological products intended to treat a rare disease or condition—generally a disease or condition that affects fewer than 200,000 individuals in the United States, or if it affects more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and making a product available in the United States for such disease or condition will be recovered from sales of the product.

Orphan drug designation must be requested before submitting a BLA. After the FDA grants orphan drug designation, the identity of the biological product and its potential orphan disease use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The first BLA applicant to receive FDA approval for a particular active moiety to treat a particular disease with FDA orphan drug designation is entitled to a seven-year exclusive marketing period in the United States for that product in the approved indication. During the seven-year marketing exclusivity period, the FDA may not approve any other applications to market a biological product containing the same active moiety for the same indication, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. A product can be considered clinically superior if it is safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity does not prevent the FDA from approving a different drug or biological product for the same disease or condition, or the same

biological product for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the BLA user fee.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA-regulated products, including biological products, are required to register and disclose certain clinical trial information on the website www.clintrials.gov. Information related to the product, patient population, phase of investigation, trial sites and investigators, and other aspects of a clinical trial are then made public as part of the registration. Sponsors are also obligated to share the results of the clinical trial after completion. Disclosure of the results of clinical trials can be delayed until the new product or new indication being studied has been approved. Competitors may use this publicly available information to gain knowledge regarding the progress of clinical development programs as well as clinical trial design.

Pediatric Information

Under the Pediatric Research Equity Act, or PREA, NDAs or BLAs or supplements to NDAs or BLAs must contain data to assess the safety and effectiveness of the biological product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the biological product is safe and effective. The FDA may grant full or partial waivers, or deferrals, for submission of data. Unless otherwise required by regulation, PREA does not apply to any biological product with orphan product designation.

Additional Controls for Biologics

To help reduce the increased risk of the introduction of adventitious agents, the PHSA emphasizes the importance of manufacturing controls for products whose attributes cannot be precisely defined. The PHSA also provides authority to the FDA to immediately suspend biologics licenses in situations where there exists a danger to public health, to prepare or procure products in the event of shortages and critical public health needs, and to authorize the creation and enforcement of regulations to prevent the introduction or spread of communicable diseases within the United States.

After a BLA is approved, the product may also be subject to official lot release as a condition of approval. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the lot manufacturing history and the results of all of the manufacturer's tests performed on the lot. The FDA may also perform certain confirmatory tests on lots of some products, such as viral vaccines, before allowing the manufacturer to release the lots for distribution. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products. As with drugs, after approval of a BLA, biologics manufacturers must address any safety issues that arise, are subject to recalls or a halt in manufacturing, and are subject to periodic inspection after approval.

Patent Term Restoration

After approval, owners of relevant biologic patents may apply for a patent term extension for a patent to include the regulatory review period. The allowable patent term extension is calculated as half of the drug's testing phase—the time from an IND application becoming effective to BLA submission—and all of the regulatory review phase—the time from BLA submission to approval, up to a maximum of five years of patent term restoration. The time can be shortened if FDA determines that the applicant did not pursue approval with appropriate due diligence. The total patent term after the extension may not exceed 14 years from the date of FDA approval of the BLA.

For patents that might expire during the BLA review phase, the patent owner may request an interim patent extension. An interim patent extension increases the patent term by one year and may be renewed up to four times. For each interim patent extension granted, the post-approval patent extension is reduced by one year.

The director of the United States Patent and Trademark Office must determine that approval of the drug or biologic covered by the patent for which a patent extension is being sought is likely. Interim patent extensions are not available for a biologic for which a BLA has not been submitted.

Biosimilars

The Biologics Price Competition and Innovation Act of 2009, or BPCIA, creates an abbreviated approval pathway for biological products shown to be highly similar to or interchangeable with an FDA-licensed reference biological product. Biosimilarity sufficient to reference a prior FDA-approved product requires that there be no differences in conditions of use, route of administration, dosage form, and strength, and no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency. Biosimilarity must be shown through analytical trials, animal trials, and a clinical trial or trials, unless the Secretary of Health and Human Services waives a required element. A biosimilar product may be deemed interchangeable with a previously approved product if it meets the higher hurdle of demonstrating 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. On March 6, 2015, the FDA approved the first biosimilar product under the BPCIA. Complexities associated with the larger, and often more complex, structures of biological products, as well as the process by which such products are manufactured, pose significant hurdles to biosimilar product implementation, which is still being evaluated by the FDA.

A reference biologic is granted 12 years of exclusivity from the time of first licensure, or BLA approval, of the reference product, and no application for a biosimilar can be submitted for four years from the date of licensure of the reference product. The first biologic product submitted under the biosimilar abbreviated approval pathway that is determined to be interchangeable with the reference product has exclusivity against a finding of interchangeability for other biologics for the same condition of use for the lesser of (i) one year after first commercial marketing of the first interchangeable biosimilar, (ii) 18 months after the first interchangeable biosimilar is approved if there is no patent challenge, (iii) eighteen months after resolution of a lawsuit over the patents of the reference biologic in favor of the first interchangeable biosimilar applicant, or (iv) 42 months after the first interchangeable biosimilar's application has been approved if a patent lawsuit is ongoing within the 42-month period.

Post-Approval Requirements

Once a BLA is approved, a product will be subject to certain post-approval requirements. For instance, the FDA closely regulates the post-approval marketing and promotion of biologics, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the Internet. Biologics may be marketed only for the approved indications and in accordance with the provisions of the approved labeling.

Adverse event reporting and submission of periodic safety summary reports is required following FDA approval of a BLA. The FDA also may require post-marketing testing, known as Phase 4 testing, REMS, and surveillance to monitor the effects of an approved product, or the FDA may place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control, biological product manufacture, packaging, and labeling procedures must continue to conform to cGMPs after approval. Biologic manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies. Registration with the FDA subjects entities to periodic unannounced inspections by the FDA, during which the agency inspects a biologic product's manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money, and effort in the areas of production and quality-control to maintain compliance with cGMPs. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with required regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

Other U.S. Healthcare Laws and Compliance Requirements

In the United States, our activities are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including but not limited to, the Centers for Medicare & Medicaid Services, or CMS, other divisions of the U.S. Department of Health and Human Services (e.g., the Office of Inspector General), the U.S. Department of Justice, or DOJ, and individual U.S. Attorney offices within the DOJ, and state and local governments. For example, sales, marketing and scientific/educational grant programs may have to comply with the anti-fraud and abuse provisions of the Social Security Act, the federal false claims laws, the privacy and security provisions of the Health Insurance Portability and Accountability Act, or HIPAA, and similar state laws, each as amended.

The federal Anti-Kickback Statute prohibits, among other things, any person or entity, from knowingly and willfully offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or in return for purchasing, leasing, ordering, recommending or arranging for the purchase, lease or order of any item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. The term remuneration has been interpreted broadly to include anything of value. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers, and/or formulary managers on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution. The exceptions and safe harbors are drawn narrowly and practices that involve remuneration that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. Our practices may not in all cases meet all of the criteria for protection under a statutory exception or regulatory safe harbor.

Additionally, the intent standard under the Anti-Kickback Statute was amended by the Patient Protection and Affordable Care Act, or the ACA, to a stricter standard such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, the ACA 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 federal False Claims Act (discussed below).

The civil monetary penalties statute imposes penalties against any person or entity who, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

Federal false claims laws, including the federal civil False Claims Act, prohibit, among other things, any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to, or approval by, the federal government or knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. As a result of a modification made by the Fraud Enforcement and Recovery Act of 2009, a claim includes “any request or demand” for money or property presented to the U.S. government. Recently, several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies’ marketing of the product for unapproved, and thus generally non-reimbursable, uses.

HIPAA created additional federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any money or property owned by, or under the control or custody of, any healthcare benefit

program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up by trick, scheme or device, a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services.

Also, many states have similar fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor. We may be subject to data privacy and security regulations by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, imposes requirements on certain types of people and entities relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH made HIPAA's security standards directly applicable to business associates, independent contractors or agents of covered entities that receive or obtain protected health information in connection with providing a service on behalf of a covered entity. HITECH also created four new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions. In addition, state laws govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Additionally, the federal Physician Payments Sunshine Act within the ACA, and its implementing regulations, require that certain manufacturers of drugs, devices, biological and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) report annually information related to certain payments or other transfers of value made or distributed to physicians and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, the physicians and teaching hospitals and to report annually certain ownership and investment interests held by physicians and their immediate family members.

In order to distribute products commercially, we must comply with state laws that require the registration of manufacturers and wholesale distributors of drug and biological products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. Several states have enacted legislation requiring pharmaceutical and biotechnology companies to establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing certain physician prescribing data to pharmaceutical and biotechnology companies for use in sales and marketing, and to prohibit certain other sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws.

If our operations are found to be in violation of any of the federal and state healthcare laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including without limitation, civil, criminal and/or administrative penalties, damages, fines, disgorgement, exclusion from participation in government programs, such as Medicare and Medicaid, injunctions, private "qui tam" actions brought by individual whistleblowers in the name of the government, or refusal to allow us to enter into government contracts, contractual damages, reputational harm, administrative burdens, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend, in part, on the extent to which third-party payors provide coverage, and establish adequate reimbursement levels for such products. In the United States, third-party payors include federal and state healthcare programs, private managed care providers, health insurers and other organizations. The process for determining whether a third-party payor will provide coverage for a product may be separate from the process for setting the price of a product or for establishing the reimbursement rate that such a payor will pay for the product. Third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the FDA-approved products for a particular indication. Third-party payors are increasingly challenging the price, examining the medical necessity and reviewing the cost-effectiveness of medical products, therapies and services, in addition to questioning their safety and efficacy. We may need to conduct expensive pharmaco-economic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain the FDA approvals. Our product candidates may not be considered medically necessary or cost-effective. A payor's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Further, one payor's determination to provide coverage for a product does not assure that other payors will also provide coverage for the product. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

Different pricing and reimbursement schemes exist in other countries. In the EU, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular product candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on health care costs has become very 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 a commercial pressure on pricing within a country.

The marketability of any product candidates for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on healthcare pricing. 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.

Healthcare Reform

In March 2010, President Obama enacted the ACA, which has begun to substantially change healthcare financing and delivery by both governmental and private insurers, and has also begun to significantly impact the pharmaceutical and biotechnology industry. The ACA will impact existing government healthcare programs and will result in the development of new programs.

Among the ACA provisions of importance to the pharmaceutical and biotechnology industries, in addition to those otherwise described above, are the following:

- an annual, nondeductible fee on any entity that manufactures or imports certain specified branded prescription drugs and biologic agents apportioned among these entities according to their market share in some government healthcare programs, that began in 2011;

- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program, retroactive to January 1, 2010, to 23.1% and 13% of the average manufacturer price for most branded and generic drugs, respectively and capped the total rebate amount for innovator drugs at 100% of the Average Manufacturer Price, or AMP;

- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturers' outpatient drugs to be covered under Medicare Part D;

- extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;

- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals beginning in 2014 and by adding new mandatory eligibility categories for individuals with income at or below 133% of the federal poverty level, thereby potentially increasing manufacturers' Medicaid rebate liability;

- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program; and

- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

We anticipate that the ACA will result in additional downward pressure on coverage and the price that we receive for any approved product, and could seriously harm our business. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products. In addition, it is possible that there will be further legislation or regulation that could harm our business, financial condition and results of operations.

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 Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. We believe that we are in material compliance with applicable environmental laws and that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations.

European Union and the Rest of the World Government Regulation

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Whether or not we obtain FDA approval of a product, we 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 CTA much like the IND prior to the commencement of human clinical trials. In the European Union, or EU, for example, a CTA must be submitted to each country's national health authority and an

independent ethics committee, much like the FDA and IRB, respectively. Once the CTA is approved in accordance with a country's requirements, clinical trial development may proceed. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

The requirements and process 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 GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational drug or biological product under EU regulatory systems, we must submit a marketing authorization application. The application used to file the BLA in the United States is similar to that required in the EU, with the exception of, among other things, country-specific document requirements.

For other countries outside of the EU, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we or our potential collaborators fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Legal Proceedings

We are not currently a party to any pending legal proceedings. From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. Regardless of outcome, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, negative publicity and reputational harm and other factors.

Facilities

We currently occupy 21,596 square feet of office space in San Francisco, California, under a lease that expires in June 2022. Additionally, we have subleased 21,960 square feet of cGMP manufacturing and laboratory space in South San Francisco, California, under a sublease that expires in May 2017. We exercised an option to enter into a ten-year lease for approximately 17,000 additional square feet of contiguous manufacturing space; the ten-year lease will become effective in June 2017. Additionally, we have subleased 8,983 square feet of research and development laboratory space in South San Francisco, California, under a sublease that expires in January 2018.

Employees

As of May 31, 2016, we had 75 full-time employees, including 15 employees with M.D. or Ph.D. degrees, and one part-time employee, who holds a Ph.D. degree. Of our workforce, 62 employees are engaged in research and development activities and 13 employees are engaged in finance, legal, human resources and general management activities. None of our employees are represented by a labor union or covered by a collective bargaining agreement. We consider our relationship with our employees to be good.

MANAGEMENT

The following table provides information regarding our executive officers and directors as of May 31, 2016:

Name	Age	Position
Executive Officers:		
Matthew Patterson	45	President, Chief Executive Officer and Director
Natalie Holles	43	Senior Vice President, Chief Operating Officer
Thomas Soloway	49	Senior Vice President, Chief Financial Officer
Suyash Prasad, M.B.B.S., F.F.P.M.	46	Senior Vice President and Chief Medical Officer
Mary Newman	57	Senior Vice President, Regulatory Affairs
David Nagler	63	Senior Vice President, Human Resources and Corporate Affairs
John Gray, Ph.D.	53	Senior Vice President, Research and Development
Non-Employee Directors:		
Jonathan Silverstein(1)	49	Chairman of Board of Directors
Louis Lange, M.D., Ph.D.	68	Director
Jonathan Leff	47	Director
Scott Morrison(2)	58	Director
Kush Parmar, M.D., Ph.D.(1)(3)	35	Director
Thomas Schuetz, M.D., Ph.D.(2)	55	Director
Stephen Squinto, Ph.D.(3)	59	Director
Thomas Woiwode, Ph.D.(2)(3)	44	Director

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

Executive Officers

Matthew Patterson is one of our co-founders and has served as our President and Chief Executive Officer and a member of our board of directors since our inception in November 2012. Mr. Patterson also served as our Chief Financial Officer and Secretary from December 2012 to September 2015. Previously, Mr. Patterson was the Entrepreneur-In-Residence at OrbiMed Advisors LLC, an investment firm and one of our principal stockholders, from November 2011 to December 2012. Prior to OrbiMed, from December 2004 to August 2011, Mr. Patterson worked for Amicus Therapeutics, Inc., a rare disease biotechnology company, most recently serving as President and Acting Chief Executive Officer. Prior to Amicus, Mr. Patterson worked at BioMarin Pharmaceutical Inc. from 1998 to 2004 and at Genzyme Corporation from 1993 to 1998. Mr. Patterson is a member of the Board of Directors of Gilda's Club of New York City, which provides social and emotional support for people living with cancer. Mr. Patterson holds a B.A. from Bowdoin College. Our board of directors believes that Mr. Patterson should serve as a director due to the perspective he brings as our founder and his expertise in the fields of business, biotechnology and drug development.

Natalie Holles has served as our Senior Vice President, Chief Operating Officer since August 2015. Previously, Ms. Holles served as Senior Vice President, Corporate Development at Hyperion Therapeutics, Inc., a rare disease pharmaceutical company, from June 2013 through its acquisition by Horizon Pharma, plc in May 2015. From August 2012 until June 2013, Ms. Holles served as the Executive Vice President, Corporate Development at Immune Design, Inc., an immunotherapy company, and from December 2010 to June 2013, Ms. Holles served as an independent life sciences corporate development consultant. Earlier in her career, Ms. Holles served as the Vice President, Business Development at KAI Pharmaceuticals, Inc. and previously held corporate development and commercial roles at InterMune, Inc. and Genentech, Inc. Ms. Holles holds an A.B. from Stanford University and an M.A. from the University of Colorado, Boulder.

Thomas Soloway has served as our Senior Vice President, Chief Financial Officer since September 2015. Prior to joining us, Mr. Soloway served as the Senior Vice President, Chief Financial Officer of Ascendis Pharma A/S, a Danish biopharmaceutical company, from January 2014 until September 2015. Prior to Ascendis, Mr. Soloway co-founded Transcept Pharmaceuticals, Inc., a biotechnology company, in 2002. At Transcept, Mr. Soloway held various positions, including Chief Financial Officer and Executive Vice President, Chief Operating Officer. Prior to joining Transcept, Mr. Soloway financed and advised early stage healthcare and life sciences companies as a Principal at Montreux Equity Partners. Mr. Soloway holds a B.S. from the University of Southern California and an M.B.A. from Georgetown University.

Suyash Prasad, M.B.B.S., F.F.P.M., has served as our Senior Vice President and Chief Medical Officer since February 2014. Prior to joining us, Dr. Prasad served as Senior Group Medical Director, Development Sciences at BioMarin Pharmaceutical, Inc., a rare genetic disease biotechnology company, from December 2010 to February 2014. Prior to joining BioMarin, Dr. Prasad served as the Director Global Medical Affairs, Personalized Genetic Health at Genzyme Corporation, a genetic disease biotechnology company, from January 2009 to December 2010 and in a country Medical Director role at Genzyme prior to that. He has also served as a senior clinical research physician at Eli Lilly. Prior to these roles, Dr. Prasad worked as a Pediatrician at Pediatric centers of excellence in the UK and Australia. Dr. Prasad is also an elected Fellow to the Faculty of Pharmaceutical Medicine of the Royal College of Physicians, UK. Dr. Prasad holds a degree from the University of Newcastle-upon-Tyne, United Kingdom and a Diploma from the Faculty of Pharmaceutical Medicine of the Royal Colleges of Physicians of the United Kingdom. Dr. Prasad is a United Kingdom board certified physician and is a member of the Royal College of Physicians and the Royal College of Paediatrics and Child Health.

Mary Newman has served as our Senior Vice President, Regulatory Affairs since October 2014. Prior to joining us, Ms. Newman held various positions at SARcode Biotherapeutics Inc., a biotechnology company, from July 2007 to April 2013, including as the Senior Vice President, Regulatory Affairs and Quality. She has also served in various management roles at BioMarin Pharmaceutical, Inc., Berlex Inc. (now Bayer HealthCare Pharmaceuticals Inc.) and Sequus Pharmaceuticals, Inc. (now Johnson and Johnson). Ms. Newman holds a B.S. from Oregon State University.

David Nagler has served as our Senior Vice President, Human Resources and Corporate Affairs since February 2015. Prior to joining us, he served as a human resources and corporate development consultant from April 2013 to February 2015. From July 2003 to March 2013, Mr. Nagler served as the Vice President Corporate Affairs at ARYx Therapeutics, Inc., a biotechnology company. He has also served as the Vice President Human Resources at Genentech, Inc. Mr. Nagler has served on the board of directors of U.C. Davis CONNECT, as well as the boards of the Northern California Chapter of the American Liver Foundation and the John Vasconcellos Legacy Project. Mr. Nagler studied at the University of California, Berkeley.

John Gray, Ph.D., has served as our Senior Vice President, Research and Development since December 2015, and as our Vice President, Research and Development from July 2014 to December 2015. Prior to joining us, Dr. Gray served as the Director of Vector Production and Development at St. Jude Children's Research Hospital from June 2003 to June 2014. Prior to St. Jude Children's Research Hospital, Dr. Gray served as the Assistant Director of the Harvard Gene Therapy Initiative and as a researcher at Pfizer Animal Health. Dr. Gray holds a B.A. from the University of California, Berkeley and a Ph.D. from the University of Colorado, Boulder.

Non-Employee Directors

Jonathan Silverstein has served as the chairman of our board of directors since December 2012. Mr. Silverstein is currently a general partner at OrbiMed, a healthcare investment firm, where he has worked since December 1998. Previously, Mr. Silverstein was a director of life sciences in the investment banking department at Sumitomo Bank. Mr. Silverstein serves on the board of directors of Intercept Pharmaceuticals, Inc., Roka Biosciences Inc., Glaukos Corp and Ascendis Pharma A/S. Mr. Silverstein also serves on the boards of directors of several private companies. Mr. Silverstein holds a B.A. from Denison University and a J.D. and

M.B.A. from the University of San Diego. Our board of directors believes that Mr. Silverstein's strategic development and capital markets experience qualifies him to serve on our board of directors.

Louis Lange, M.D., Ph.D. has served as a member of our board of directors since August 2015. Dr. Lange is currently a general partner at Asset Management Ventures, an investment firm, where he has worked since June 2009. Dr. Lange was the co-founder and served as the President and Chief Executive Officer of Cardiogen Sciences, Inc., a biotechnology company, from April 2014 until it was acquired by us in August 2015. Dr. Lange also co-founded CV Therapeutics, Inc. in 1990 and served as the Chairman, Chief Executive Officer and Chief Scientific Officer until it was acquired by Gilead Sciences, Inc. in 2009. Dr. Lange has also served as the Chief of Cardiology and Professor of Medicine at Jewish Hospital at Harvard University and Washington University. Dr. Lange served on the board of directors of Maxygen, Inc. from December 2005 to August 2013, CymaBay Therapeutics, Inc. from November 2003 to October 2015, and Esperion Therapeutics, Inc. from February 2010 to May 2014. Dr. Lange also serves as a member of the Board of Trustees at the University of Rochester, The Gladstone Foundation, is a senior advisor to Gilead and was on the board of directors of BIO (the trade organization of biotech companies) from 1998 to 2009, as well as other private companies. Dr. Lange holds a B.A. from the University of Rochester, an M.D. from Harvard Medical School and a Ph.D. from Harvard University. Our board of directors believes that Dr. Lange's deep experience in molecular cardiology and biotechnology business development qualifies him to serve on our board of directors.

Jonathan Leff has served as a member of our board of directors since November 2014. Mr. Leff is currently a partner at Deerfield Management Company, L.P., an investment firm, where he has worked since January 2013. Previously, Mr. Leff was a managing director at Warburg Pincus, a private equity investment firm where he worked from July 1996 to December 2012. Mr. Leff serves on the board of Nivalis Therapeutics, Inc. and previously served on the boards of directors of Talon Therapeutics, Inc., Allos Therapeutics, Inc., Inspire Pharmaceuticals, Inc., InterMune, Inc. and Sophiris Bio Inc. Mr. Leff also serves on the boards of directors of several private companies. Mr. Leff holds an A.B. from Harvard University and an M.B.A. from Stanford University Graduate School of Business. Our board of directors believes that Mr. Leff understanding of financial investment and business development in our industry qualifies him to serve on our board of directors. Mr. Leff has informed us that he intends to resign from our board of directors prior to the effectiveness of the registration statement of which this prospectus is a part.

Scott Morrison has served as a member of our board of directors since December 2015. From 1996 to December 2015, Mr. Morrison was a partner with Ernst & Young LLP, a public accounting firm, where he also served as U.S. Life Sciences Leader from 2002 to December 2015. Mr. Morrison has held roles on the boards of directors of numerous life sciences industry organizations. Since 1999, he has served on the board of directors of the Biotechnology Institute, where has also served on the audit committee since 2002. Mr. Morrison has previously served on the boards of directors of the Life Sciences Foundation, the Bay Area Biosciences Association and the Emerging Companies Section of the Biotechnology Industry Organization. He holds a B.S. from the University of California-Berkeley and is a certified public accountant (inactive). Our board of directors believes that Mr. Morrison's extensive experience in public accounting and the life sciences industry qualifies him to serve on our board of directors.

Kush Parmar, M.D., Ph.D., has served as a member of our board of directors since July 2013. Dr. Parmar is currently a managing partner at 5AM Ventures, a venture capital firm, where he has worked since June 2010. Previously, Dr. Parmar was a National Institute of Health Physician Scientist Fellow at Harvard Medical School, completed clinical clerkships at the Massachusetts General & Brigham and Women's Hospitals and consulted for an oncology startup. Dr. Parmar currently serves as on the board of directors of several private companies. He is also a Fellow of the Society of Kauffman Fellows. He holds an A.B. from Princeton University, a Ph.D. from Harvard University and an M.D. from Harvard Medical School. Our board of directors believes that Dr. Parmar's significant experience in advising biotechnology companies qualifies him to serve on our board of directors.

Thomas Schuetz, M.D., Ph.D., is our co-founder and has served as a member of our board of directors since July 2013. Dr. Schuetz is currently the Chief Executive Officer of Compass Therapeutics, LLC, a biotechnology company, where he has worked since July 2014. Previously, Dr. Schuetz was a consultant in the biotechnology industry from May 2012 to June 2014, including a consultant for us from July 2012 to June 2013. Prior to consulting, Dr. Schuetz was a venture partner at OrbiMed, a healthcare investment firm, where he worked from November 2007 to May 2012. Dr. Schuetz has also served as the Chief Medical Officer of Therion Biologics Corporation and the Vice President of Clinical Affairs at Transkaryotic Therapies, Inc. (now Shire Pharmaceuticals, Inc.). Dr. Schuetz has served as the Chief Medical Resident at Massachusetts General Hospital and completed a medical oncology fellowship at the Dana-Farber Cancer Institute. Dr. Schuetz also serves on the board of directors of Relypsa, Inc. and a private company. Dr. Schuetz holds a B.S. from Xavier University, an M.D. from Harvard Medical School and a Ph.D. from Harvard University. Dr. Schuetz is Board Certified in Medical Oncology. Our board of directors believes that Dr. Schuetz's clinical and executive experience and medical background qualify him to serve on our board of directors.

Stephen Squinto, Ph.D., has served as a member of our board of directors since April 2015. Dr. Squinto is currently a venture partner at OrbiMed, a healthcare investment firm, where he has worked since January 2015. Previously, Dr. Squinto co-founded Alexion Pharmaceuticals Inc., a rare disease biotechnology company, and served in various roles from April 1992 to December 2014, including as its Executive Vice President and Chief Global Operations Officer. Dr. Squinto has also held various positions at Regeneron Pharmaceuticals, Inc. and a joint academic position at both the Tulane University and Louisiana State University Medical Schools. Dr. Squinto holds a B.A. and a Ph.D. from Loyola University of Chicago. Our board of directors believes that Dr. Squinto's experience with rare disease research and development qualifies him to serve on our board of directors.

Thomas Woiwode, Ph.D., has served as a member of our board of directors since July 2013. Dr. Woiwode has been with Versant Ventures since 2002 in various capacities, serving as a venture partner since June 2011 and a managing director since July 2014. He has served in a number of operating roles over this time, most recently as the Chief Operating Officer of Okairos. Dr. Woiwode also co-founded EuroVentures, a wholly owned biotech incubator within Versant Ventures, and in this role, served as the founding Chief Business Officer for three biotech companies created within Versant. Dr. Woiwode also served as a research scientist at XenoPort, Inc. prior to joining Versant Ventures. Dr. Woiwode serves on the board of directors of several private companies. Dr. Woiwode holds a B.A. and a B.S. from the University of California, Berkeley and a Ph.D. from Stanford University. Our board of directors believes that Dr. Woiwode's experience with biotechnology company development and strategic planning qualifies him to serve on our board of directors.

Election of Officers

Our executive officers are elected by, and serve at the discretion of, our board of directors. There are no family relationships among any of our directors or executive officers.

Board of Directors

Our board of directors may establish the authorized number of directors from time to time by resolution. Our board of directors currently consists of nine members and will consist of eight members upon Mr. Leff's resignation prior to the effectiveness of the registration statement of which this prospectus is a part. Our current certificate of incorporation and voting agreement among certain investors provide for one director to be elected by holders of our common stock, three directors to be elected by specific holders of our Series A convertible preferred stock, one director to be elected by a specific holder of our Series B convertible preferred stock and all other directors to be elected by the holders of our common stock and of every other class or series of voting stock (including all convertible preferred stock) voting together as a single class on an as-converted to common stock basis. Dr. Parmar and Messrs. Silverstein and Woiwode are the designees of our Series A convertible preferred stock, Mr. Leff is the designee of our Series B convertible preferred stock, Mr. Patterson is the designee of our common stock and Drs. Schuetz and Squinto and Mr. Morrison are designees of our common stock and preferred stock voting together.

The voting agreement and the provisions of our certificate of incorporation by which Messrs. Leff, Morrison, Patterson and Silverstein and Drs. Parmar, Schuetz, Squinto and Woiwode were elected will terminate in connection with our initial public offering and there will be no contractual obligations regarding the election of our directors.

Dr. Lange was the President and Chief Executive Officer of Cardiogen Sciences, Inc., or Cardiogen, and was elected to our board of directors in connection with our acquisition of Cardiogen in August 2015.

Each of our current directors will continue to serve until the election and qualification of his or her successor, or his or her earlier death, resignation or removal.

Classified Board of Directors

Our restated certificate of incorporation and restated bylaws that will be in effect immediately prior to the completion of this offering provide for a classified board of directors consisting of three classes of directors, each serving staggered three-year terms. As a result, one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms. Our directors will be divided among the three classes as follows.

- the Class I directors will be Messrs. Silverstein, Woiwode and Schuetz, and their terms will expire at the first annual meeting of stockholders following this offering;
- the Class II directors will be Drs. Parmar, Squinto and Lange, and their terms will expire at the second annual meeting of stockholders following this offering; and
- the Class III directors will be Messrs. Morrison and Patterson, and their terms will expire at the third annual meeting of stockholders following this offering.

Each director's term continues until the election and qualification of his or her successor, or his or her earlier death, resignation or removal. Our restated certificate of incorporation and restated bylaws that will be in effect immediately prior to the completion of this offering will authorize only our board of directors to fill vacancies on our board of directors. Any increase or decrease in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors.

The classification of our board of directors may have the effect of delaying or preventing changes in control of our company. See "Description of Capital Stock—Anti-Takeover Provisions—Restated Certificate of Incorporation and Restated Bylaws Provisions."

Director Independence

In connection with this offering, we have applied to list our common stock on The NASDAQ Global Market, or NASDAQ. Under NASDAQ rules, independent directors must comprise a majority of a listed company's board of directors within a specified period of the completion of this offering. In addition, NASDAQ rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent. Under NASDAQ rules, a director will only qualify as an "independent director" if, in the opinion of that company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Securities Exchange Act of 1934, as amended. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of

the audit committee, the board of directors or any other board committee: (i) accept, directly or indirectly, any consulting, advisory or other compensatory fee from the listed company or any of its subsidiaries; or (ii) be an affiliated person of the listed company or any of its subsidiaries.

Our board of directors has undertaken a review of the independence of each director and considered whether each director has a material relationship with us that could compromise his ability to exercise independent judgment in carrying out his responsibilities. As a result of this review, our board of directors determined that Messrs. Leff, Morrison and Silverstein and Drs. Lange, Parmar, Schuetz, Squinto and Silverstein, representing eight of our nine directors, are "independent directors" as defined under the applicable rules and regulations of the Securities and Exchange Commission, or the SEC, and the listing requirements and rules of NASDAQ. In making these determinations, our board of directors reviewed and discussed information provided by the directors and us with regard to each director's business and personal activities and relationships as they may relate to us and our management, including the beneficial ownership of our capital stock by each non-employee director and the transactions involving them described in the section entitled "Certain Relationships and Related-Party Transactions."

Committees of Our Board of Directors

Our board of directors has an audit committee, a compensation committee and a nominating and corporate governance committee, each of which will have the composition and responsibilities described below as of the closing of our initial public offering. Members serve on these committees until their resignation or until otherwise determined by our board of directors. Each committee will operate under a charter approved by our board of directors. Following this offering, copies of each committee's charter will be posted on the investor relations section of our website at www.audentestx.com.

Audit Committee

Our audit committee is composed of Messrs. Morrison, Schuetz and Woiwode. Mr. Morrison is the chairperson of our audit committee. Messrs. Morrison, Schuetz and Woiwode each meet the requirements for independence under the current NASDAQ listing standards and SEC rules and regulations. Each member of our audit committee is financially literate. In addition, our board of directors has determined that Mr. Morrison is an "audit committee financial expert" as defined in Item 407(d)(5)(ii) of Regulation S-K promulgated under the Securities Act. This designation does not impose any duties, obligations or liabilities that are greater than are generally imposed on members of our audit committee and our board of directors. Our audit committee is responsible for, among other things:

- our accounting and financial reporting processes, including our financial statement audits and the integrity of our financial statements;
- our compliance with legal and regulatory requirements;
- reviewing and approving related person transactions;
- selecting and hiring our registered independent public accounting firm;
- the qualifications, independence and performance of our independent auditors; and
- the preparation of the audit committee report to be included in our annual proxy statement.

Compensation Committee

Our compensation committee is composed of Messrs. Squinto, Parmar and Woiwode. Mr. Squinto is the chairperson of our compensation committee. The composition of our compensation committee meets the requirements for independence under the current NASDAQ listing standards and SEC rules and regulations. Each member of this committee is (i) an outside director, as defined pursuant to Section 162(m) of the Internal

Revenue Code of 1986, as amended, or the Code, and (ii) a non-employee director, as defined in Rule 16b-3 promulgated under the Exchange Act. Our compensation committee is responsible for, among other things:

- evaluating, recommending, approving and reviewing executive officer and director compensation arrangements, plans, policies and programs;
- administering our cash-based and equity-based compensation plans; and
- making recommendations to our board of directors regarding any other board of director responsibilities relating to executive compensation.

Nominating and Corporate Governance Committee

Our nominating and corporate governance committee is composed of Messrs. Silverstein and Parmar. Mr. Silverstein is the chairperson of our nominating and corporate governance committee. The composition of our nominating and corporate governance committee meets the requirements for independence under the current NASDAQ listing standards and SEC rules and regulations. Our nominating and corporate governance committee is responsible for, among other things:

- identifying, considering and recommending candidates for membership on our board of directors;
- overseeing the process of evaluating the performance of our board of directors; and
- advising our board of directors on other corporate governance matters.

Compensation Committee Interlocks and Insider Participation

None of our executive officers has served as a member of the board of directors, or as a member of the compensation or similar committee, of any entity that has one or more executive officers who served on our board of directors or compensation committee during the year ended December 31, 2015.

Codes of Business Conduct and Ethics

Our board of directors has adopted a code of business conduct and ethics that will become effective upon completion of this offering that applies to all of our employees, officers and directors, including our Chief Executive Officer, Chief Financial Officer and other executive and senior financial officers. The full text of our code of conduct will be posted on the investor relations section of our website at www.audentestx.com. The reference to our website address in this prospectus does not include or incorporate by reference the information on our website into this prospectus. We intend to disclose future amendments to certain provisions of our code of conduct, or waivers of these provisions, on our website or in public filings to the extent required by the applicable rules and exchange requirements.

Non-Employee Director Compensation

The following table presents the total compensation for each person who served as a non-employee member of our board of directors in the year ended December 31, 2015. Other than as set forth in the table, in the year ended December 31, 2015, we did not pay any fees to, make any equity awards or non-equity awards to or pay any other compensation to the non-employee members of our board of directors. Mr. Patterson, our Chief Executive Officer, received no compensation for his service as a director in the year ended December 31, 2015.

Name[1]	Fees Earned or Paid in Cash	Option Awards[2]	Total
Scott Morrison	$ —	$ 192,746	$ 192,746
Thomas Schuetz	31,000	8,620	39,620
Stephen Squinto	24,750	34,511	59,261

(1) Louis Lange, Jonathan Leff, Kush Parmar, Jonathan Silverstein and Thomas Woiwode also served as non-employee members of our board of directors in 2015. None of these directors were paid any compensation during 2015, nor did they hold any outstanding options to purchase shares of common stock as of December 31, 2015. As of December 31, 2015, Mr. Morrison held outstanding options to purchase 33,635 shares of common stock with an exercise price of $9.50 per share, Dr. Schuetz held outstanding options to purchase 18,086 shares of common stock with an exercise price of $0.79 per share and options to purchase 5,605 shares of common stock at an exercise price of $2.19 per share, and Dr. Squinto held outstanding options to purchase 22,423 shares of common stock with an exercise price of $2.19 per share.

(2) The amounts reported in the Option Awards column represent the grant date fair value of the stock options granted to the directors during the year ended December 31, 2015 as computed in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic 718. The assumptions used in calculating the grant date fair value of the stock options reported in the Option Awards column are set forth in Note 10 to our audited consolidated financial statements included in this prospectus. Note that the amounts reported in this column reflect the accounting cost for these stock options, and do not correspond to the actual economic value that may be received by our directors from the options.

In May 2015, we entered into an offer letter with Dr. Schuetz in connection with the termination of his consulting services, and in April 2015, we entered into an offer letter with Dr. Squinto in connection with his appointment to our board of directors. Pursuant to the offer letters, Drs. Schuetz and Squinto received options to purchase 5,605 and 22,423 shares of common stock, respectively, which vest in 12 equal quarterly installments, subject to accelerated vesting upon a change in control of our company. In connection with Mr. Morrison's appointment to our board of directors in December 2015, Mr. Morrison received an option to purchase 33,635 shares of common stock, which vests in 12 equal quarterly installments, subject to accelerated vesting upon a change in control of our company. Additionally, Drs. Schuetz and Squinto and Mr. Morrison are entitled to receive an annual cash retainer of $33,000.

In the future, we intend to adopt a formal policy regarding the compensation of all of our non-employee directors.

EXECUTIVE COMPENSATION

The following tables and accompanying narrative disclosure set forth information about the compensation provided to certain of our executive officers during the years ended December 31, 2014 and 2015. These executive officers, who include our principal executive officer and the two most highly-compensated executive officers (other than our principal executive officer) who were serving as executive officers as of December 31, 2015, the end of our last completed fiscal year, were:

- Matthew Patterson, President, Chief Executive Officer and Director;
- Suyash Prasad, M.D., Senior Vice President and Chief Medical Officer; and
- John Gray, Ph.D., Senior Vice President, Research and Development.

We refer to these individuals in this section as our "Named Executive Officers."

Summary Compensation Table

The following table presents summary information regarding the total compensation that was awarded to, earned by or paid to our Named Executive Officers for services rendered during the years ended December 31, 2014 and 2015.

Name and Principal Position	Year	Salary	Bonus	Option Award[1]	Non-Equity Incentive Plan Compensation[2]	All Other Compensation	Total
Matthew Patterson	2015	$416,000	$ —	$1,322,186	$101,920	$ —	$1,840,106
President, Chief Executive Officer and Director	2014	341,250	—	—	92,138	—	433,388
Suyash Prasad, M.D.[3]	2015	340,000	2,500[4]	312,545	59,500	—	714,545
Senior Vice President and Chief Medical Officer	2014	278,666	90,000[5]	56,610	65,500	—	490,776
John Gray, Ph.D.	2015	275,000	11,500[4]	357,952	38,500	85,239[6]	768,191
Senior Vice President, Research and Development							

(1) The amounts reported in the Option Awards column represent the grant date fair value of the stock options granted to the Named Executive Officers during the years ended December 31, 2014 and 2015 as computed in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic 718. The assumptions used in calculating the grant date fair value of the stock options reported in the Option Awards column are set forth in Note 10 to our audited consolidated financial statements included in this prospectus. Note that the amounts reported in this column reflect the accounting cost for these stock options, and do not correspond to the actual economic value that may be received by our Named Executive Officers from the options.

(2) For additional information regarding the non-equity incentive plan compensation, see "—2015 Non-Equity Incentive Plan Awards and Bonuses."

(3) Dr. Prasad's employment with our company commenced in February 2014.

(4) Represents amounts awarded at the discretion of our board of directors for exceptional performance in 2015.

(5) Represents signing bonus paid to Dr. Prasad.

(6) Represents expenses paid by us in connection with Dr. Gray's relocation to San Francisco, California.

2015 Non-Equity Incentive Plan Awards and Bonuses

Annual bonuses for our executive officers are based on the achievement of corporate performance objectives, which in 2015 included the achievement of preclinical and business development milestones. In December 2015, based on the achievement of these corporate performance objectives, our board of directors

determined that approximately 70% of each executive officer's target bonus should be awarded. For 2015, Mr. Patterson's target bonus was equal to 35% of his annual base salary of $416,000, Dr. Prasad's target bonus was equal to 25% of his annual base salary of $340,000 and Dr. Gray's target bonus was equal to 20% of his annual base salary of $275,000. Additionally, our board of directors determined that Drs. Prasad and Gray should receive a slightly higher bonus for exceptional performance in 2015. Accordingly, Mr. Patterson and Drs. Prasad and Gray were awarded the 2015 annual bonuses reflected in the table above.

2015 Equity Awards

In February and May 2015, our board of directors granted Mr. Patterson and Drs. Prasad and Gray options to purchase 141,297, 24,217 and 17,939 shares of common stock, respectively, with an exercise price of $2.19 per share. Additionally, after a review of the equity held by our Named Executive Officers in comparison to equity held by executive officers of our peer companies, in December 2015 our board of directors granted Mr. Patterson and Drs. Prasad and Gray options to purchase 189,819, 47,454 and 56,945 shares of common stock, respectively, with an exercise price of $9.50 per share. All of these options vest quarterly over four years.

Outstanding Equity Awards at Fiscal Year-End Table

The following table presents, for each of the Named Executive Officers, information regarding outstanding stock options held as of December 31, 2015.

	Option Awards[1]			
Name	Number of Securities Underlying Unexercised Options Exercisable	Number of Securities Underlying Unexercised Options Unexercisable	Option Exercise Price	Option Expiration Date
Mr. Patterson	197,327[2]	89,695	$0.79	9/25/2023
	—	141,297[3]	2.19	2/04/2025
	—	189,819[5]	9.50	12/18/2025
Dr. Prasad	42,381[3]	54,489	0.79	2/18/2024
	3,531[4]	20,686	2.19	5/27/2025
	—	47,454[5]	9.50	12/18/2025
Dr. Gray	22,423[3]	49,333	1.05	9/17/2024
	2,615[4]	15,324	2.19	5/27/2025
	—	56,945[5]	9.50	12/18/2025

(1) All of the outstanding option awards were granted under our 2012 Equity Incentive Plan.

(2) Vests with respect to 25% of the shares underlying the option on the one year anniversary of the vesting commencement date and the remaining 75% of the shares underlying the option vest in 16 equal quarterly installments thereafter. If the Named Executive Officer is terminated without cause or resigns for good reason during the period beginning 90 days prior to and ending on the first anniversary of a change in control of our company, 100% of the then-unvested shares underlying the option will vest.

(3) Vests with respect to 25% of the shares underlying the option on the one year anniversary of the vesting commencement date and the remaining 75% of the shares underlying the option vest in 16 equal quarterly installments thereafter.

(4) Vests in equal monthly installments over four years.

(5) Vests in equal quarterly installments over four years. If the Named Executive Officer is terminated without cause or resigns for good reason during the period beginning 90 days prior to and ending on the first anniversary of a change in control of our company, 100% of the then-unvested shares underlying the option will vest.

Employment Agreements

We have entered into employment agreements with our Named Executive Officers that provide for at-will employment and include each Named Executive Officer's base salary, a discretionary annual incentive bonus opportunity and standard employee benefit plan participation. These agreements also provide for severance benefits upon termination of employment or a change in control of our company.

If a Named Executive Officer is terminated for "cause" or in the event of a Named Executive Officer's death, "disability" or voluntary separation from service at any time and for any reason, such Named Executive Officer will be entitled to receive (i) any earned but unpaid base salary, (ii) other unpaid vested amounts or benefits under the compensation, incentive and benefits plans we have in which such Named Executive Officers participates and (iii) reimbursement for all reasonable and necessary expenses incurred in connection with such Named Executive Officer's performance of services on our behalf in accordance with our applicable policies and guidelines, in each case as of the effective date of such separation from service. The compensation referred to in (i)-(iii) above is collectively referred to as Accrued Compensation.

If a Named Executive Officers is terminated without cause or resigns for "good reason," and such Named Executive Officer delivers to us a signed general release of claims, such Named Executive Officer will be entitled to receive (i) the Accrued Compensation, (ii) a lump sum cash payment in an amount equal to six months of such Named Executive Officer's base salary and (iii) reimbursement for any monthly COBRA premium payments for 12 months, subject to certain limitations.

If a Named Executive Officer is terminated without cause or resigns for good reason, in each case during the period of time commencing 90 days prior to the execution of a definitive agreement providing for the consummation of a change in control and ending on the first anniversary of the consummation of such change in control, and provided that such Named Executive Officer delivers to us a signed general release of claims, such Named Executive Officer will be entitled to receive (i) the Accrued Compensation, (ii) a lump sum cash payment in an amount equal to six months of his base salary, (iii) reimbursement for any monthly COBRA premium payments for the 12 months, subject to certain limitations, (iv) accelerated vesting of 100% of the unvested stock or equity awards granted to such Named Executive Officer pursuant to the terms of the employment agreement, if any, and (v) accelerated vesting of 100% of the unvested portion of any equity awards granted to such Named Executive Officer after the effective date of the employment agreement.

Under the employment agreements, "cause" generally means (i) failure to satisfactorily perform duties after there has been delivered a written demand for performance which describes the specific deficiencies in performance and the specific manner in which performance must be improved, and which provides 30 business days from the date of notice to remedy such performance deficiencies; (ii) conviction of or plea of nolo contendere to a felony or a crime involving moral turpitude which our board of directors believes has had or will have a detrimental effect on our reputation or business, (iii) engaging in an act of gross negligence or willful misconduct in the performance of employment obligations and duties, (iv) committing an act of fraud against, material misconduct or willful misappropriation of property belonging to us; (v) engaging in any other misconduct that has had or will have a material adverse effect on our reputation or business; or (vi) breach of the Employee Invention Assignment and Confidentiality Agreement or other unauthorized misuse of the our trade secrets or proprietary information.

Under the employment agreements, "change in control" means (i) a sale, conveyance, exchange or transfer (excluding any venture-backed or similar investments) in which any person or entity, other than persons or entities who as of immediately prior to such sale, conveyance, exchange or transfer own securities, either directly or indirectly, becomes the beneficial owner, directly or indirectly, of securities representing more than 50% of the total voting power of all its then outstanding voting securities; (ii) our merger or consolidation in which our voting securities immediately prior to the merger or consolidation do not represent, or are not converted into securities that represent, a majority of the voting power of all voting securities of the surviving

entity immediately after the merger or consolidation; or (iii) a sale of substantially all of our assets or our liquidation or dissolution.

Under the employment agreements, "disability" has the meaning set forth in Section 22(e)(3) of the Code.

Under the employment agreements, "good reason" means any of the following taken without the Named Executive Officer's written consent and provided (i) we receive, within 30 days following the occurrence of any of the events set forth in clauses (a) through (id) below, written notice from the Named Executive Officer specifying the specific basis for the Named Executive Officer's belief that the Named Executive Officer is entitled to terminate employment for Good Reason, (ii) we fail to cure the event constituting Good Reason within 30 days after receipt of such written notice of the event, and (iii) the Named Executive Officer terminates employment within the earlier of 10 days following expiration of such cure period or receipt from us that such deficiencies will not be cured: (a) a material change, adverse to the Named Executive Officer, in the Named Executive Officer's position, titles, offices or duties; (b) an assignment of any significant duties to the Named Executive Officer that are inconsistent with the Named Executive Officer's positions or offices held; (c) a decrease in base salary by more than 10% (other than in connection with a general decrease in the base salary of all other executive officers); or (d) relocation to a facility or a location more than 50 miles from the then-current location.

Employee Benefit and Stock Compensation Plans

2012 Equity Incentive Plan

Our board of directors adopted the 2012 Equity Incentive Plan, or the 2012 Plan, in December 2012 and our stockholders subsequently approved it in December 2012. We subsequently amended the 2012 Plan in July 2013, September 2014 and November 2014. Our board of directors, or a committee thereof appointed by our board of directors, administers the 2012 Plan and the awards granted under it. The plan administrator has the authority to modify outstanding awards under the 2012 Plan. The 2012 Plan provides for the grant of both incentive stock options, which qualify for favorable tax treatment to their recipients under Section 422 of the Code, and nonstatutory stock options, as well as for the issuance of shares of restricted stock awards, or RSAs, restricted stock units, or RSUs, and stock appreciation rights, or SARs. We may grant incentive stock options only to our employees and employees of our majority-owned subsidiaries. We may grant nonstatutory stock options, RSAs, RSUs and SARs to our employees, directors and consultants and employees, directors and consultants of our majority-owned subsidiaries. The exercise price of each stock option must be at least equal to the fair market value of our common stock on the date of grant, unless expressly determined in writing by the plan administrator. The exercise price of incentive stock options granted to 10% stockholders must be at least equal to 110% of the fair market value of our common stock on the date of grant. The maximum permitted term of options granted under the 2012 Plan is ten years, except that the maximum permitted term of incentive stock options granted to 10% stockholders is five years. In the event of an acquisition (as defined in the 2012 Plan), the 2012 Plan provides that, unless the applicable option agreement provides otherwise or our board of directors or compensation committee takes certain actions, such as accelerating the vesting of the awards or providing for the assumption, conversion or replacement of the option by an acquirer, awards held by current employees, directors and consultants will terminate if not vested or exercised prior to the effective time of the acquisition.

As of March 31, 2016, we had reserved 3,107,517 shares of our common stock for issuance under the 2012 Plan and 682,512 shares remained available for future grant. We will cease issuing awards under the 2012 Plan upon the implementation of the 2016 Equity Incentive Plan, or the 2016 Plan. The 2016 Plan will become effective on the date immediately prior to the date of this prospectus. As a result, we will not grant any additional options under the 2012 Plan following that date, and the 2012 Plan will terminate at that time. However, any outstanding options granted under the 2012 Plan will remain outstanding, subject to the terms of the 2012 Plan and stock option agreements, until such outstanding options are exercised or until they terminate or expire by

their terms. Options granted under the 2012 Plan have terms similar to those described below with respect to options to be granted under the 2016 Plan.

2016 Equity Incentive Plan

We adopted the 2016 Plan, which will become effective on the date immediately prior to the date of this prospectus and serve as the successor to the 2012 Plan. We reserved 1,500,000 shares of our common stock to be issued under the 2016 Plan. The number of shares reserved for issuance under the 2016 Plan will increase automatically on January 1 of each calendar, year continuing through the tenth calendar year during the term of the 2016 Plan by the number of shares equal to 5% of the total outstanding shares of our common stock as of the immediately preceding December 31. However, our board of directors may reduce the amount of the increase in any particular year. In addition, the following shares of our common stock will be available for grant and issuance under the 2016 Plan:

- shares subject to options or SARs granted under the 2016 Plan that cease to be subject to the option or SAR for any reason other than exercise of the option or SAR;
- shares subject to awards granted under the 2016 Plan that are subsequently forfeited or repurchased by us at the original issue price;
- shares subject to awards granted under the 2016 Plan that otherwise terminate without shares being issued;
- shares surrendered, cancelled, or exchanged for cash or a different award (or combination thereof);
- shares subject to awards under the 2016 Plan that are used to pay the exercise price of an award or withheld to satisfy the tax withholding obligations related to any award;
- shares reserved but not issued or subject to outstanding awards under the 2012 Plan on the date of this prospectus;
- shares issuable upon the exercise of options or subject to other awards under the 2012 Plan prior to the date of this prospectus that cease to be subject to such options or other awards by forfeiture or otherwise after the date of this prospectus;
- shares issued under the 2012 Plan that are repurchased by us at the original issuance price or forfeited after the date of this prospectus; and
- shares subject to awards under the 2012 Plan that are used to pay the exercise price of an option or withheld to satisfy the tax withholding obligations related to any award.

The 2016 Plan authorizes the award of stock options, RSAs, SARs, RSUs, performance awards and stock bonuses. No person will be eligible to receive more than 1,000,000 shares in any calendar year under the 2016 Plan other than a new employee of ours, who will be eligible to receive no more than 2,000,000 shares under the plan in the calendar year in which the employee commences employment. No participant will be eligible to receive more than $5.0 million in performance awards in any calendar year. No more than 10,000,000 shares will be issued pursuant to the exercise of incentive stock options. The aggregate number of shares of our common stock that may be subject to awards granted to any one non-employee director pursuant to the 2016 Plan in any calendar year shall not exceed 150,000.

The 2016 Plan will be administered by our compensation committee, all of the members of which are outside directors as defined under applicable federal tax laws, or by our board of directors acting in place of our

compensation committee. The compensation committee will have the authority to construe and interpret the 2016 Plan, grant awards and make all other determinations necessary or advisable for the administration of the plan.

The 2016 Plan provides for the grant of awards to our employees, directors, consultants, independent contractors and advisors, provided the consultants, independent contractors, directors and advisors render services not in connection with the offer and sale of securities in a capital-raising transaction. The exercise price of stock options must be at least equal to the fair market value of our common stock on the date of grant. The compensation committee has the authority to reprice any outstanding stock option or SAR (by reducing the exercise price of any outstanding option or SAR, canceling an option or SAR in exchange for cash or another equity award) under the 2016 Plan without the approval of our stockholders.

We anticipate that, in general, options will vest over a four-year period. Options may vest based on time or achievement of performance conditions. Our compensation committee may provide for options to be exercised only as they vest or to be immediately exercisable with any shares issued on exercise being subject to our right of repurchase that lapses as the shares vest. The maximum term of options granted under the 2016 Plan is 10 years, except that the maximum permitted term of incentive stock options granted to 10% stockholders is five years.

An RSA is an offer by us to sell shares of our common stock subject to restrictions, which may vest based on time or achievement of performance conditions. The price, if any, of an RSA will be determined by the compensation committee. Unless otherwise determined by the compensation committee at the time of award, vesting will cease on the date the holder no longer provides services to us and unvested shares will be forfeited to or repurchased by us.

SARs provide for a payment, or payments, in cash or shares of our common stock, to the holder based upon the difference between the fair market value of our common stock on the date of exercise and the stated exercise price at grant up to a maximum amount of cash or number of shares. SARs may vest based on time or achievement of performance conditions.

RSUs represent the right to receive shares of our common stock at a specified date in the future, subject to forfeiture of that right because of termination of employment or failure to achieve certain performance conditions. If an RSU has not been forfeited, then on the date specified in the RSU agreement, we will deliver to the holder of the RSU shares of our common stock (which may be subject to additional restrictions), cash or a combination of our common stock and cash. We anticipate that, in general, RSUs will vest over a four-year period.

Performance awards cover a number of shares of our common stock that may be settled upon achievement of the pre-established performance conditions as provided in the 2016 Plan in cash or by issuance of the underlying shares. These awards are subject to forfeiture prior to settlement due to termination of employment or failure to achieve the performance conditions.

Stock bonuses may be granted as additional compensation for past or future service or performance, and therefore, no payment will be required for any shares awarded under a stock bonus. Unless otherwise determined by the compensation committee at the time of award, vesting will cease on the date the holder no longer provides services to us and unvested shares (if any) will be forfeited to us.

The 2016 Plan permits the grant of performance-based stock and cash awards that may qualify as performance-based compensation that is not subject to the $1.0 million limitation on income tax deductibility imposed by Section 162(m) of the Code. Our compensation committee may structure awards so that the stock or cash will be issued or paid only following the achievement of certain pre-established performance goals during a designated performance period.

Awards granted under the 2016 Plan may not be transferred in any manner other than by will or by the laws of descent and distribution or as determined by our compensation committee. Unless otherwise permitted by

our compensation committee, stock options may be exercised during the lifetime of the optionee only by the optionee or the optionee's guardian or legal representative. Options granted under the 2016 Plan generally may be exercised for a period of three months after the termination of the optionee's service to us, for a period of 12 months in the case of death or disability, or such shorter period (not less than six months) or longer period as our compensation committee may provide. Options generally terminate immediately upon termination of employment for cause.

If we are party to a merger or consolidation, sale of all or substantially all assets or similar change in control transaction, outstanding awards, including any vesting provisions, may be continued, assumed or substituted by the successor company. In the alternative, outstanding awards may be cancelled in exchange for a payment in cash or securities of the successor entity or acquirer. Outstanding awards may also be cancelled in exchange for no consideration. Outstanding awards that are not converted, assumed, substituted or cashed out will accelerate in full and expire upon the closing of the transaction. Awards held by non-employee directors will immediately vest as to all or any portion of the shares subject to the stock award and will become exercisable at such times and on such conditions as the compensation committee determines.

The 2016 Plan will terminate ten years from the date our board of directors approved it, unless it is terminated earlier by our board of directors. Our board of directors may amend or terminate the 2016 Plan at any time. If our board of directors amends the 2016 Plan, it does not need to ask for stockholder approval of the amendment unless required by applicable law.

2016 Employee Stock Purchase Plan

We adopted a 2016 Employee Stock Purchase Plan, or the 2016 ESPP, in order to enable eligible employees to purchase shares of our common stock at a discount following the date of this offering. Purchases will be accomplished through participation in discrete offering periods. The 2016 ESPP is intended to qualify as an employee stock purchase plan under Section 423 of the Code. We have reserved 210,000 shares of our common stock for issuance under the 2016 ESPP. The number of shares reserved for issuance under the 2016 ESPP will increase automatically on January 1 of each calendar year beginning after the first offering date and continuing through the first ten calendar years by the number of shares equal to 1% of the total outstanding shares of our common stock as of the immediately preceding December 31. However, our board of directors or compensation committee may reduce the amount of the increase in any particular year. The aggregate number of shares issued over the term of the 2016 ESPP will not exceed 2,100,000 shares of our common stock.

Our compensation committee will administer the 2016 ESPP. Our employees generally are eligible to participate in the 2016 ESPP. Our compensation committee may in its discretion elect to exclude employees who do not meet certain eligibility requirements under applicable law. Employees who are 5% stockholders, or would become 5% stockholders as a result of their participation in the 2016 ESPP, are ineligible to participate in the 2016 ESPP. We may impose additional restrictions on eligibility. Under the 2016 ESPP, eligible employees will be able to acquire shares of our common stock by accumulating funds through payroll deductions. Our eligible employees will be able to select a rate of payroll deduction between 1% and 15% of their eligible cash compensation. We will also have the right to amend or terminate the 2016 ESPP at any time. The 2016 ESPP will terminate on the tenth anniversary of the first purchase date under the 2016 ESPP unless it is terminated earlier by our board of directors.

The 2016 ESPP is implemented through a series of offering periods under which our employees who meet the eligibility requirements for participation in that offering period will automatically be granted a nontransferable option to purchase shares in that offering period. For subsequent offering periods, new participants will be required to enroll in a timely manner. Once an employee is enrolled, participation will be automatic in subsequent offering periods. The time and duration of the offering periods and the purchase periods will be determined by the compensation committee, provided that an offering period will in no event be longer than 27 months, except as otherwise provided by an applicable subplan. Offering periods may be consecutive or

overlapping; purchase periods will be consecutive. Each offering period may consist of one or more purchase periods. The compensation committee will determine the duration and commencement date of each offering period and purchase period, provided that a purchase period will not end later than the close of the offering period in which it begins. An employee's participation automatically ends upon termination of employment for any reason.

No participant will have the right to purchase shares of our common stock in an amount, when aggregated with purchase rights under all our employee stock purchase plans that are also in effect in the same calendar year, that have a fair market value of more than $25,000, determined as of the first day of the applicable purchase period, for each calendar year in which that right is outstanding. In addition, no participant will be permitted to purchase more than 2,100 shares during any one purchase period or a lesser amount determined by our compensation committee. The purchase price for shares of our common stock purchased under the 2016 ESPP will be 85% of the lesser of the fair market value of our common stock on (i) the first trading day of the applicable offering period and (ii) the last trading day of each purchase period in the applicable offering period. The fair market value of our common stock for purposes of our first offering period under the 2016 ESPP will depend on the date on which the compensation committee first implements the 2016 ESPP.

If we experience a change in control transaction, any offering period that commenced prior to the closing of the proposed change in control transaction will be shortened and terminated on a new purchase date. The new purchase date will occur prior to the closing of the proposed change in control transaction and the 2016 ESPP will then terminate on the closing of the proposed change in control.

401(k) Plan

We sponsor a retirement plan intended to qualify for favorable tax treatment under Section 401(a) of the Code, containing a cash or deferred feature that is intended to meet the requirements of Section 401(k) of the Code. U.S. employees who have attained at least 21 years of age are generally eligible to participate in the plan concurrent with, or any time following their second payroll following the employees' date of hire, subject to certain eligibility requirements. Participants may make pre-tax contributions to the plan from their eligible earnings up to the statutorily prescribed annual limit on pre-tax contributions under the Code. Participants who are 50 years of age or older may contribute additional amounts based on the statutory limits for catch-up contributions. Pre-tax contributions by participants and the income earned on those contributions are generally not taxable to participants until withdrawn. Participant contributions are held in trust as required by law. No minimum benefit is provided under the plan. An employee's interest in his or her pre-tax deferrals is 100% vested when contributed. Although the plan provides for a discretionary employer matching contribution, to date we have not made such a contribution on behalf of employees. The Plan permits all eligible Plan participants to contribute between 1% and 100% of eligible compensation, on a pre-tax basis, into their accounts.

Limitations on Liability and Indemnification Matters

Our restated certificate of incorporation that will become effective in connection with the completion of this offering contains provisions that limit the liability of our directors for monetary damages to the fullest extent permitted by the Delaware General Corporation Law, or DGCL. Consequently, our directors will not be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duties as directors, except liability for:

- any breach of the director's duty of loyalty to us or our stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the DGCL; or

- any transaction from which the director derived an improper personal benefit.

Our restated certificate of incorporation and our restated bylaws that will become effective in connection with the completion of this offering require us to indemnify our directors and officers to the maximum extent not prohibited by the DGCL and allow us to indemnify other employees and agents as set forth in the DGCL. Subject to certain limitations, our restated bylaws also require us to advance expenses incurred by our directors and officers for the defense of any action for which indemnification is required or permitted.

We have entered, and intend to continue to enter, into separate indemnification agreements with our directors, officers and certain of our key employees, in addition to the indemnification provided for in our restated certificate of incorporation and restated bylaws. These agreements, among other things, require us to indemnify our directors, officers and key employees for certain expenses, including attorneys' fees, judgments, penalties, fines and settlement amounts actually incurred by these individuals in any action or proceeding arising out of their service to us or any of our subsidiaries or any other company or enterprise to which these individuals provide services at our request. Subject to certain limitations, our indemnification agreements also require us to advance expenses incurred by our directors, officers and key employees for the defense of any action for which indemnification is required or permitted.

We believe that provisions of our restated certificate of incorporation, bylaws and indemnification agreements are necessary to attract and retain qualified directors, officers and key employees. We also maintain directors' and officers' liability insurance.

The limitation of liability and indemnification provisions in our restated certificate of incorporation and restated bylaws may discourage stockholders from bringing a lawsuit against our directors and officers for breach of their fiduciary duty. They may also reduce the likelihood of derivative litigation against our directors and officers, even though an action, if successful, might benefit us and other stockholders. Further, a stockholder's investment may be adversely affected to the extent that we pay the costs of settlement and damage awards against directors and officers as required by these indemnification provisions.

At present, there is no pending litigation or proceeding involving any of our directors or executive officers as to which indemnification is required or permitted, and we are not aware of any threatened litigation or proceeding that may result in a claim for indemnification.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, executive officers or persons controlling us, we have been informed that, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

CERTAIN RELATIONSHIPS AND RELATED-PARTY TRANSACTIONS

In addition to the executive officer and director compensation arrangements discussed above under "Management—Non-Employee Director Compensation" and "Executive Compensation," below we describe transactions since January 1, 2013 to which we have been or will be a participant, in which the amount involved in the transaction exceeds or will exceed $120,000 and in which any of our directors, executive officers or beneficial holders of more than 5% of any class of our capital stock, or any immediate family member of, or person sharing the household with, any of these individuals, had or will have a direct or indirect material interest.

Equity Financings

Series A Convertible Preferred Stock Financing

In three closings in July 2013, December 2013 and November 2014, we sold an aggregate of 5,022,876 shares of our Series A convertible preferred stock at a purchase price of $5.9727 per share for an aggregate purchase price of approximately $30.0 million. Each share of our Series A convertible preferred stock will convert automatically into one share of our common stock immediately prior to the completion of this offering.

The purchasers of our Series A convertible preferred stock are entitled to specified registration rights. For additional information, see "Description of Capital Stock—Registration Rights." The following table summarizes the Series A convertible preferred stock purchased by our directors, executive officers and beneficial holders of more than 5% of our capital stock. The terms of these purchases were the same for all purchasers of our Series A convertible preferred stock.

Name of Stockholder	Shares of Series A Convertible Preferred Stock	Total Purchase Price
OrbiMed Private Investments IV, LP[1]	2,511,441	$14,999,997
Entities affiliated with 5AM Ventures[2]	1,423,147	8,499,993
Entities affiliated with Versant Ventures[3]	1,088,288	6,499,998

(1) Jonathan Silverstein, a member of our board of directors, is a general partner at OrbiMed, and Stephen Squinto, a member of our board of directors, is a venture partner at OrbiMed.

(2) Consists of shares held by 5AM Ventures III, L.P. and 5AM Co-Investors III, L.P. Kush M. Parmar, a member of our board of directors, is a partner of 5AM Ventures.

(3) Consists of shares held by Versant Venture Capital IV, L.P. and Versant Side Fund IV, L.P. Thomas F. Woiwode, a member of our board of directors, is the managing director of Versant Ventures.

Series B Convertible Preferred Stock Financing

In November 2014, we sold an aggregate of 3,796,635 shares of our Series B convertible preferred stock at a purchase price of $11.1942 per share for an aggregate purchase price of approximately $42.5 million. Each share of our Series B convertible preferred stock will convert automatically into one share of our common stock immediately prior to the completion of this offering.

The purchasers of our Series B convertible preferred stock are entitled to specified registration rights. For additional information, see "Description of Capital Stock—Registration Rights." The following table summarizes the Series B convertible preferred stock purchased by our directors, executive officers and beneficial holders of more than 5% of our capital stock. The terms of these purchases were the same for all purchasers of our Series B convertible preferred stock.

Name of Stockholder	Shares of Series B Convertible Preferred Stock	Total Purchase Price
OrbiMed Private Investments IV, LP[1]	1,071,992	$12,000,003
Entities affiliated with Deerfield Management[2]	803,993	9,000,002
Entities affiliated with 5AM Ventures[3]	632,772	7,083,332
Entities affiliated with Versant Ventures[4]	483,885	5,416,668
Sofinnova Venture Partners IX, L.P.	446,663	4,999,998

(1) Jonathan Silverstein, a member of our board of directors, is a general partner at OrbiMed, and Stephen Squinto, a member of our board of directors, is a venture partner at OrbiMed.
(2) Consists of shares held by Deerfield Special Situations Fund, L.P. and Deerfield Private Design Fund III, L.P. Jonathan Leff, a member of our board of directors, is a partner of Deerfield Management.
(3) Consists of shares held by 5AM Ventures III, L.P. and 5AM Co-Investors III, L.P. Kush Parmar, a member of our board of directors, is a partner of 5AM Ventures.
(4) Consists of shares held by Versant Venture Capital IV, L.P. and Versant Side Fund IV, L.P. Thomas Woiwode, a member of our board of directors, is the managing director of Versant Ventures.

Series C Convertible Preferred Stock Financing

In October 2015, we sold an aggregate of 4,325,954 shares of our Series C convertible preferred stock at a purchase price of $15.0256 per share for an aggregate purchase price of approximately $65.0 million. Each share of our Series C convertible preferred stock will convert automatically into one share of our common stock immediately prior to the completion of this offering.

The purchasers of our Series C convertible preferred stock are entitled to specified registration rights. For additional information, see "Description of Capital Stock—Registration Rights." The following table summarizes the Series C convertible preferred stock purchased by our directors, executive officers and beneficial holders of more than 5% of our capital stock. The terms of these purchases were the same for all purchasers of our Series C convertible preferred stock.

Name of Stockholder	Shares of Series C Convertible Preferred Stock	Total Purchase Price
OrbiMed Private Investments IV, LP[1]	332,766	$ 4,999,994
Entities affiliated with Deerfield Management[2]	199,660	2,999,998
Entities affiliated with 5AM Ventures[3]	266,212	3,999,999
Entities affiliated with Versant Ventures[4]	199,659	2,999,998
Sofinnova Venture Partners IX, L.P.	798,640	11,999,998

(1) Jonathan Silverstein, a member of our board of directors, is a general partner at OrbiMed, and Stephen Squinto, a member of our board of directors, is a venture partner at OrbiMed.
(2) Consists of shares held by Deerfield Special Situations Fund, L.P. and Deerfield Private Design Fund III, L.P. Jonathan Leff, a member of our board of directors, is a partner of Deerfield Management.
(3) Consists of shares held by 5AM Ventures III, L.P. and 5AM Co-Investors III, L.P. Kush Parmar, a member of our board of directors, is a partner of 5AM Ventures.

(4) Consists of shares held by Versant Venture Capital IV, L.P. and Versant Side Fund IV, L.P. Thomas Woiwode, a member of our board of directors, is the managing director of Versant Ventures.

Cardiogen Acquisition

In August 2015, we acquired Cardiogen Sciences, Inc., and in consideration issued an aggregate of 46,969 shares of our Series B convertible preferred stock, 1,293,058 shares of our common stock and the opportunity to receive additional cash or shares of our common stock upon achievement of a certain milestone. Each share of our Series B convertible preferred stock will convert automatically into one share of our common stock immediately prior to the completion of this offering.

The holders of our Series B convertible preferred stock are entitled to specified registration rights. For additional information, see "Description of Capital Stock—Registration Rights." The following table summarizes the Series B convertible preferred stock acquired in connection with the Cardiogen acquisition by our directors, executive officers and beneficial holders of more than 5% of our capital stock. The same terms applied to all acquisitions of shares of Series B convertible preferred stock in the Cardiogen acquisition.

Name of Stockholder	Shares of Common Stock	Shares of Series B Convertible Preferred Stock
Entities affiliated with Louis Lange[1]	587,300	24,633

(1) Consists of shares held by Louis G. Lange, Amygdala Lange Trust, Lange Minors' Trust, Asset Management Ventures Fund, L.P. and Camp Lowell, LLC.

Transactions with OrbiMed

In 2013, we reimbursed entities affiliated with OrbiMed, a holder of more than 5% of our capital stock, $400,000 for certain expenses incurred in connection with the founding of our company.

Potential Insider Participation

Our existing institutional investors associated with our board have indicated an interest in purchasing shares of common stock in this offering with an aggregate value of approximately $30.0 million at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, fewer or no shares in this offering to any of these parties, or any of these parties may determine to purchase more, fewer or no shares in this offering.

Amended and Restated Investors' Rights Agreement

We have entered into an amended and restated investors' rights agreement with certain holders of our common stock and holders of our convertible preferred stock, including entities with which certain of our directors are affiliated. These stockholders are entitled to rights with respect to the registration of their shares following our initial public offering under the Securities Act. For a description of these registration rights, see "Description of Capital Stock—Registration Rights."

Equity Grants to Executive Officers and Directors

We have granted stock options to our executive officers and certain directors, as more fully described in the sections entitled "Executive Compensation" and "Management—Non-Employee Director Compensation," respectively.

Indemnification Agreements

In connection with this offering, we intend to enter into indemnification agreements with each of our directors and executive officers. The indemnification agreements, our restated certificate of incorporation and our restated bylaws will require us to indemnify our directors to the fullest extent not prohibited by Delaware law. Subject to certain limitations, our restated bylaws also require us to advance expenses incurred by our directors and officers. For more information regarding these agreements, see "Executive Compensation—Limitations on Liability and Indemnification Matters."

Review, Approval or Ratification of Transactions with Related-Parties

In connection with this offering, we adopted a written related-person transactions policy that will become effective upon completion of this offering and provides that our executive officers, directors, nominees for election as a director, beneficial owners of more than 5% of our common stock, and any members of the immediate family of the foregoing persons, are not permitted to enter into a material related-person transaction with us without the review and approval of our audit committee, or a committee composed solely of independent directors in the event it is inappropriate for our audit committee to review such transaction due to a conflict of interest. The policy provides that any request for us to enter into a transaction with an executive officer, director, nominee for election as a director, beneficial owner of more than 5% of our common stock or with any of their immediate family members or affiliates, in which the amount involved exceeds $120,000 will be presented to our audit committee for review, consideration and approval. In approving or rejecting any such proposal, we expect that our audit committee will consider the relevant facts and circumstances available and deemed relevant to the audit committee, including, but not limited to, whether the transaction is on terms no less favorable than terms generally available to an unaffiliated third party under the same or similar circumstances and the extent of the related person's interest in the transaction.

PRINCIPAL STOCKHOLDERS

The following table sets forth certain information with respect to the beneficial ownership of our common stock as of May 31, 2016, and as adjusted to reflect the sale of common stock by us in this offering, for:

- each of our directors;
- each of our named executive officers;
- all of our directors and executive officers as a group; and
- each person, or group of affiliated persons, known by us to be the beneficial owner of more than 5% of our common stock.

We have determined beneficial ownership in accordance with the rules of the SEC, and the information is not necessarily indicative of beneficial ownership for any other purpose. Except as indicated by the footnotes below, we believe, based on information furnished to us, that the persons and entities named in the table below have sole voting and sole investment power with respect to all shares of common stock that they beneficially owned, subject to applicable community property laws.

Applicable percentage ownership is based on 16,020,378 shares of common stock issued and outstanding as of May 31, 2016 and assumes the conversion of all outstanding shares of preferred stock into an aggregate of 13,820,301 shares of our common stock. For purposes of computing the applicable percentage of shares beneficially owned by a person after this offering in the table below, we have assumed that 5,000,000 shares of common stock will be issued by us in our initial public offering based on an assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated offering price range set forth on the cover of this prospectus. In computing the number of shares of common stock beneficially owned by a person and the percentage ownership of that person, we deemed to be outstanding all shares of common stock subject to options held by that person or entity that are currently exercisable or that will become exercisable within 60 days of May 31, 2016. We did not deem these shares outstanding, however, for the purpose of computing the percentage ownership of any other person. Unless otherwise indicated, the address of each beneficial owner listed in the table on the following page is c/o Audentes Therapeutics, Inc., 600 California Street, 17th Floor, San Francisco, California 94108.

Our existing institutional investors associated with our board have indicated an interest in purchasing shares of common stock in this offering with an aggregate value of approximately $30.0 million at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, fewer or no shares in this offering to any of these parties, or any of these parties may determine to purchase more, fewer or no shares in this offering. The following table does not reflect any potential purchases by these parties.

Name of Beneficial Owner	Shares Beneficially Owned Prior to this Offering		Shares Beneficially Owned After this Offering	
	Number	Percentage	Number	Percentage
5% Stockholders:				
OrbiMed Private Investments IV, LP[1]	4,768,304	29.8%	4,768,304	22.7%
Entities affiliated with 5AM Ventures[2]	2,322,131	14.5	2,322,131	11.0
Entities affiliated with Versant Ventures[3]	1,771,832	11.1	1,771,832	8.4
Sofinnova Venture Partners IX, L.P.[4]	1,245,303	7.8	1,245,303	5.9
Entities affiliated with Deerfield Management[5]	1,003,653	6.3	1,003,653	4.8
Directors and Named Executive Officers:				
Matthew Patterson[6]	453,568	2.8	453,568	2.1
Suyash Prasad[7]	67,484	*	67,484	*
John Gray[8]	48,227	*	48,227	*
Louis Lange[9]	611,933	3.8	611,933	2.9
Jonathan Leff	—	—	—	—
Scott Morrison[10]	5,606	*	5,606	*
Kush Parmar[2]	2,322,131	14.5	2,322,131	11.0
Thomas Schuetz[11]	107,388	*	107,388	*
Jonathan Silverstein[1]	4,768,304	29.8	4,768,304	22.7
Stephen Squinto[12]	9,343	*	9,343	*
Thomas Woiwode[3]	1,771,832	11.1	1,771,832	8.4
All executive officers and directors as a group (15 persons)[13]	10,217,124	62.0	10,217,124	47.6

* Represents beneficial ownership of less than one percent.

(1) Represents shares of common stock held by OrbiMed Private Investments IV, LP, or OPI IV. OrbiMed Capital GP IV LLC, or GP IV, is the sole general partner of OPI IV. OrbiMed Advisors LLC, or OrbiMed, is the managing member of GP IV. Samuel D. Isaly is the managing member of OrbiMed. By virtue of such relationships, GP IV, OrbiMed, and Mr. Isaly may be deemed to have voting and investment power with respect to the shares held by OPI IV. Jonathan T. Silverstein, a member of OrbiMed, is a member of our board of directors. The address of OPI IV is c/o OrbiMed Advisors LLC, 601 Lexington Avenue, 54th floor, New York, New York 10022.

(2) Represents (i) 2,263,790 shares held by 5AM Ventures III, L.P., or 5AM Ventures, and (ii) 58,341 shares held by 5AM Co-Investors III, L.P., or 5AM Co-Investors. 5AM Partners III, LLC, or 5AM Partners, is the general partner of each of 5AM Ventures and 5AM Co-Investors, and may be deemed to have sole voting and investment power over the shares held by each of 5AM Ventures and 5AM Co-Investors. Andrew Schwab, John Diekman and Scott Rocklage are the managing members of 5AM Partners. Kush Parmar, a member of our board of directors, is a managing partner at 5AM Venture Management, LLC, which is an affiliate of 5AM Partners. The address of 5AM Ventures is 2200 Sand Hill Road, Suite 110, Menlo Park, California 94025.

(3) Represents (i) 1,760,743 shares held by Versant Venture Capital IV, L.P., or VVC IV, and (ii) 11,089 shares held by Versant Side Fund IV, L.P., or VSF IV. Versant Ventures IV, LLC, or VV IV, is the sole general partner of each of VVC IV and VSF IV. Thomas Woiwode, a member of our board of directors, together with Brian Atwood, Bradley Bolzon, Samuel Colella, Ross Jaffe, William Link, Kirk Nielsen, Robin Praeger, Rebecca Robertson and Charles Warden, are the managing directors of VV IV and may be deemed to share voting and investment power over the shares held by each of the VVC IV and VSF IV. The address of Versant Ventures is One Sansome Street, Suite 3630, San Francisco, California 94104.

(4) Represents shares of common stock held by Sofinnova Venture Partners IX, L.P., or SVP IX. Sofinnova Management IX, L.L.C., or SM IX, is the general partner of SVP IX and Dr. Michael F. Powell, Dr. James I. Healy and Dr. Anand Mehra, the managing members of SM IX, may be deemed to have shared voting and investment power over the shares held by SVP IX. The address for SVP IX is c/o Sofinnova Ventures, 3000 Sand Hill Road, Building 4, Suite 250, Menlo Park, California 94025.

(5) Represents (i) 367,827 shares held by Deerfield Special Situations Fund, L.P., or Deerfield Fund, and (ii) 635,826 shares held by Deerfield Private Design Fund III, L.P., or Deerfield Fund III. Deerfield Mgmt, L.P. is the general partner of Deerfield Fund, and Deerfield Mgmt III, L.P. is the general partner of Deerfield Fund III. Deerfield Management Company, L.P. is the investment manager of Deerfield Fund and Deerfield Fund III. James Flynn is the sole member of the general partner of each of Deerfield Mgmt III, L.P., Deerfield Mgmt, L.P. and Deerfield Management Company, L.P. and may be deemed to have voting and investment power over the shares held by Deerfield Fund and Deerfield Fund III. The address of Deerfield Management Company, L.P. is 780 Third Avenue, 37th Floor, New York, New York 10017.

(6) Represents (i) 201,814 shares of common stock and (ii) 251,754 shares underlying options to purchase common stock that are exercisable within 60 days of May 31, 2016.
(7) Represents 67,484 shares underlying options to purchase common stock that are exercisable within 60 days of May 31, 2016.
(8) Represents 48,227 shares of common stock underlying options to purchase common stock that are exercisable within 60 days of May 31, 2016.
(9) Represents (i) 475,799 shares held by Mr. Lange, (ii) 25,678 shares held by Amygdala Lange Trust, of which Mr. Lange's domestic partner is a trustee, (iii) 8,558 shares held by Lange Minors' Trust, of which Mr. Lange's domestic partner is a trustee, (iv) 87,343 shares held by Asset Management Ventures Fund, L.P., of which Mr. Lange is a general partner, and (v) 14,555 shares held by Camp Lowell, LLC, of which Mr. Lange is president.
(10) Represents 5,606 shares underlying options to purchase common stock that are exercisable with 60 days of May 31, 2016.
(11) Represents (i) 89,695 shares of common stock and (ii) 17,693 shares underlying options to purchase common stock that are exercisable within 60 days of May 31, 2016.
(12) Represents 9,343 shares underlying options to purchase common stock that are exercisable within 60 days of May 31, 2016.
(13) Represents (i) 9,765,709 shares of common stock and (ii) 451,415 shares underlying options to purchase common stock that are exercisable within 60 days of May 31, 2016.

DESCRIPTION OF CAPITAL STOCK

Upon the completion of this offering and the filing of our restated certificate of incorporation, our authorized capital stock will consist of 300,000,000 shares of common stock, $0.00001 par value per share, and 10,000,000 shares of undesignated preferred stock, $0.00001 par value per share. The following description summarizes the most important terms of our capital stock. Because it is only a summary, it does not contain all the information that may be important to you. For a complete description, you should refer to our restated certificate of incorporation and restated bylaws, which are included as exhibits to the registration statement of which this prospectus forms a part.

Pursuant to the provisions of our certificate of incorporation, all of our outstanding convertible preferred stock will automatically convert into common stock effective immediately prior to the completion of this offering. Assuming the conversion of all outstanding shares of our convertible preferred stock into 13,820,301 shares of common stock, as of March 31, 2016, there were 16,020,378 shares of our common stock issued and outstanding, held by approximately 58 stockholders of record, and no shares of our preferred stock outstanding. Our board of directors is authorized, without stockholder approval, to issue additional shares of our capital stock.

Common Stock

Dividend Rights

Subject to preferences that may apply to any shares of preferred stock outstanding at the time, the holders of our common stock are entitled to receive dividends out of funds legally available if our board of directors, in its discretion, determines to issue dividends and then only at the times and in the amounts that our board of directors may determine. See “Dividend Policy” above.

Voting Rights

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders. We do not intend to provide for cumulative voting for the election of directors in our restated certificate of incorporation. Accordingly, holders of a majority of the shares of our common stock will be able to elect all of our directors. Our restated certificate of incorporation will establish a classified board of directors, to be divided into three classes with staggered three-year terms. Only one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms.

No Preemptive or Similar Rights

Our common stock is not entitled to preemptive rights, and is not subject to conversion, redemption or sinking fund provisions.

Right to Receive Liquidation Distributions

Upon our liquidation, dissolution or winding-up, the assets legally available for distribution to our stockholders would be distributable ratably among the holders of our common stock and any participating preferred stock outstanding at that time, subject to prior satisfaction of all outstanding debt and liabilities and the preferential rights of and the payment of liquidation preferences, if any, on any outstanding shares of preferred stock.

Preferred Stock

Pursuant to the provisions of our certificate of incorporation, each currently-outstanding share of convertible preferred stock will automatically be converted into one share of common stock effective immediately prior to the completion of this offering. Following this offering, no shares of preferred stock will be outstanding.

Pursuant to our restated certificate of incorporation that will become effective in connection with the completion of this offering, our board of directors will be authorized, subject to limitations prescribed by Delaware law, to issue from time to time up to 10,000,000 shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each series and to fix the designation, powers, preferences and rights of the shares of each series and any of their qualifications, limitations or restrictions, in each case without further vote or action by our stockholders. Our board of directors will also be able to increase or decrease the number of shares of any series of preferred stock, but not below the number of shares of that series then outstanding, without any further vote or action by our stockholders. Our board of directors may be able to authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of our common stock. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in control of our company and might adversely affect the market price of our common stock and the voting and other rights of the holders of our common stock. We have no current plan to issue any shares of preferred stock.

Stock Options

As of March 31, 2016, we had outstanding options to purchase an aggregate of 2,279,086 shares of our common stock, with a weighted-average exercise price of approximately $3.99 per share. Subsequent to March 31, 2016, we issued options to purchase an aggregate of 82,739 shares of our common stock, with an exercise price of $7.54 per share. We also expect to grant stock options to purchase up to 101,127 shares of our common stock on the date of this prospectus, with an exercise price equal to the initial public offering price of our common stock.

Registration Rights

The holders of certain outstanding shares of our common stock and the holders of shares of our common stock issuable upon conversion of our convertible preferred stock, or their permitted transferees, are entitled to rights with respect to the registration of these shares under the Securities Act. These shares are referred to as registrable securities. Upon completion of this offering, there will be approximately 15,375,756 registrable securities outstanding. These rights are provided under the terms of an amended and restated investors' rights agreement between us and the holders of these shares, which was entered into in connection with our preferred stock financings and with our Common Stock Purchase Agreement with Genethon, and include demand registration rights, short-form registration rights and piggyback registration rights. In any registration made pursuant to such amended and restated investors' rights agreement, all fees, costs and expenses of underwritten registrations, including fees and disbursements of one counsel to the selling stockholders not to exceed $30,000, will be borne by us and all selling expenses, including estimated underwriting discounts and selling commissions, will be borne by the holders of the shares being registered.

The registration rights terminate five years following the completion of this offering or, with respect to any particular stockholder, at such time as we have completed this offering and that stockholder can sell all of its shares during any three-month period pursuant to Rule 144 of the Securities Act.

Demand Registration Rights

Under the terms of the amended and restated investors' rights agreement, if we receive a written request, at any time after 180 days following the effective date of this offering, from the holders of at least 66.67% of the common stock (i) issued or issuable upon conversion of then-outstanding shares of preferred stock held by preferred stockholders under the amended and restated investors' rights agreement and (ii) then held by Genethon, voting together as a single class on an as-converted to common stock basis, that we file a registration statement under the Securities Act covering the registration of outstanding registrable securities, then we will be required to use commercially reasonable efforts to register, as soon as practicable, and in any event within 90 days of such written request, all of the shares requested to be registered for public resale, if the amount of registrable securities to be registered will have anticipated aggregate gross proceeds (net of underwriting discounts, commissions, taxes and certain fees and expenses of counsel for selling stockholders) of at least $25.0 million. We are required to effect only two registrations pursuant to this provision of the amended and restated investors' rights agreement. We may postpone the filing of a registration statement no more than once during any 12-month period for up to 90 days if our board of directors determines that the filing would be materially detrimental to us and our stockholders, but we shall not register any securities for our own account or that of any other stockholder during such 90-day period, subject to certain exceptions. We are not required to effect a demand registration under certain additional circumstances specified in the amended and restated investors' rights agreement.

Form S-3 Registration Rights

Any holder of registrable securities then outstanding can request that we register all or part of their shares on Form S-3 if we are eligible to file a registration statement on Form S-3 and if the aggregate price to the public of the shares offered is at least $5.0 million (net of underwriting discounts, commissions, taxes and certain fees and expenses of counsel for selling stockholders). We shall not be obligated to effect a registration if we have effected two registrations within the 12-month period immediately preceding the date of the request. We may postpone the filing of a registration statement no more than once during any 12-month period for up to 90 days if our board of directors determines that the filing would be materially detrimental to us and our stockholders, but we shall not register any securities for our own account or that of any other stockholder during such 90-day period, subject to certain exceptions. We are not required to effect a registration on Form S-3 under certain additional circumstances specified in the amended and restated investors' rights agreement.

Piggyback Registration Rights

In connection with this offering, holders of our registrable securities were entitled to, and the necessary percentage of holders waived, their rights to notice of this offering and to include their registrable securities in this offering. If we register any of our securities for public sale in another offering, holders of registrable securities will have the right to include their shares in the registration statement. However, this right does not apply to a registration relating to employee benefit plans, a registration relating to a corporate reorganization, a registration on any registration form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of registrable securities or a registration in which the only common stock being registered is common stock issuable upon conversion of debt securities that are also being registered. If the total number of securities requested by stockholders to be included in such offering exceeds the number of securities to be sold (other than by us) that the underwriters in their reasonable discretion determine is compatible with the success of the offering, then we will be required to include in the offering only that number of securities that we and the underwriters determine will not jeopardize the success of the offering. In this case, the number of shares held by the selling stockholders to be registered will be allocated among the selling stockholders in proportion the number of registrable securities owned or held by each selling stockholders or in such other proportions as mutually agreed to by all such selling stockholders. However, the number of shares to be registered by these holders cannot be reduced below 30% of the total shares covered by the registration statement, other than in the initial public offering.

Anti-Takeover Provisions

The provisions of Delaware law, our restated certificate of incorporation and our restated bylaws, as we expect they will be in effect immediately prior to the completion of this offering, could have the effect of delaying, deferring or discouraging another person from acquiring control of our company. These provisions, which are summarized below, may have the effect of discouraging takeover bids. They are also designed, in part, to encourage persons seeking to acquire control of us to negotiate first with our board of directors. We believe that the benefits of increased protection of our potential ability to negotiate with an unfriendly or unsolicited acquirer outweigh the disadvantages of discouraging a proposal to acquire us because negotiation of these proposals could result in an improvement of their terms.

Delaware Law

We are subject to the provisions of Section 203 of the Delaware General Corporation Law, or DGCL, regulating corporate takeovers. In general, DGCL Section 203 prohibits a publicly held Delaware corporation from engaging in a business combination with an interested stockholder for a period of three years following the date on which the person became an interested stockholder unless:

- prior to the date of the transaction, the board of directors of the corporation approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;

- the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, but not the outstanding voting stock owned by the interested stockholder, (i) shares owned by persons who are directors and also officers and (ii) shares owned by employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or

- at or subsequent to the date of the transaction, the business combination is approved by the board of directors of the corporation and authorized at an annual or special meeting of stockholders, and not by written consent, by the affirmative vote of at least 66.67% of the outstanding voting stock that is not owned by the interested stockholder.

Generally, a business combination includes a merger, asset or stock sale, or other transaction or series of transactions together resulting in a financial benefit to the interested stockholder. An interested stockholder is a person who, together with affiliates and associates, owns or, within three years prior to the determination of interested stockholder status, did own 15% or more of a corporation's outstanding voting stock. We expect the existence of this provision to have an anti-takeover effect with respect to transactions our board of directors does not approve in advance. We also anticipate that DGCL Section 203 may also discourage attempts that might result in a premium over the market price for the shares of common stock held by stockholders.

Restated Certificate of Incorporation and Restated Bylaws Provisions

Our restated certificate of incorporation and our restated bylaws, as we expect they will be in effect immediately prior to the completion of this offering, include a number of provisions that could deter hostile takeovers or delay or prevent changes in control of our company, including the following:

- *Board of Directors Vacancies.* Our restated certificate of incorporation and restated bylaws will authorize only our board of directors to fill vacant directorships, including newly created seats. In addition, the number of directors constituting our board of directors will be permitted to be set only by a resolution adopted by a majority vote of our entire board of directors. These provisions would

prevent a stockholder from increasing the size of our board of directors and then gaining control of our board of directors by filling the resulting vacancies with its own nominees. This makes it more difficult to change the composition of our board of directors but promotes continuity of management.

- *Classified Board.* Our restated certificate of incorporation and restated bylaws will provide that our board of directors will be classified into three classes of directors, each with staggered three-year terms. A third party may be discouraged from making a tender offer or otherwise attempting to obtain control of us as it is more difficult and time consuming for stockholders to replace a majority of the directors on a classified board of directors. See "Management—Board of Directors."

- *Stockholder Action; Special Meetings of Stockholders.* Our restated certificate of incorporation will provide that our stockholders may not take action by written consent, but may only take action at annual or special meetings of our stockholders. As a result, a holder controlling a majority of our capital stock would not be able to amend our restated bylaws or remove directors without holding a meeting of our stockholders called in accordance with our restated bylaws. Further, our restated bylaws and restated certificate of incorporation will provide that special meetings of our stockholders may be called only by a majority of our board of directors, the chairman of our board of directors, our Chief Executive Officer or our President, thus prohibiting a stockholder from calling a special meeting. These provisions might delay the ability of our stockholders to force consideration of a proposal or for stockholders controlling a majority of our capital stock to take any action, including the removal of directors.

- *Advance Notice Requirements for Stockholder Proposals and Director Nominations.* Our restated bylaws will provide advance notice procedures for stockholders seeking to bring business before our annual meeting of stockholders or to nominate candidates for election as directors at our annual meeting of stockholders. Our restated bylaws also will specify certain requirements regarding the form and content of a stockholder's notice. These provisions might preclude our stockholders from bringing matters before our annual meeting of stockholders or from making nominations for directors at our annual meeting of stockholders if the proper procedures are not followed. We expect that these provisions might also discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to obtain control of our company.

- *No Cumulative Voting.* The Delaware General Corporation Law provides that stockholders are not entitled to the right to cumulate votes in the election of directors unless a corporation's certificate of incorporation provides otherwise. Our restated certificate of incorporation will not provide for cumulative voting.

- *Directors Removed Only for Cause.* Our restated certificate of incorporation will provide that stockholders may remove directors only for cause and only by the affirmative vote of the holders of at least two-thirds of our outstanding common stock.

- *Amendment of Charter Provisions.* Any amendment of the above expected provisions in our restated certificate of incorporation would require approval by holders of at least two-thirds of our outstanding common stock.

- *Issuance of Undesignated Preferred Stock.* Our board of directors has the authority, without further action by the stockholders, to issue up to 10,000,000 shares of undesignated preferred stock with rights and preferences, including voting rights, designated from time to time by our board of directors. The existence of authorized but unissued shares of preferred stock would enable our board of directors to render more difficult or to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or other means.

- *Choice of Forum.* Our restated certificate of incorporation will provide that the Court of Chancery of the State of Delaware will be the exclusive forum for: any derivative action or proceeding brought on our behalf; any action asserting a breach of fiduciary duty; any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, our restated certificate of incorporation or our restated bylaws; any action to interpret, apply, enforce or determine the validity of our restated certificate of incorporation or our restated bylaws; or any action asserting a claim against us that is governed by the internal affairs doctrine. The enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be inapplicable or unenforceable.

Exchange Listing

We have applied to list our common stock on The NASDAQ Global Market under the symbol "BOLD."

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, LLC. The transfer agent's address is 6201 15th Avenue, Brooklyn, New York 11219, and its telephone number is (800) 937-5449.

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has not been a public market for shares of our common stock, and we cannot predict the effect, if any, that market sales of shares of our common stock or the availability of shares of our common stock for sale will have on the market price of our common stock prevailing from time to time. Nevertheless, sales of substantial amounts of our common stock, including shares issued upon exercise of outstanding options, in the public market following this offering could adversely affect market prices prevailing from time to time and could impair our ability to raise capital through the sale of our equity securities.

Upon the completion of this offering, we will have a total of 21,020,378 shares of our common stock outstanding, assuming (i) the automatic conversion of shares of our convertible preferred stock outstanding as of March 31, 2016 into 13,820,301 shares of our common stock effective immediately prior to the completion of this offering and (ii) the sale and issuance of 5,000,000 shares of our common stock in this offering, at an assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated offering price range set forth on the cover page of this prospectus. Of these outstanding shares, all of the shares of common stock sold in this offering will be freely tradable, except that any shares purchased in this offering by our affiliates, as that term is defined in Rule 144 under the Securities Act, would only be able to be sold in compliance with the Rule 144 limitations described below.

The remaining outstanding shares of our common stock will be deemed "restricted securities" as defined in Rule 144. Restricted securities may be sold in the public market only if they are registered under the Securities Act or if they qualify for an exemption from registration under Rule 144 or Rule 701 promulgated under the Securities Act, which rules are summarized below. In addition, all of our security holders have entered into market standoff agreements with us or lock-up agreements with the underwriters under which they have agreed, subject to specific exceptions, not to sell any of our stock for at least 180 days following the date of this prospectus, as described below. As a result of these agreements and the provisions of our amended and restated investors' rights agreement described above under "Description of Capital Stock—Registration Rights," subject to the provisions of Rule 144 or Rule 701, shares will be available for sale in the public market as follows:

- beginning on the date of this prospectus, all of the shares sold in this offering will be immediately available for sale in the public market; and

- beginning 181 days after the date of this prospectus, 16,020,378 additional shares will become eligible for sale in the public market, of which 9,765,709 shares will be held by affiliates and subject to the volume and other restrictions of Rule 144, as described below.

The foregoing does not reflect the potential purchase of any shares by our existing institutional investors associated with our board pursuant to their indications of interest to purchase shares of common stock in this offering with an aggregate value of approximately $30.0 million at the initial public offering price.

Lock-Up and Market Standoff Agreements

All of our directors, executive officers and our security holders are subject to lock-up agreements or market standoff provisions that, subject to certain exceptions, prohibit them from directly or indirectly offering, pledging, selling, contracting to sell, selling any option or contract to purchase, purchasing any option or contract to purchase, granting any option, right or warrant to purchase or otherwise transferring or disposing of any shares of our common stock, options to acquire shares of our common stock or any securities convertible into or exercisable or exchangeable for common stock, whether now owned or hereafter acquired, or entering into any swap or any other agreement or any transaction that transfer, in whole or in part, directly or indirectly, the economic consequence of ownership, for a period of 180 days following the date of this prospectus, without the prior written consent of Merrill Lynch, Pierce, Fenner & Smith Incorporated and Cowen and Company, LLC. See the section entitled "Underwriting."

Rule 144

In general, under Rule 144 as currently in effect, once we have been subject to public company reporting requirements for at least 90 days, a person who is not deemed to have been one of our affiliates for purposes of the Securities Act at any time during the 90 days preceding a sale and who has beneficially owned the shares proposed to be sold for at least six months, including the holding period of any prior owner other than our affiliates, is entitled to sell those shares without complying with the manner of sale, volume limitation or notice provisions of Rule 144, subject to compliance with the public information requirements of Rule 144. If such a person has beneficially owned the shares proposed to be sold for at least one year, including the holding period of any prior owner other than our affiliates, then that person would be entitled to sell those shares without complying with any of the requirements of Rule 144.

In general, under Rule 144, as currently in effect, our affiliates or persons selling shares on behalf of our affiliates are entitled to sell upon expiration of the lock-up and market standoff agreements described above, within any three-month period, a number of shares that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately 210,204 shares immediately after this offering; or

- the average weekly trading volume of our common stock during the four calendar weeks preceding the filing of a notice on Form 144 with respect to that sale.

Sales under Rule 144 by our affiliates or persons selling shares on behalf of our affiliates are also subject to certain manner of sale provisions and notice requirements and to the availability of current public information about us.

Rule 701

Rule 701 generally allows a stockholder who purchased shares of our common stock pursuant to a written compensatory plan or contract and who is not deemed to have been an affiliate of our company during the immediately preceding 90 days to sell these shares in reliance upon Rule 144, but without being required to comply with the public information, holding period, volume limitation or notice provisions of Rule 144. Rule 701 also permits affiliates of our company to sell their Rule 701 shares under Rule 144 without complying with the holding period requirements of Rule 144. All holders of Rule 701 shares, however, are required by that rule to wait until 90 days after the date of this prospectus before selling those shares pursuant to Rule 701 and are subject to the lock-up and market standoff agreements described above.

Stock Options

In connection with this offering, we intend to file a registration statement on Form S-8 under the Securities Act covering all of the shares of our common stock subject to outstanding options and the shares of our common stock reserved for issuance under our stock plans. We expect to file this registration statement as soon as permitted under the Securities Act. However, the shares registered on Form S-8 may be subject to the volume limitations and the manner of sale, notice and public information requirements of Rule 144 and will not be eligible for resale until expiration of the lock-up and market standoff agreements to which they are subject.

Registration Rights

We have granted demand, piggyback and Form S-3 registration rights to certain of our stockholders to sell our common stock. Registration of the sale of these shares under the Securities Act would result in these shares becoming freely tradable without restriction under the Securities Act immediately upon the effectiveness of the registration, except for shares purchased by affiliates. For a further description of these rights, see "Description of Capital Stock—Registration Rights."

MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS FOR NON-U.S. HOLDERS OF COMMON STOCK

This section summarizes the material U.S. federal income tax considerations relating to the acquisition, ownership and disposition of our common stock by "non-U.S. holders" (as defined below) pursuant to this offering. This summary does not provide a complete analysis of all potential U.S. federal income tax considerations relating thereto. The information provided below is based upon provisions of the Internal Revenue Code of 1986, as amended (the "Code"), Treasury regulations promulgated thereunder, administrative rulings and judicial decisions currently in effect. These authorities may change at any time, possibly retroactively, or the Internal Revenue Service, or IRS, might interpret the existing authorities differently. In either case, the tax considerations of owning or disposing of our common stock could differ from those described below. As a result, we cannot assure you that the tax consequences described in this discussion will not be challenged by the IRS or will be sustained by a court if challenged by the IRS.

This summary does not address the tax considerations arising under the laws of any non-U.S., state or local jurisdiction, or under U.S. federal gift and estate tax laws, except to the limited extent provided below. In addition, this discussion does not address tax considerations applicable to an investor's particular circumstances or to investors that may be subject to special tax rules, including, without limitation:

- banks, insurance companies or other financial institutions;
- partnerships or entities or arrangements treated as partnerships or other pass-through entities for U.S. federal tax purposes (or investors in such entities);
- corporations that accumulate earnings to avoid U.S. federal income tax;
- persons subject to the alternative minimum tax or medicare contribution tax;
- tax-exempt organizations or tax-qualified retirement plans;
- controlled foreign corporations or passive foreign investment companies;
- persons who acquired our common stock as compensation for services;
- dealers in securities or currencies;
- traders in securities that elect to use a mark-to-market method of accounting for their securities holdings;
- persons that own, or are deemed to own, more than 5% of our capital stock (except to the extent specifically set forth below);
- certain former citizens or long-term residents of the United States;
- persons who hold our common stock as a position in a hedging transaction, "straddle," "conversion transaction" or other risk reduction transaction;
- persons who do not hold our common stock as a capital asset within the meaning of Section 1221 of the Code (generally, for investment purposes); or
- persons deemed to sell our common stock under the constructive sale provisions of the Code.

In addition, if a partnership or entity classified as a partnership for U.S. federal income tax purposes is a beneficial owner of our common stock, the tax treatment of a partner in the partnership or an owner of the entity will depend upon the status of the partner or other owner and the activities of the partnership or other entity. Accordingly, this summary does not address tax considerations applicable to partnerships that hold our common stock, and partners in such partnerships should consult their tax advisors.

INVESTORS CONSIDERING THE PURCHASE OF OUR COMMON STOCK SHOULD CONSULT THEIR OWN TAX ADVISORS REGARDING THE APPLICATION OF THE U.S. FEDERAL INCOME AND ESTATE TAX LAWS TO THEIR PARTICULAR SITUATIONS AND THE CONSEQUENCES OF FOREIGN, STATE OR LOCAL LAWS, AND TAX TREATIES

Non-U.S. Holder Defined

For purposes of this summary, a "non-U.S. holder" is any holder of our common stock, other than a partnership, that is not:

- an individual who is a citizen or resident of the United States;
- a corporation, or other entity taxable as a corporation for U.S. federal income tax purposes, created or organized under the laws of the United States, any state therein or the District of Columbia;
- a trust if it (1) is subject to the primary supervision of a U.S. court and one of more U.S. persons have authority to control all substantial decisions of the trust or (2) has a valid election in effect under applicable U.S. Treasury regulations to be treated as a U.S. person; or
- an estate whose income is subject to U.S. income tax regardless of source.

If you are a non-U.S. citizen who is an individual, you may, in many cases, be deemed to be a resident alien, as opposed to a nonresident alien, by virtue of being present in the United States for at least 31 days in the calendar year and for an aggregate of at least 183 days during a three- year period ending in the current calendar year. For these purposes, all the days present in the current year, one-third of the days present in the immediately preceding year, and one-sixth of the days present in the second preceding year are counted. Resident aliens are subject to U.S. federal income tax as if they were U.S. citizens. Such an individual is urged to consult his or her own tax advisor regarding the U.S. federal income tax consequences of the ownership or disposition of our common stock.

Dividends

We do not expect to declare or make any distributions on our common stock in the foreseeable future and the terms of our credit facility currently restrict our ability to pay dividends. If we do pay dividends on shares of our common stock, however, such distributions will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. Distributions in excess of our current and accumulated earnings and profits will constitute a return of capital that is applied against and reduces, but not below zero, a non-U.S. holder's adjusted tax basis in shares of our common stock. Any remaining excess will be treated as gain realized on the sale or other disposition of our common stock. See "—Sale of Common Stock."

Any dividend paid to a non-U.S. holder on our common stock that is not effectively connected with a non-U.S. holder's conduct of a trade or business in the United States will generally be subject to U.S. withholding tax at a 30% rate. The withholding tax might apply at a reduced rate under the terms of an applicable income tax treaty between the United States and the non-U.S. holder's country of residence subject to the discussion below regarding the Foreign Account Tax Compliance Act. You should consult your tax advisors

regarding your entitlement to benefits under a relevant income tax treaty. Generally, in order for us or our paying agent to withhold tax at a lower treaty rate, a non-U.S. holder must certify its entitlement to treaty benefits. A non-U.S. holder generally can meet this certification requirement by providing a Form W-8BEN or Form W-8BEN-E (or any successor form) or appropriate substitute form to us or our paying agent. If the non-U.S. holder holds the stock through a financial institution or other agent acting on the holder's behalf, the holder will be required to provide appropriate documentation to the agent. The holder's agent will then be required to provide certification to us or our paying agent, either directly or through other intermediaries. If you are eligible for a reduced rate of U.S. federal withholding tax under an income tax treaty, you may obtain a refund or credit of any excess amounts withheld by filing an appropriate claim for a refund with the IRS in a timely manner.

Dividends received by a non-U.S. holder that are effectively connected with a U.S. trade or business conducted by the non-U.S. holder, and if required by an applicable income tax treaty between the United States and the non-U.S. holder's country of residence, are attributable to a permanent establishment maintained by the non-U.S. holder in the United States, are not subject to U.S. withholding tax. To obtain this exemption, a non-U.S. holder must provide us or our paying agent with an IRS Form W-8ECI properly certifying such exemption. Such effectively connected dividends, although not subject to withholding tax, are taxed at the same graduated rates applicable to U.S. persons, net of certain deductions and credits. In addition to being taxed at graduated tax rates, dividends received by corporate non-U.S. holders that are effectively connected with a U.S. trade or business of the corporate non-U.S. holder may also be subject to a branch profits tax at a rate of 30% or such lower rate as may be specified by an applicable tax treaty.

Sale of Common Stock

Subject to the discussion below regarding Backup Withholding and the Foreign Account Tax Compliance Act, non-U.S. holders will generally not be subject to U.S. federal income tax on any gains realized on the sale, exchange or other disposition of our common stock unless:

- the gain (i) is effectively connected with the conduct by the non-U.S. holder of a U.S. trade or business and (ii) if required by an applicable income tax treaty between the United States and the non-U.S. holder's country of residence, is attributable to a permanent establishment maintained by the non-U.S. holder in the United States (in which case the special rules described below apply);

- the non-U.S. holder is an individual who is present in the United States for 183 days or more in the taxable year of the sale, exchange or other disposition of our common stock, and certain other requirements are met (in which case the gain would be subject to a flat 30% tax, or such reduced rate as may be specified by an applicable income tax treaty, which may be offset by U.S. source capital losses, even though the individual is not considered a resident of the United States); or

- the rules of the Foreign Investment in Real Property Tax Act, or FIRPTA, treat the gain as effectively connected with a U.S. trade or business.

The FIRPTA rules may apply to a sale, exchange or other disposition of our common stock if we are, or were within the shorter of the five-year period preceding the disposition and the non-U.S. holder's holding period, a "U.S. real property holding corporation," or USRPHC. In general, we would be a USRPHC if interests in U.S. real estate comprised at least half of the value of our business assets. We do not believe that we are a USRPHC and we do not anticipate becoming one in the future. Even if we become a USRPHC, as long as our common stock is regularly traded on an established securities market, such common stock will be treated as U.S. real property interests only if beneficially owned by a non-U.S. holder that actually or constructively owned more than 5% of our outstanding common stock at some time within the five-year period preceding the disposition.

If any gain from the sale, exchange or other disposition of our common stock, (i) is effectively connected with a U.S. trade or business conducted by a non-U.S. holder and (ii) if required by an applicable

income tax treaty between the United States and the non-U.S. holder's country of residence, is attributable to a permanent establishment maintained by such non-U.S. holder in the United States, then the gain generally will be subject to U.S. federal income tax at the same graduated rates applicable to U.S. persons, net of certain deductions and credits. If the non-U.S. holder is a corporation, under certain circumstances, that portion of its earnings and profits that is effectively connected with its U.S. trade or business, subject to certain adjustments, generally would be subject also to a "branch profits tax." The branch profits tax rate is 30%, although an applicable income tax treaty between the United States and the non-U.S. holder's country of residence might provide for a lower rate.

U.S. Federal Estate Tax

The estates of nonresident alien individuals generally are subject to U.S. federal estate tax on property with a U.S. situs. Because we are a U.S. corporation, our common stock will be U.S. situs property and therefore will be included in the taxable estate of a nonresident alien decedent, unless an applicable estate tax treaty between the United States and the decedent's country of residence provides otherwise.

Backup Withholding and Information Reporting

The Code and the Treasury regulations require those who make specified payments to report the payments to the IRS. Among the specified payments are dividends and proceeds paid by brokers to their customers. The required information returns enable the IRS to determine whether the recipient properly included the payments in income. This reporting regime is reinforced by "backup withholding" rules. These rules require the payors to withhold tax from payments subject to information reporting if the recipient fails to cooperate with the reporting regime by failing to provide his taxpayer identification number to the payor, furnishing an incorrect identification number, or failing to report interest or dividends on his returns. The backup withholding tax rate is currently 28%. The backup withholding rules do not apply to payments to corporations, whether domestic or foreign, provided they establish such exemption.

Payments to non-U.S. holders of dividends on common stock generally will not be subject to backup withholding, and payments of proceeds made to non-U.S. holders by a broker upon a sale of common stock will not be subject to information reporting or backup withholding, in each case so long as the non-U.S. holder certifies its nonresident status (and we or our paying agent do not have actual knowledge or reason to know the holder is a U.S. person or that the conditions of any other exemption are not, in fact, satisfied) or otherwise establishes an exemption. The certification procedures to claim treaty benefits described under "—Dividends" will generally satisfy the certification requirements necessary to avoid the backup withholding tax. We must report annually to the IRS any dividends paid to each non-U.S. holder and the tax withheld, if any, with respect to these dividends. Copies of these reports may be made available to tax authorities in the country where the non-U.S. holder resides.

Under the Treasury regulations, the payment of proceeds from the disposition of shares of our common stock by a non-U.S. holder made to or through a U.S. office of a broker generally will be subject to information reporting and backup withholding unless the beneficial owner certifies, under penalties of perjury, among other things, its status as a non-U.S. holder (and the broker does not have actual knowledge or reason to know the holder is a U.S. person) or otherwise establishes an exemption. The payment of proceeds from the disposition of shares of our common stock by a non-U.S. holder made to or through a non-U.S. office of a broker generally will not be subject to backup withholding and information reporting, except as noted below. Information reporting, but not backup withholding, will apply to a payment of proceeds, even if that payment is made outside of the United States, if you sell our common stock through a non-U.S. office of a broker that is:

- a U.S. person (including a foreign branch or office of such person);
- a "controlled foreign corporation" for U.S. federal income tax purposes;

- a foreign person 50% or more of whose gross income from certain periods is effectively connected with a U.S. trade or business; or

- a foreign partnership if at any time during its tax year (i) one or more of its partners are U.S. persons who, in the aggregate, hold more than 50% of the income or capital interests of the partnership or (ii) the foreign partnership is engaged in a U.S. trade or business;

unless the broker has documentary evidence that the beneficial owner is a non-U.S. holder and certain other conditions are satisfied, or the beneficial owner otherwise establishes an exemption (and the broker has no actual knowledge or reason to know to the contrary).

Backup withholding is not an additional tax. Any amounts withheld from a payment to a holder of common stock under the backup withholding rules can be credited against any U.S. federal income tax liability of the holder and may entitle the holder to a refund, provided that the required information is furnished to the IRS in a timely manner.

Foreign Account Tax Compliance Act

A U.S. federal withholding tax of 30% may apply to dividends and the gross proceeds of a disposition of our common stock paid to a foreign financial institution (as specifically defined by the applicable rules) unless such institution enters into an agreement with the U.S. government to withhold on certain payments and to collect and provide to the U.S. tax authorities substantial information regarding U.S. account holders of such institution (which includes certain equity holders of such institution, as well as certain account holders that are foreign entities with U.S. owners). This U.S. federal withholding tax of 30% will also apply to dividends and the gross proceeds of a disposition of our common stock paid to a non-financial foreign entity unless such entity provides the withholding agent with either a certification that it does not have any substantial direct or indirect U.S. owners or provides information regarding direct and indirect U.S. owners of the entity. The 30% federal withholding tax described in this paragraph cannot be reduced under an income tax treaty with the United States or by providing an IRS Form W-8BEN or similar documentation. The withholding tax described above will not apply if the foreign financial institution or non-financial foreign entity otherwise qualifies for an exemption from the rules. Under certain circumstances, a non-U.S. holder might be eligible for refunds or credits of such taxes. Holders should consult with their own tax advisors regarding the possible implications of the withholding described herein.

The withholding provisions described above generally apply to proceeds from a sale or other disposition of common stock if such sale or other disposition occurs on or after January 1, 2019 and to payments of dividends on our common stock.

THE PRECEDING DISCUSSION OF U.S. FEDERAL TAX CONSIDERATIONS IS FOR GENERAL INFORMATION ONLY. IT IS NOT TAX ADVICE TO ANY NON-U.S. HOLDER IN ITS PARTICULAR CIRCUMSTANCES. EACH PROSPECTIVE INVESTOR SHOULD CONSULT ITS OWN TAX ADVISOR REGARDING THE PARTICULAR U.S. FEDERAL, STATE, LOCAL AND FOREIGN TAX CONSEQUENCES OF PURCHASING, HOLDING AND DISPOSING OF OUR COMMON STOCK, INCLUDING THE CONSEQUENCES OF ANY PROPOSED CHANGE IN APPLICABLE LAWS.

UNDERWRITING

Merrill Lynch, Pierce, Fenner & Smith Incorporated, Cowen and Company, LLC and Piper Jaffray & Co. are acting as representatives of each of the underwriters named below. Subject to the terms and conditions set forth in an underwriting agreement among us and the underwriters, we have agreed to sell to the underwriters, and each of the underwriters has agreed, severally and not jointly, to purchase from us, the number of shares of common stock set forth opposite its name below.

Underwriter	Number of Shares
Merrill Lynch, Pierce, Fenner & Smith Incorporated	
Cowen and Company, LLC	
Piper Jaffray & Co.	
Wedbush Securities Inc.	
Total	5,000,000

Subject to the terms and conditions set forth in the underwriting agreement, the underwriters have agreed, severally and not jointly, to purchase all of the shares sold under the underwriting agreement if any of these shares are purchased. If an underwriter defaults, the underwriting agreement provides that the purchase commitments of the nondefaulting underwriters may be increased or the underwriting agreement may be terminated.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act, or to contribute to payments the underwriters may be required to make in respect of those liabilities.

The underwriters are offering the shares, subject to prior sale, when, as and if issued to and accepted by them, subject to approval of legal matters by their counsel, including the validity of the shares, and other conditions contained in the underwriting agreement, such as the receipt by the underwriters of officer's certificates and legal opinions. The underwriters reserve the right to withdraw, cancel or modify offers to the public and to reject orders in whole or in part.

Commissions and Discounts

The representatives have advised us that the underwriters propose initially to offer the shares to the public at the public offering price set forth on the cover page of this prospectus and to dealers at that price less a concession not in excess of $ per share. After the initial offering, the public offering price, concession or any other term of the offering may be changed.

The following table shows the public offering price, underwriting discount and proceeds before expenses to us. The information assumes either no exercise or full exercise by the underwriters of their option to purchase additional shares.

	Per Share	Without Option	With Option
Public offering price	$	$	$
Underwriting discounts and commissions	$	$	$
Proceeds, before expenses, to us	$	$	$

The expenses of the offering, not including the underwriting discount, are estimated to be approximately $3.7 million. We have also agreed to reimburse the underwriters for up to $30,000 for their Financial Industry Regulatory Authority, Inc., or FINRA, counsel fee. In accordance with FINRA Rule 5110, this reimbursement is deemed underwriting compensation for this offering.

Option to Purchase Additional Shares

We have granted an option to the underwriters, exercisable for 30 days after the date of this prospectus, to purchase up to 750,000 additional shares at the public offering price, less the underwriting discount. If the underwriters exercise this option, each will be obligated, subject to conditions contained in the underwriting agreement, to purchase a number of additional shares proportionate to that underwriter's initial amount reflected in the above table.

No Sales of Similar Securities

We, our executive officers and directors and substantially all our other existing security holders have agreed not to sell or transfer any common stock or securities convertible into, exchangeable for, exercisable for, or repayable with common stock, for 180 days after the date of this prospectus without first obtaining the written consent of Merrill Lynch, Pierce, Fenner & Smith Incorporated and Cowen and Company, LLC. Specifically, we and these other persons have agreed, with certain limited exceptions, not to directly or indirectly

- offer, pledge, sell or contract to sell any common stock,
- sell any option or contract to purchase any common stock,
- purchase any option or contract to sell any common stock,
- grant any option, right or warrant for the sale of any common stock,
- lend or otherwise dispose of or transfer any common stock,
- request or demand that we file a registration statement related to the common stock, or
- enter into any swap or other agreement that transfers, in whole or in part, the economic consequence of ownership of any common stock whether any such swap or transaction is to be settled by delivery of shares or other securities, in cash or otherwise.

This lock-up provision applies to common stock and to securities convertible into or exchangeable or exercisable for or repayable with common stock. It also applies to common stock owned now or acquired later by the person executing the agreement or for which the person executing the agreement later acquires the power of disposition.

NASDAQ Global Market Listing

We have applied to list our common stock on The NASDAQ Global Market under the symbol "BOLD."

Before this offering, there has been no public market for our common stock. The initial public offering price will be determined through negotiations between us and the representatives. In addition to prevailing market conditions, the factors to be considered in determining the initial public offering price are

- the valuation multiples of publicly traded companies that the representatives believe to be comparable to us,
- our financial information,
- the history of, and the prospects for, our company and the industry in which we compete,

- an assessment of our management, its past and present operations, and the prospects for, and timing of, our future revenues,

- the present state of our development and

- the above factors in relation to market values and various valuation measures of other companies engaged in activities similar to ours.

An active trading market for the shares may not develop. It is also possible that after the offering the shares will not trade in the public market at or above the initial public offering price.

The underwriters do not expect to sell more than 5% of the shares in the aggregate to accounts over which they exercise discretionary authority.

Price Stabilization, Short Positions and Penalty Bids

Until the distribution of the shares is completed, SEC rules may limit underwriters and selling group members from bidding for and purchasing our common stock. However, the representatives may engage in transactions that stabilize the price of the common stock, such as bids or purchases to peg, fix or maintain that price.

In connection with the offering, the underwriters may purchase and sell our common stock in the open market. These transactions may include short sales, purchases on the open market to cover positions created by short sales and stabilizing transactions. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in the offering. "Covered" short sales are sales made in an amount not greater than the underwriters' option to purchase additional shares described above. The underwriters may close out any covered short position by either exercising their option to purchase additional shares or purchasing shares in the open market. In determining the source of shares to close out the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase shares through the option granted to them. "Naked" short sales are sales in excess of such option. The underwriters must close out any naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of our common stock in the open market after pricing that could adversely affect investors who purchase in the offering. Stabilizing transactions consist of various bids for or purchases of shares of common stock made by the underwriters in the open market prior to the completion of the offering.

The underwriters may also impose a penalty bid. This occurs when a particular underwriter repays to the underwriters a portion of the underwriting discount received by it because the representatives have repurchased shares sold by or for the account of such underwriter in stabilizing or short covering transactions.

Similar to other purchase transactions, the underwriters' purchases to cover the syndicate short sales may have the effect of raising or maintaining the market price of our common stock or preventing or retarding a decline in the market price of our common stock. As a result, the price of our common stock may be higher than the price that might otherwise exist in the open market. The underwriters may conduct these transactions on the NASDAQ Global Market, in the over-the-counter market or otherwise.

Neither we nor any of the underwriters make any representation or prediction as to the direction or magnitude of any effect that the transactions described above may have on the price of our common stock. In addition, neither we nor any of the underwriters make any representation that the representatives will engage in these transactions or that these transactions, once commenced, will not be discontinued without notice.

Electronic Distribution

In connection with the offering, certain of the underwriters or securities dealers may distribute prospectuses by electronic means, such as e-mail.

Other Relationships

Some of the underwriters and their affiliates have engaged in, and may in the future engage in, investment banking and other commercial dealings in the ordinary course of business with us or our affiliates. They have received, or may in the future receive, customary fees and commissions for these transactions. An affiliate of Cowen and Company, LLC served as the placement agent for our Series C convertible preferred stock financing in October 2015.

In addition, in the ordinary course of their business activities, the underwriters and their affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers. Such investments and securities activities may involve securities and/or instruments of ours or our affiliates. The underwriters and their affiliates may also make investment recommendations and/or publish or express independent research views in respect of such securities or financial instruments and may hold, or recommend to clients that they acquire, long and/or short positions in such securities and instruments.

Notice to Prospective Investors in the European Economic Area

In relation to each member state of the European Economic Area, no offer of ordinary shares which are the subject of the offering has been, or will be made to the public in that Member State, other than under the following exemptions under the Prospectus Directive:

(i) to any legal entity which is a qualified investor as defined in the Prospectus Directive;

(ii) to fewer than 150 natural or legal persons (other than qualified investors as defined in the Prospectus Directive), subject to obtaining the prior consent of the Representatives for any such offer; or

(iii) in any other circumstances falling within Article 3(2) of the Prospectus Directive,

provided that no such offer of ordinary shares referred to in (a) to (c) above shall result in a requirement for the company or any representative to publish a prospectus pursuant to Article 3 of the Prospectus Directive, or supplement a prospectus pursuant to Article 16 of the Prospectus Directive.

Each person located in a Member State to whom any offer of ordinary shares is made or who receives any communication in respect of an offer of ordinary shares, or who initially acquires any ordinary shares will be deemed to have represented, warranted, acknowledged and agreed to and with each representative and the company that (i) it is a "qualified investor" within the meaning of the law in that Member State implementing Article 2(1)(e) of the Prospectus Directive; and (ii) in the case of any ordinary shares acquired by it as a financial intermediary as that term is used in Article 3(2) of the Prospectus Directive, the ordinary shares acquired by it in the offer have not been acquired on behalf of, nor have they been acquired with a view to their offer or resale to, persons in any Member State other than qualified investors, as that term is defined in the Prospectus Directive, or in circumstances in which the prior consent of the Representatives has been given to the offer or resale; or where ordinary shares have been acquired by it on behalf of persons in any Member State other than qualified investors, the offer of those ordinary shares to it is not treated under the Prospectus Directive as having been made to such persons.

The company, the representatives and their respective affiliates will rely upon the truth and accuracy of the foregoing representations, acknowledgments and agreements.

This prospectus has been prepared on the basis that any offer of shares in any Member State will be made pursuant to an exemption under the Prospectus Directive from the requirement to publish a prospectus for offers of shares. Accordingly any person making or intending to make an offer in that Member State of shares which are the subject of the offering contemplated in this prospectus may only do so in circumstances in which no obligation arises for the company or any of the representatives to publish a prospectus pursuant to Article 3 of the Prospectus Directive in relation to such offer. Neither the company nor the representatives have authorized, nor do they authorize, the making of any offer of shares in circumstances in which an obligation arises for the company or the representatives to publish a prospectus for such offer.

For the purposes of this provision, the expression an "offer of ordinary shares to the public" in relation to any ordinary shares in any Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the ordinary shares to be offered so as to enable an investor to decide to purchase or subscribe the ordinary shares, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State, the expression "Prospectus Directive" means Directive 2003/71/EC (as amended) and includes any relevant implementing measure in each Member State.

The above selling restriction is in addition to any other selling restrictions set out below.

Notice to Prospective Investors in the United Kingdom

In addition, in the United Kingdom, this document is being distributed only to, and is directed only at, and any offer subsequently made may only be directed at persons who are "qualified investors" (as defined in the Prospectus Directive) (i) who have professional experience in matters relating to investments falling within Article 19 (5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended (the "Order") and/or (ii) who are high net worth companies (or persons to whom it may otherwise be lawfully communicated) falling within Article 49(2)(a) to (d) of the Order (all such persons together being referred to as "relevant persons"). This document must not be acted on or relied on in the United Kingdom by persons who are not relevant persons. In the United Kingdom, any investment or investment activity to which this document relates is only available to, and will be engaged in with, relevant persons.

Notice to Prospective Investors in Switzerland

The shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX, or on any other stock exchange or regulated trading facility in Switzerland. This document has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this document nor any other offering or marketing material relating to the shares or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this document nor any other offering or marketing material relating to the offering, the company or the shares have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, or FINMA, and the offer of shares has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares.

Notice to Prospective Investors in the Dubai International Financial Centre

This prospectus relates to an Exempt Offer in accordance with the Offered Securities Rules of the Dubai Financial Services Authority, or DFSA. This prospectus is intended for distribution only to persons of a type specified in the Offered Securities Rules of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus nor taken steps to verify the information set forth herein and has no responsibility for the prospectus. The shares to which this prospectus relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the shares offered should conduct their own due diligence on the shares. If you do not understand the contents of this prospectus you should consult an authorized financial advisor.

Notice to Prospective Investors in Australia

No placement document, prospectus, product disclosure statement or other disclosure document has been lodged with the Australian Securities and Investments Commission, or ASIC, in relation to the offering. This prospectus does not constitute a prospectus, product disclosure statement or other disclosure document under the Corporations Act 2001, or Corporations Act, and does not purport to include the information required for a prospectus, product disclosure statement or other disclosure document under the Corporations Act.

Any offer in Australia of the shares may only be made to persons, or Exempt Investors, who are "sophisticated investors" (within the meaning of section 708(8) of the Corporations Act), "professional investors" (within the meaning of section 708(11) of the Corporations Act) or otherwise pursuant to one or more exemptions contained in section 708 of the Corporations Act so that it is lawful to offer the shares without disclosure to investors under Chapter 6D of the Corporations Act.

The shares applied for by Exempt Investors in Australia must not be offered for sale in Australia in the period of 12 months after the date of allotment under the offering, except in circumstances where disclosure to investors under Chapter 6D of the Corporations Act would not be required pursuant to an exemption under section 708 of the Corporations Act or otherwise or where the offer is pursuant to a disclosure document which complies with Chapter 6D of the Corporations Act. Any person acquiring shares must observe such Australian on-sale restrictions.

This prospectus contains general information only and does not take account of the investment objectives, financial situation or particular needs of any particular person. It does not contain any securities recommendations or financial product advice. Before making an investment decision, investors need to consider whether the information in this prospectus is appropriate to their needs, objectives and circumstances, and, if necessary, seek expert advice on those matters.

Notice to Prospective Investors in Hong Kong

The shares have not been offered or sold and will not be offered or sold in Hong Kong, by means of any document, other than (a) to "professional investors" as defined in the Securities and Futures Ordinance (Cap. 571) of Hong Kong and any rules made under that Ordinance; or (b) in other circumstances which do not result in the document being a "prospectus" as defined in the Companies Ordinance (Cap. 32) of Hong Kong or which do not constitute an offer to the public within the meaning of that Ordinance. No advertisement, invitation or document relating to the shares has been or may be issued or has been or may be in the possession of any person for the purposes of issue, whether in Hong Kong or elsewhere, which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" as defined in the Securities and Futures Ordinance and any rules made under that Ordinance.

Notice to Prospective Investors in Japan

The shares have not been and will not be registered under the Financial Instruments and Exchange Law of Japan (Law No. 25 of 1948, as amended) and, accordingly, will not be offered or sold, directly or indirectly, in Japan, or for the benefit of any Japanese Person or to others for re-offering or resale, directly or indirectly, in Japan or to any Japanese Person, except in compliance with all applicable laws, regulations and ministerial guidelines promulgated by relevant Japanese governmental or regulatory authorities in effect at the relevant time. For the purposes of this paragraph, "Japanese Person" shall mean any person resident in Japan, including any corporation or other entity organized under the laws of Japan.

Notice to Prospective Investors in Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore, or SFA, (ii) to a relevant person pursuant to Section 275(1), or any person pursuant to Section 275(1A), and in accordance with the conditions specified in Section 275, of the SFA, or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

(i) a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or

(ii) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor,

securities (as defined in Section 239(1) of the SFA) of that corporation or the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the shares pursuant to an offer made under Section 275 of the SFA except:

(i) to an institutional investor or to a relevant person defined in Section 275(2) of the SFA, or to any person arising from an offer referred to in Section 275(1A) or Section 276(4)(i)(B) of the SFA;

(ii) where no consideration is or will be given for the transfer;

(iii) where the transfer is by operation of law;

(iv) as specified in Section 276(7) of the SFA; or

(v) as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore.

Notice to Prospective Investors in Canada

The shares may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 *Prospectus Exemptions* or subsection 73.3(1) of the *Securities Act* (Ontario), and are permitted clients, as defined in National Instrument 31-103 *Registration*

Requirements, Exemptions and Ongoing Registrant Obligations. Any resale of the shares must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 (or, in the case of securities issued or guaranteed by the government of a non-Canadian jurisdiction, section 3A.4) of National Instrument 33-105 *Underwriting Conflicts* (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

LEGAL MATTERS

The validity of the shares of common stock offered by this prospectus will be passed upon for us by Fenwick & West LLP, San Francisco, California. Certain legal matters relating to this offering will be passed upon for the underwriters by Cooley LLP, San Francisco, California.

EXPERTS

The financial statements of Audentes Therapeutics, Inc. at December 31, 2014 and 2015, and for each of the years in the two-year period ended December 31, 2015, are included herein in reliance upon the report of KPMG LLP, independent registered public accounting firm, appearing elsewhere herein, and upon the authority of said firm as experts in accounting and auditing.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

We have filed with the Securities and Exchange Commission, or the SEC, a registration statement on Form S-1 under the Securities Act with respect to the shares of common stock offered hereby. This prospectus, which constitutes a part of the registration statement, does not contain all of the information set forth in the registration statement or the exhibits filed therewith. For further information about us and the common stock offered hereby, reference is made to the registration statement and the exhibits filed therewith. Statements contained in this prospectus regarding the contents of any contract or any other document that is filed as an exhibit to the registration statement are not necessarily complete, and in each instance we refer you to the copy of such contract or other document filed as an exhibit to the registration statement. A copy of the registration statement and the exhibits filed therewith may be inspected without charge at the public reference room maintained by the SEC, located at 100 F Street, NE, Washington, DC 20549, and copies of all or any part of the registration statement may be obtained from that office. Please call the SEC at 1-800-SEC-0330 for further information about the public reference room. The SEC also maintains a website that contains reports, proxy and information statements and other information regarding registrants that file electronically with the SEC. The address of the website is www.sec.gov.

We currently do not file periodic reports with the SEC. Upon the closing of our initial public offering, we will be required to file periodic reports, proxy statements and other information with the SEC pursuant to the Exchange Act. These periodic reports, proxy statements and other information will be available for inspection and copying at the SEC's public reference facilities and the website of the SEC referred to above.

We also maintain a website at www.audentestx.com. Upon completion of this offering, you may access these materials at our website free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. Information contained on our website is not a part of this prospectus and the inclusion of our website address in this prospectus is an inactive textual reference only.

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INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

Report of Independent Registered Public Accounting Firm

The Board of Directors
Audentes Therapeutics, Inc.:

We have audited the accompanying consolidated balance sheets of Audentes Therapeutics, Inc. and subsidiary as of December 31, 2014 and 2015, and the related consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows for each of the years in the two-year period ended December 31, 2015. These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits.

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

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of Audentes Therapeutics, Inc. and subsidiary as of December 31, 2014 and 2015, and the results of their operations and their cash flows for each of the years in the two-year period ended December 31, 2015, in conformity with U.S. generally accepted accounting principles.

/s/ KPMG LLP

San Francisco, California
March 9, 2016, except for the second paragraph of Note 15, as to which the date is July 7, 2016

AUDENTES THERAPEUTICS, INC.
Consolidated Balance Sheets
(in thousands, except shares and per share data)

	December 31, 2014	December 31, 2015
Assets		
Current assets:		
Cash and cash equivalents	$ 45,599	$ 72,058
Short-term investments	14,851	23,169
Restricted cash	50	730
Prepaid expenses and other current assets	441	3,682
Total current assets	60,941	99,639
Restricted cash-long-term	—	2,930
Property and equipment, net	264	2,968
Goodwill	—	3,631
Intangible assets	—	8,000
Long-term investments	1,698	—
Other assets	106	301
Total assets	$ 63,009	$117,469
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,240	$ 2,789
Accrued liabilities	1,871	4,797
Facility lease obligations	—	137
Total current liabilities	3,111	7,723
Facility lease obligations	34	519
Contingent acquisition consideration payable	—	4,278
Deferred tax liability, net	—	3,260
Total liabilities	3,145	15,780
Stockholders' equity:		
Convertible preferred stock, Series Seed, $0.00001 par value: 1,400,000 shares authorized as of December 31, 2014 and 2015; 627,867 shares issued and outstanding as of December 31, 2014 and 2015, aggregate liquidation preference of $1,400 as of December 31, 2014 and 2015	1,378	1,378
Convertible preferred stock, Series A, $0.00001 par value: 11,199,876 shares authorized as of December 31, 2014 and 2015; 5,022,876 shares issued and outstanding as of December 31, 2014 and 2015; aggregate liquidation preference of $30,000 as of December 31, 2014 and 2015	28,757	28,757
Convertible preferred stock, Series B, $0.00001 par value: 8,500,000 and 8,570,366 shares authorized as of December 31, 2014 and 2015, respectively; 3,796,635 and 3,843,604 shares issued and outstanding as of December 31, 2014 and 2015, respectively; aggregate liquidation preference of $42,500 and $43,026 as of December 31, 2014 and 2015, respectively	42,268	42,835
Convertible preferred stock, Series C, $0.00001 par value: zero and 9,684,789 shares authorized as of December 31, 2014 and 2015, respectively; zero and 4,325,954 shares issued and outstanding as of December 31, 2014 and 2015, respectively; aggregate liquidation preference of zero and $65,000 as of December 31, 2014 and 2015, respectively	—	62,780
Common stock, $0.00001 par value, 28,000,000 and 50,000,000 shares authorized as of December 31, 2014 and 2015, respectively; 761,100 and 2,106,152 shares issued and outstanding as of December 31, 2014 and 2015, respectively	—	—
Additional paid-in capital	1,756	6,692
Accumulated deficit	(14,285)	(40,743)
Accumulated other comprehensive loss	(10)	(10)
Total stockholders' equity	59,864	101,689
Total liabilities and stockholders' equity	$ 63,009	$117,469

See accompanying notes to consolidated financial statements.

AUDENTES THERAPEUTICS, INC.
Consolidated Statements of Operations and Comprehensive Loss
(in thousands)

	Year Ended December 31,	
	2014	2015
Operating expenses:		
Research and development	$ 9,280	$ 20,235
General and administrative	1,670	6,491
Total operating expenses	10,950	26,726
Loss from operations	(10,950)	(26,726)
Interest income	6	245
Other income, net	125	23
Net loss	(10,819)	(26,458)
Unrealized losses on available-for-sale securities	(10)	—
Net loss and comprehensive loss	$ (10,829)	$ (26,458)
Net loss per share, basic and diluted	$ (21.56)	$ (23.03)
Shares used in computing net loss per share, basic and diluted	501,707	1,148,827
Pro forma net loss per share, basic and diluted (unaudited)		$ (2.28)
Shares used in computing pro forma net loss per share, basic and diluted		11,621,249

See accompanying notes to consolidated financial statements.

AUDENTES THERAPEUTICS, INC.
Consolidated Statements of Stockholders' Equity
Years ended December 31, 2014 and 2015
(in thousands, except shares)

	Series Seed Convertible Preferred Stock		Series A Convertible Preferred stock		Series B Convertible Preferred Stock		Series C Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Loss	Stockholders' Equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at January 1, 2014	627,867	$1,378	2,511,435	$13,759	—	$ —	—	$ —	498,704	$—	$1,241	$ (3,466)	$—	$ 12,912
Issuance of common stock to Genethon (see note 7)	—	—	—	—	—	—	—	—	262,396	—	336	—	—	336
Issuance of Series A convertible preferred stock, net of $2 in issuance costs	—	—	2,511,441	14,998	—	—	—	—	—	—	—	—	—	14,998
Issuance of Series B convertible preferred stock, net of $217 in issuance costs	—	—	—	—	3,796,635	42,268	—	—	—	—	—	—	—	42,268
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	179	—	—	179
Net loss	—	—	—	—	—	—	—	—	—	—	—	(10,819)	—	(10,819)
Other comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	—	(10)	(10)
Balance at December 31, 2014	627,867	1,378	5,022,876	28,757	3,796,635	42,268	—	—	761,100	—	1,756	(14,285)	(10)	59,864
Issuance of common stock for acquisition of business (see note 6)	—	—	—	—	—	—	—	—	1,293,058	—	3,575	—	—	3,575
Exercise of stock options	—	—	—	—	—	—	—	—	51,994	—	70	—	—	70
Issuance of Series B convertible preferred stock, for acquisition of business (see Note 6)	—	—	—	—	46,969	567	—	—	—	—	—	—	—	567
Issuance of Series C convertible preferred stock, net of $2,208 in issuance costs	—	—	—	—	—	—	4,325,954	62,780	—	—	—	—	—	62,780
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	1,291	—	—	1,291
Net loss	—	—	—	—	—	—	—	—	—	—	—	(26,458)	—	(26,458)
Balance at December 31, 2015	627,867	$1,378	5,022,876	$28,757	3,843,604	$42,835	4,325,954	$62,780	2,106,152	$—	$6,692	$(40,743)	$ (10)	$101,689

See accompanying notes to consolidated financial statements.

AUDENTES THERAPEUTICS, INC.
Consolidated Statements of Cash Flows
(in thousands)

	Year Ended December 31,	
	2014	2015
Cash flows from operating activities:		
Net loss	$(10,819)	$(26,458)
Adjustments to reconcile net loss to net cash used in operating activities:		
Amortization of discount on investments	—	660
Depreciation and amortization	35	166
Stock-based compensation	515	1,291
Accretion of asset retirement obligation	—	136
Non-cash change in fair value of contingent acquisition consideration payable	—	131
Other	(9)	—
Changes in operating assets and liabilities:		
Restricted cash	—	(3,610)
Prepaid expenses and other current assets	(300)	(3,188)
Other assets	(36)	(195)
Accounts payable	857	628
Accrued liabilities	1,683	2,634
Facility lease obligations	5	290
Net cash used in operating activities	(8,069)	(27,515)
Cash flows from investing activities:		
Cash acquired in business acquisition	—	142
Purchases of property and equipment	(125)	(1,686)
Proceeds from sales and maturities of marketable securities	—	32,792
Purchases of marketable securities	(16,539)	(40,124)
Net cash used in investing activities	(16,664)	(8,876)
Cash flows from financing activities:		
Proceeds from issuance of Series A convertible preferred stock, net of issuance costs	14,998	—
Proceeds from issuance of Series B convertible preferred stock, net of issuance costs	42,388	—
Proceeds from issuance of Series C convertible preferred stock, net of issuance costs	—	62,780
Proceeds from exercises of stock options	—	70
Net cash provided by financing activities	57,386	62,850
Net increase in cash and cash equivalents	32,653	26,459
Cash and cash equivalents at beginning of period	12,946	45,599
Cash and cash equivalents at end of period	$ 45,599	$ 72,058
Noncash investing and financing activities:		
Increase (decrease) in accounts payable, facility lease obligations and accrued liabilities related to property and equipment purchases	$ 65	$ 1,140
Preferred stock issuance costs	$ (120)	$ —

See accompanying notes to consolidated financial statements.

AUDENTES THERAPEUTICS, INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Organization and Basis of Presentation

Audentes Therapeutics, Inc., or the Company, was incorporated in the State of Delaware on November 13, 2012. The Company is a biotechnology company focused on developing and commercializing gene therapy products for patients suffering from serious, life-threatening rare diseases caused by single gene defects. The Company's principal operations are located in San Francisco, California, and it operates in one business segment.

The accompanying consolidated financial statements include the accounts of Audentes Therapeutics, Inc., and its wholly owned subsidiary, Audentes Therapeutics UK Ltd. All intercompany balances and transactions have been eliminated in consolidation.

Liquidity

In the course of its development activities, the Company has sustained operating losses and expects such losses to continue over the next several years. The Company's ultimate success depends on the outcome of its research and development activities. The Company has incurred net losses from operations since inception and had an accumulated deficit of $40.7 million, as of December 31, 2015. The Company intends to raise additional capital through the issuance of additional equity, and potentially through strategic alliances with partner companies. If financing is not available at adequate levels, the Company may need to reevaluate its operating plans. Management believes currently available resources will provide sufficient funds to enable the Company to meet its operating plans for at least the next twelve months. However, if the Company's anticipated operating results are not achieved in future periods, planned expenditures may need to be reduced in order to extend the time period over which the then-available resources would be able to fund the Company's operations.

2. Summary of Significant Accounting Policies

Use of Estimates

The accompanying consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles, or GAAP. The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities, as of the date of the financial statements, and the reported amounts of any expenses during the reporting period. On an ongoing basis, management evaluates its estimates, including those related to accrued liabilities, fair value of assets, common stock, income taxes, and stock-based compensation. Management bases its estimates on historical experience, and on various other market-specific relevant assumptions that management believes to be reasonable, under the circumstances. Actual results may differ from those estimates.

Reclassifications

The Company has made certain reclassifications to the consolidated financial statements and related disclosures for the year ended December 31, 2014 to conform to current period presentation. These reclassifications had no impact on previously reported consolidated comprehensive loss.

Financial Instruments

The following methods were used to estimate the fair value of each class of financial instrument:

Cash and cash equivalents: Cash and cash equivalents consist of bank deposits, money market funds and commercial money market instruments with original maturities of three months or less used for operational purposes. Cash equivalents are recorded at fair value.

Short-term and long-term investments: Short-term investments consist of debt securities with original maturities of 12 months or less and greater than three months. Long-term investments consist of debt securities with maturities greater than 12 months. Short-term and long-term investments are classified as available-for-sale investments and are recognized at fair value.

Restricted Cash: Restricted cash consists of cash pledged as security for letters of credit and is typically held in money market funds.

The Company regularly reviews its investment portfolio to identify and evaluate investments that have indications of possible impairment. Factors considered in determining whether a loss is other-than-temporary include: the length of time and extent to which the fair market value has been lower than the cost basis, the financial condition and near-term prospects of the investee, credit quality, likelihood of recovery, and the Company's ability to hold the investment for a period of time sufficient to allow for any anticipated recovery in fair market value. Unrealized gains and losses, net of tax, are included in accumulated other comprehensive loss as a separate component of stockholders' equity. The amortization of premiums and discounts on the investments, and realized gains and losses on available-for-sale securities are included in other income, net on the statements of operations and comprehensive loss. The Company uses the specific-identification method to determine cost in calculating realized gains and losses upon sale of its marketable securities.

Fair Value Measurements

Fair value is defined as the price at which an asset could be exchanged in a current transaction between knowledgeable, willing parties. A liability's fair value is defined as the amount that would be paid to transfer the liability to a new obligor, not the amount that would be paid to settle the liability with the creditor. Where available, fair value is based on observable market prices, or parameters derived from such prices. Where observable prices or inputs are not available, valuation models are applied. These valuation techniques involve some level of management estimation and judgment. The degree of management estimation and judgment is dependent on the price transparency for the instruments, or market, and the instruments' complexity. The authoritative accounting guidance describes a fair value hierarchy based on three levels of inputs that may be used to measure fair value, of which the first two are considered observable and the last is considered unobservable. These levels of inputs are as follows:

Level 1—Observable inputs such as unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date.

Level 2—Inputs (other than quoted prices included in Level 1) are either directly or indirectly observable for the asset or liability. These include quoted prices for similar assets or liabilities in active markets and quoted prices for identical or similar assets or liabilities in markets that are not active.

Level 3—Unobservable inputs that reflect management's best estimate of what market participants would use in pricing the asset or liability at the measurement date. Consideration is given to the risk inherent in the valuation technique and the risk inherent in the inputs to the model.

Money market funds are valued using quoted market price, and are included in cash equivalents on the Company's balance sheets. Marketable securities are valued using quoted prices for similar assets or liabilities in active markets and quoted prices for identical or similar assets or liabilities in markets that are not active, and are included in cash equivalents, short-term investments or long-term investments on the Company's consolidated balance sheets.

Restricted Cash

Restricted cash consists of money market funds held by the Company's financial institution as collateral for the Company's obligations under its facility leases and is classified as current or long-term depending on the lease requirements for the respective letters of credit.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash and cash equivalents. The Company's cash and cash equivalents are held by a financial institution in the United States. Amounts on deposit may at times exceed federally insured limits. Management believes that the financial institution is financially sound, and accordingly, has concluded that minimal credit risk exists with respect to the financial institution.

Concentration of Manufacturing Risk

As of December 31, 2015, the Company had manufacturing arrangements with Genethon located in Evry, France, and the University of Florida for the supply of materials for use in preclinical and future clinical studies. If the Company were to experience any disruptions in either party's ability or willingness to continue to provide manufacturing services, the Company may experience significant delays in its product development timelines and may incur substantial costs to secure alternative sources of manufacturing.

Deferred Offering Costs

Deferred offering costs, consisting of legal, accounting, printer and filing fees related to the proposed initial public offering, or IPO, are capitalized. The deferred offering costs will be offset against proceeds from the IPO upon completion of the offering. In the event the offering is terminated, all capitalized deferred offering costs will be expensed. As of December 31, 2015, $2.3 million of deferred offering costs were capitalized, which are included in prepaid expenses and other current assets in the accompanying consolidated balance sheets. No offering costs were deferred as of December 31, 2014.

Property and Equipment

Property and equipment are stated at cost, less accumulated depreciation and amortization. Depreciation and amortization are computed using the straight-line method over the estimated useful lives of the respective assets. Depreciation and amortization begins at the time the asset is placed into service. Maintenance and repairs are charged to operations as incurred. Upon sale or retirement of assets, the cost and related accumulated depreciation and amortization are removed from the consolidated balance sheets and the resulting gain or loss is reflected in operations.

The estimated useful lives of property and equipment are as follows:

Research and development equipment	5 years
Furniture and office equipment	5 years
Computer equipment	3 years
Software	3 years
Leasehold improvements	Shorter of remaining lease term or estimated useful life

Impairment of Long-Lived Assets

The Company evaluates its long-lived assets, including property and equipment, for impairment whenever events or changes in circumstances indicate that the carrying value of these assets may not be recoverable. Recoverability of these assets is measured by comparison of the carrying amount of each asset to the future undiscounted cash flows the asset is expected to generate over its remaining life. If the asset is considered to be impaired, the amount of any impairment is measured as the difference between the carrying value and the fair value of the impaired asset. The Company has recorded no impairment of any long-lived assets during any of the periods presented.

The Company records goodwill in a business combination when the total consideration exceeds the fair value of the net tangible and identifiable intangible assets acquired. Goodwill and intangible assets with indefinite lives are not amortized but are subject to an annual impairment analysis.

The Company performs its annual impairment review of goodwill and indefinite lived intangibles during December and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. If it is determined that the full carrying amount of an asset is not recoverable, an impairment loss is recorded in the amount by which the carrying amount of the asset exceeds its fair value. The Company has recorded no impairment of any long-lived assets during any of the periods presented.

As of December 31, 2015, the Company had only one reporting unit.

Business Combinations

The Company allocates the purchase price of acquired businesses to the tangible and intangible assets acquired and liabilities assumed based upon their estimated fair values on the acquisition date. The purchase price allocation process requires management to make estimates and assumptions, notably at the acquisition date with respect to intangible assets and in-process research and development.

Contingent Consideration Payable

The Company determines the fair value of contingent consideration payable on the acquisition date using a probability-based income approach utilizing an appropriate discount rate. Each reporting period thereafter, the Company revalues these obligations and records increases or decreases in their fair value as adjustments to research and development expense. Changes in the fair value of the contingent consideration payable can result from adjustments to the estimated probability and assumed timing of achieving the underlying milestones, as well as from changes to the discount rates.

Accrued Research and Development Costs

The Company accrues for estimated costs of research and development activities conducted by third-party service providers, which include the conduct of preclinical studies and clinical trials, and contract manufacturing activities. The Company records the estimated costs of research and development activities based upon the estimated amount of services provided, and includes these costs in accrued liabilities in the consolidated balance sheets and within research and development expense in the consolidated statements of operations and comprehensive loss. These costs are a significant component of the Company's research and development expenses. The Company accrues for these costs based on factors such as estimates of the work completed and in accordance with agreements established with its third-party service providers. The Company makes significant judgments and estimates in determining the accrued liabilities balance in each reporting period.

Research and Development Costs

Research and development costs are expensed as incurred and consist primarily of personnel and consultant costs, lab supplies, allocated facility and other costs, fees paid to third parties to conduct research and development activities on the Company's behalf and expenses incurred in connection with license agreements.

Facility Lease Obligations

Rent expense is recognized on a straight-line basis over the non-cancelable term of the Company's operating leases and, accordingly, the Company records the difference between cash rent payments and the recognition of rent expense as a deferred rent asset or liability. Incentives granted under the Company's facility leases, including any allowances to fund leasehold improvements, are deferred and recognized as adjustments to rent expense on a straight-line basis over the term of the lease.

Under the terms of its sublease for manufacturing facilities, the Company assumed a restoration obligation from the previous tenant. The liability is being accreted to rent expense through the end of the lease term. In addition, the Company received approximately $0.2 million of laboratory equipment for de minimis consideration. This amount has been recorded in property and equipment and will be depreciated when placed in service. The related liability will be amortized over the remaining lease term as a reduction to rent expense.

Stock-Based Compensation

Stock-based awards issued to employees and directors, including stock options, are recorded at fair value as of the grant date using the Black-Scholes option pricing model and recognized as expense on a straight line-basis over the employee's or director's requisite service period (generally the vesting period). Because non-cash stock compensation expense is based on awards ultimately expected to vest, it is reduced by an estimate for future forfeitures. Forfeitures are estimated at the time of grant and revised, if necessary, in subsequent periods if actual forfeitures differ from estimates.

Stock-based awards and stock options issued to non-employees are recorded at fair value and remeasured at the end of each period as they vest using the Black-Scholes option pricing model. Expense is recognized over the vesting period which is generally the same as the service period.

Income Taxes

The Company uses the asset and liability method of accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on the differences between the financial reporting and the

tax basis of assets and liabilities, and are measured using the enacted tax rates and laws that will be in effect when the differences are expected to reverse. The Company must then assess the likelihood that the resulting deferred tax assets will be realized. A valuation allowance is provided when it is more likely than not that some portion, or all of a deferred tax asset will not be realized. Due to the Company's lack of earnings history, the net deferred tax assets have been fully offset by a valuation allowance.

The Company recognizes benefits of uncertain tax positions if it is more likely than not that such positions will be sustained upon examination based solely on their technical merits, as the largest amount of benefit that is more likely than not to be realized upon the ultimate settlement. The Company's policy is to recognize interest and penalties related to the underpayment of income taxes as a component of income tax expense or benefit. To date, there have been no interest charges or penalties related to unrecognized tax benefits.

Interest Income

Interest income consists of interest earned from investments and money market accounts.

Other Income

Other income consists primarily of foreign currency gains and losses.

Net Loss per Share

Basic net loss per share is calculated by dividing the net loss by the weighted average number of shares of common stock outstanding during the period without consideration of common stock equivalents. Diluted net loss per common share is the same as basic net loss per common share, since the effects of potentially dilutive securities are antidilutive.

Pro Forma Net Loss per Share

Pro forma basic and diluted net loss per share has been computed to give effect to the assumed conversion of the shares of convertible preferred stock into common stock as if such conversions had occurred at the beginning of the period. The pro forma net loss per share does not include the shares expected to be sold and related proceeds to be received from an initial public offering.

Recent Accounting Pronouncements

In April 2015, the FASB issued ASU 2015-05, *Customer's Accounting for Fees Paid in a Cloud Computing Arrangement,* which provides explicit guidance to help companies evaluate the accounting for fees paid by a customer in a cloud computing arrangement. The new guidance clarifies that if a cloud computing arrangement includes a software license, the customer should account for the license consistent with its accounting for other software licenses. If the arrangement does not include a software license, the customer should account for the arrangement as a service contract. ASU 2015-05 is effective for public business entities for the fiscal years, and the interim reporting periods within those years beginning after December 15, 2015. An entity can elect to adopt the amendments either prospectively or retrospectively. Early adoption is permitted for all entities. The Company does not anticipate adoption of this guidance to have a material impact to its financial statements or disclosures.

In September 2015, the FASB issued ASU No. 2015-16, *Simplifying the Accounting for Measurement-Period Adjustments* (ASU 2015-16). The amended guidance requires that an acquirer recognize adjustments to

provisional amounts that are identified during the measurement period in the reporting period in which the adjustment amounts are determined. The amendments are effective prospectively for the fiscal years, and the interim reporting periods within those years, beginning on or after December 15, 2015 and early adoption is permitted. The Company has elected to early adopt ASU 2015-16. The guidance did not have a material impact to the Company's financial statements or disclosures.

In November 2015 the FASB issued ASU 2015-17*, Balance Sheet Classification of Deferred Taxes* (ASU 2015-17), which requires that deferred tax liabilities and assets be classified as noncurrent in a classified statement of financial position. ASU is effective for the Company in its first quarter of fiscal 2017, with early application permitted and, upon adoption, may be applied either prospectively or retrospectively. The Company has elected to early adopt ASU 2015-17 and applied its provisions retrospectively in the accompanying consolidated financial statements. This guidance did not have a material impact to the Company's financial statements or disclosures.

In January 2016, the FASB issued ASU No. 2016-01, *Financial Instruments - Overall* (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities (ASU 2016-01). ASU 2016-01 addresses certain aspects of recognition, measurement, presentation, and disclosure of financial instruments. ASU 20196-01 is effective for fiscal years, and interim periods within those years, beginning after December 15, 2017, which for the Company is January 1, 2018. The Company is currently evaluating the impact that the standard will have on its consolidated financial statements.

In February 2016, the FASB issued ASU No. 2016-02, *Leases* (Topic 842). Under the new guidance, (with the exception of short-term leases) at the commencement date, lessees will be required to recognize a lease liability and a right-of-use asset. Lessor accounting is largely unchanged, while lessees will no longer be provided with a source of off-balance sheet financing. Public business entities should apply the amendments in ASU 2016-02 for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years (January 1, 2019, for the Company). Early application is permitted. Lessees (for capital and operating leases) must apply a modified retrospective transition approach for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements. The modified retrospective approach would not require any transition accounting for leases that expired before the earliest comparative period presented. The Company is currently evaluating the impact that the standard will have on its consolidated financial statements.

3. Cash Equivalents and Available for Sale Securities

The fair value and amortized cost of cash equivalents and available-for-sale debt securities by major security type as of December 31, 2014 and 2015 are presented in the tables that follow:

	December 31, 2014			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Market Value
	(in thousands)			
Money market funds	$36,989	$ —	$ —	$36,989
Commercial paper	598	—	—	598
Corporate debt securities	19,281	1	(11)	19,271
Total cash equivalents and available-for sale securities	$56,868	$ 1	$ (11)	$56,858

	December 31, 2015			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Market Value
	(in thousands)			
Money market funds	$19,787	$ —	$ —	$19,787
Commercial paper	3,996	—	—	3,996
Corporate debt securities	16,548	—	(8)	16,540
U.S. Gov't Agency Securities	4,016	—	(2)	4,014
Total cash equivalents and available-for sale securities	$44,347	$ —	$ (10)	$44,337

Realized gains and losses on the sale of marketable securities during the years ended December 31, 2014 and 2015 were not material.

The following table summarizes the amortized cost and estimated fair value of investments in marketable securities designated as available-for-sale and classified by the contractual maturity date of the security for the years ended December 31, 2014 and 2015:

	December 31, 2014			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Market Value
	(in thousands)			
Less than one year	$55,167	$ 1	$ (8)	$55,160
1 – 2 years	1,701	—	(3)	1,698
Total cash equivalents and available-for sale securities	$56,868	$ 1	$ (11)	$56,858

	December 31, 2015			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Market Value
Less than one year	$44,347	$ —	$ (10)	$44,337
Total cash equivalents and available-for sale securities	$44,347	$ —	$ (10)	$44,337

4. Fair Value Measurements

Assets Measured at Fair Value

The Company's financial instruments are valued using quoted prices in active markets or based upon other observable inputs. The following tables set forth the fair value of the Company's financial assets as of the years ended December 31, 2014 and 2015:

	December 31, 2014			
		Fair Value Measurements Using		
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$36,989	$36,989	$ —	$ —
Commercial paper	598	—	598	—
Corporate debt securities	19,271	—	19,271	—
Total financial assets	$56,858	$36,989	$19,869	$ —

	December 31, 2015			
		Fair Value Measurements Using		
	Total	Level 1	Level 2	Level 3
		(in thousands)		
Money market funds	$19,787	$19,787	$ —	$ —
Commercial paper	3,996	—	3,996	—
Corporate debt securities	16,540	—	16,540	—
U.S. Gov't Agency Securities	4,014	—	4,014	—
Total financial assets	$44,337	$19,787	$24,550	$ —

Liabilities Measured at Fair Value

The Company's financial liabilities are valued based upon observable inputs when available or upon estimates made by management. The following table sets forth the fair value of the Company's financial liabilities as of December 31, 2015 (there were no financial liabilities as of December 31, 2014):

	December 31, 2015			
		Fair Value Measurements Using		
	Total	Level 1	Level 2	Level 3
		(in thousands)		
Contingent acquisition consideration payable	$ 4,278	$ —	$ —	$ 4,278
Asset retirement obligation	136	—	—	136
Total financial liabilities	$ 4,414	$ —	$ —	$ 4,414

The Company's contingent acquisition consideration payable (resulting from the Cardiogen acquisition (see Note 6)) is estimated using a probability-based income approach utilizing an appropriate discount rate. Key assumptions used by management to estimate the fair value of contingent acquisition consideration payable include estimated probabilities, the estimated timing of when a milestone may be attained and assumed discount periods and rates. Subsequent changes in the fair value of the contingent acquisition consideration payable, resulting from management's revision of key assumptions, will be recorded in research and development expense in the accompanying consolidated statement of operations and comprehensive loss. The probability-based income approach used by management to estimate the fair value of the contingent acquisition consideration is most sensitive to changes in the estimated probabilities.

The following is a summary of the contingent acquisition consideration payable, recorded as a non-current liability in the accompanying consolidated balance sheets:

	(In thousands)
Balance, December 31, 2014	$ —
Fair value of contingent payments at acquisition	4,147
Addition of contingent acquisition consideration payable related to the Cardiogen acquisition	131
Balance, December 31, 2015	$4,278

Under the terms of its sublease for manufacturing facilities, the Company assumed a restoration obligation from the previous tenant. The liability is being accreted to rent expense through the end of the lease term. The asset retirement obligation is included in facilities lease obligations in the accompanying consolidated balance sheets.

	(In thousands)
Balance, December 31, 2014	$ —
Accretion expense	136
Balance, December 31, 2015	$ 136

5. Balance Sheet Components

Property and Equipment, Net

Property and equipment, net, consist of the following:

	December 31,	
	2014	2015
	(in thousands)	
Furniture and office equipment	$ 165	$ 168
Computer equipment	67	67
Software	5	87
Leasehold improvements	65	64
Laboratory equipment	—	723
Construction in progress	—	2,063
Total property and equipment	302	3,172
Less accumulated depreciation and amortization	(38)	(204)
Property and equipment, net	$ 264	$2,968

Property and equipment depreciation expense for the years ended December 31, 2014 and 2015 was approximately $35,000 and $166,000, respectively.

Accrued Liabilities

Accrued liabilities consist of the following:

	December 31,	
	2014	2015
	(in thousands)	
Accrued payroll and related expenses	$ 518	$1,152
Accrued research and development expenses	1,204	2,682
Other	149	963
Total accrued liabilities	$1,871	$4,797

Facility Lease Obligations

Facility lease obligations consist of the following as of December 31, 2014 and 2015:

	2014			2015		
	Long-term	Current	Total	Long-term	Current	Total
Equipment purchase obligation	$ —	$ —	$ —	$ 44	$ 107	$ 151
Asset retirement obligation	—	—	—	136	—	136
Deferred rent	34	—	34	339	30	369
	$ 34	$ —	$ 34	$ 519	$ 137	$ 656

6. Business Combination

In August 2015, the Company acquired Cardiogen Sciences, Inc., or Cardiogen, a biotechnology company focused on the discovery and development of AAV gene therapy products for rare, inherited arrhythmogenic diseases. As consideration for the acquisition, the Company issued 1,293,058 shares of common stock, of which 133,986 shares have been held back from the Cardiogen shareholders to cover potential indemnification requirements, as specified in the merger agreement, and 46,969 shares of Series B preferred stock. Additionally, upon first dosing of a patient in a human clinical study involving AT307 for the treatment of CASQ2-CPVT, the Company is obligated to pay to former Cardiogen shareholders $4.2 million in common stock plus an additional $5.8 million in either cash or common stock, at the Company's election, for aggregate contingent consideration of $10.0 million.

The acquisition of Cardiogen was accounted for as the purchase of a business. The related acquisition costs, consisting primarily of legal and accounting expenses in the amount of $0.4 million for the year ended December 31, 2015, were expensed. These legal and accounting expenses are included in general and administrative expenses on the consolidated statements of operations and comprehensive loss for the year ended December 31, 2015.

The following is the total consideration paid for the business combination:

	Amount (in thousands)
Fair value of shares issued	$4,142
Fair value of contingent payments	4,147
Total consideration	$8,289

The estimated fair value of the shares issued was determined by the Company's board of directors. The estimated fair value of the contingent payments is based on the risk adjusted present value of management's estimated likelihood and timing of the first dosing of a patient in a human clinical study involving AT307.

In connection with the Company's acquisition of Cardiogen, it acquired a license agreement previously entered into by Cardiogen with Fondazione Salvatore Maugeri, or FSM, an Italian non-profit organization. Under the license agreement, the Company obtained an exclusive worldwide license to certain intellectual property to develop, use and commercialize products related to recessive CPVT, as well as to several additional inherited arrhythmias. The Company may terminate the license agreement with FSM for convenience upon prior written

notice. Either party may terminate the agreement upon prior written notice for the uncured material breach of the agreement by the other party, or the other party's bankruptcy or liquidation.

The acquisition of Cardiogen provided the Company with certain intellectual property through the license agreement with FSM, including Cardiogen's lead program for CASQ2-CPVT. The Company determined that the fair value of such intellectual property was approximately $8.0 million. The fair value of the intellectual property was determined using the excess earnings approach. The excess earnings approach considers the economics related to the intellectual property. The assumptions underlying the fair value calculation include: estimated revenue attributable to the intellectual property, future research and development expenses, an estimated effective income tax rate and an estimated discount rate.

Primarily as a result of the deferred tax liability recognized in the acquisition, the Company recognized goodwill of $3.6 million equal to the excess of the purchase consideration over the fair value of the assets acquired and liabilities assumed. See Note 2 for accounting policies for goodwill, intangible assets and contingent consideration payable.

The following table summarizes the allocation of the consideration paid of $8.3 million to the fair values of the assets acquired and liabilities assumed at the acquisition date:

	Amount (in thousands)
In process research and development	$ 8,000
Deferred tax liability	(3,260)
Goodwill	3,631
Liabilities assumed (net of cash acquired)	(82)
Net assets acquired	$ 8,289

The results of operations of Cardiogen have been included in the Company's consolidated statements of operations and comprehensive loss from the acquisition date. Pro forma results of operations have not been presented because the acquisition was not material to the Company's results of operations.

7. License and Collaboration Agreements

REGENXBIO License Agreements

REGENXBIO XLMTM and Pompe License

In July 2013, the Company entered into an exclusive license agreement with REGENXBIO Inc. (formerly ReGenX Biosciences, LLC), or REGENXBIO. Under the agreement, REGENXBIO granted the Company an exclusive worldwide license under certain patent rights to make, have made, use, import, sell and offer for sale licensed products in the treatment of both X-linked myotubular myopathy, or XLMTM, and Pompe disease using two adeno-associated virus serotypes.

As consideration for the licensed rights, the Company paid REGENXBIO an initial fee of $0.3 million in cash and issued 50,228 shares of common stock with a fair value at the date of issuance of $39,200. The Company will also owe REGENXBIO (i) an annual maintenance fee; (ii) up to $17.7 million in combined milestone fees, a small portion of which may be paid in the form of shares of the Company's common stock;

(iii) mid to high single digit royalty percentages on net sales of licensed products and (iv) mid-single digit to low twenties royalty percentages of any sublicense fees it receives from sublicensees for the licensed patent rights.

The agreement will expire upon the expiration, lapse, abandonment or invalidation of the last claim of the licensed patent rights to expire, lapse or become abandoned or unenforceable in all countries worldwide. The Company may terminate the agreement upon prior written notice. REGENXBIO may terminate the agreement immediately if the Company or its affiliates become insolvent, if the Company is late by a specified number of days in paying money due under the agreement or if the Company or its affiliates commence any action against REGENXBIO or its licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate the agreement for material breach that is not cured within a specified number of days.

The initial fee, consisting of the $39,200 in common stock and $0.3 million paid in cash was recorded as research and development expense on the date of the agreement. The annual maintenance fee will be recorded as expense each year. Milestone payments will be recorded as research and development expenses once achievement of each associated milestone has occurred or the achievement is considered probable. As of December 31, 2015, none of the development milestones had been reached or were probable of achievement.

REGENXBIO CPVT License

On November 3, 2015, the Company entered into a license agreement with REGENXBIO Inc., or REGENXBIO, pursuant to which REGENXBIO granted the Company an exclusive worldwide license under certain patent rights to make, have made, use, import, sell and offer for sale licensed products for the treatment of CPVT in humans using AAV9. Within a specified time and upon written notice the Company may elect to substitute for, or add to, CPVT certain other inherited arrhythmias.

The agreement will continue on a country-by-country and licensed product-by-licensed product basis and expire upon the later of the expiration, lapse, abandonment or invalidation of the last claim of the licensed patent rights to expire, lapse or become abandoned or unenforceable in such country, or ten years after first commercial sale of such licensed product in such country. The Company may terminate the agreement in its entirety or for each elected disease indication upon prior written notice. REGENXBIO may terminate the agreement immediately in case of the Company's bankruptcy or other similar events, if the Company is late in paying money due under the agreement and does not pay in full within a specified number of days after receiving written notice, or if the Company or the Company's affiliates commence any action against REGENXBIO or its licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate the agreement for material breach that is not cured within a specified number of days.

As consideration for the licensed rights, the Company paid REGENXBIO an upfront fee of $1.0 million.

For each additional indication the Company may elect to pursue under the licensed rights, it agreed to pay REGENXBIO a fee of $0.5 million upon such election. The Company will also owe REGENXBIO (i) an annual maintenance fee for each covered indication; (ii) up to $8.8 million in combined development and regulatory milestone fees for each indication and each licensed product; (iii) up to $45.0 million in combined commercial milestone fees based on various annual aggregate net sales thresholds, beginning when annual aggregate net sales of the licensed products equals or exceeds $100.0 million; (iv) mid-single digit to low teens royalty percentages on net sales of licensed products sold by the Company, its affiliates and sublicensees and (v) a low twenties percentage of any sublicense fees the Company receives from sublicensees for the licensed products and certain fees the Company receives from the sale or transfer of specified rights related to a licensed product.

REGENXBIO Crigler-Najjar Syndrome License

On November 3, 2015, the Company entered into a license agreement with REGENXBIO, pursuant to which REGENXBIO granted the Company an exclusive worldwide license to make, have made, use, import, sell, and offer for sale the licensed products for the treatment of Crigler-Najjar syndrome in humans using AAV8.

The agreement will continue on a country-by-country and licensed product-by-licensed product basis and expire upon the later of the expiration, lapse, abandonment or invalidation of the last claim of the licensed patent rights to expire, lapse or become abandoned or unenforceable in such country, or ten years after first commercial sale of such licensed product in such country. The Company may terminate the agreement upon prior written notice. REGENXBIO may terminate the agreement immediately in case of the Company's bankruptcy, or other similar events, if the Company is late in paying money due under the agreement and does not pay in full within a specified number of days after receiving written notice, or if the Company or its affiliates commence any action against REGENXBIO or its licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate the agreement for material breach that is not cured within a specified number of days.

As consideration for the licensed rights, the Company paid REGENXBIO an upfront fee of $0.2 million and agreed to pay an additional $0.4 million upon the occurrence of certain events. The Company will also owe REGENXBIO (i) an annual maintenance fee; (ii) up to $7.6 million in combined development and regulatory milestone fees per licensed product; (iii) mid-single digit to low teens royalty percentages on net sales of licensed products sold by the Company, its affiliates and sublicensees and (iv) a low twenties percentage of certain sublicense fees it receives from sublicensees for the licensed products and certain fees the Company receives from the sale or transfer of specified rights related to a licensed product.

Genethon Collaboration Agreement

In January 2014, the Company entered into a collaborative development agreement with Genethon, a French not-for-profit organization. In connection with the entry into the collaborative development agreement, the Company issued 262,396 shares of common stock to Genethon, of which 87,465 shares vested immediately, 87,465 shares vested in January 2015 and 87,466 shares will vest in January 2016. Unvested shares are subject to a repurchase option at the Company's election in the event of any termination of the agreement. Unvested shares will become fully vested in the event the Company undergoes a change in control or an initial public offering. Genethon also received certain registration rights and information rights similar to those held by the Company's preferred stockholders. The first one-third of vested shares issued to Genethon was recorded as research and development expense at the estimated fair value on the date of issuance. The remaining two-thirds of unvested shares are remeasured at each reporting period at estimated fair value and will be recorded as research and development expense over the remaining vesting term (see Note 10).

Subject to certain limitations on patents that are co-owned or in-licensed by the Company, Genethon granted the Company a royalty-free, exclusive, worldwide license under certain background intellectual property rights controlled by Genethon to research, develop, make and commercialize certain products for the treatment of XLMTM. In addition, the collaboration agreement provides that new intellectual property arising from the performance of the development plan will be owned jointly by both parties, and Genethon granted the Company a royalty-free, exclusive, worldwide license to Genethon's interest in such new intellectual property to research, develop, make and commercialize certain products for the treatment of XLMTM. The agreement provides Genethon with the exclusive right to manufacture licensed product for preclinical and clinical purposes, subject to Genethon's ability to supply required quantities in accordance with applicable timelines. Manufacturing costs will be paid by the Company. Additionally, the agreement specifies that Genethon will be paid by the Company

for research and development activities it performs pursuant to mutually agreed upon research and development plans as determined by a joint development committee. Costs incurred under the agreement are recorded to research and development expense as services are performed.

Either party may terminate the agreement for the other party's uncured material breach of the agreement or for the other party's bankruptcy. The Company may terminate the agreement for convenience upon prior written notice. Genethon may terminate the agreement upon raising an objection to continued development on grounds of a safety or efficacy issue and upon prior written notice of such objection.

The Company conducts business with Genethon, which results in payables in the Euro currency. The Company does not engage in hedging activities to offset the risk of exchange rate fluctuations on these payables. During the years ended December 31, 2014 and 2015, the Company benefited from foreign exchange gains on these accounts payable of approximately $0.1 million, reported as other income in its consolidated statements of operations and comprehensive loss.

University of Florida License Agreement

Effective July 2015, the Company entered into a license agreement with the University of Florida Research Foundation, or UFRF. Under the agreement, UFRF granted the Company an exclusive, worldwide license under certain patent rights and a non-exclusive license to certain related know-how for the treatment of Pompe. The Company agreed to pay an upfront license fee, an annual maintenance fee until first commercial sale of a licensed product, up to $1.2 million in combined development and regulatory milestone payments and a low single digit royalty on net sales of licensed products sold by the Company and its sublicensees, subject to minimum annual royalty payments following the first commercial sale of a licensed product. The Company is obligated to pay royalties on a country-by-country basis until the later of expiration of the last valid claim within the licensed patent rights in such country and ten years after first commercial sale of a licensed product in such country. The Company also agreed to pay to UFRF certain percentages of sublicense fees it receives from sublicensees of the licensed patent rights based on the stage of development at the time the sublicense is executed.

Under the agreement, the Company is obligated to perform a specified development plan and to use commercially reasonable efforts to market and commercialize at least one licensed product which has obtained regulatory approval. The Company is also obligated to achieve a number of diligence milestones, including the achievement of first commercial sale within a specific time period. If the Company fails to meet any of these diligence milestones and the deadlines are not extended in accordance with the terms of the agreement, then UFRF may terminate the agreement.

The Company may terminate the agreement for convenience upon prior written notice. UFRF may terminate the agreement upon prior written notice for breach of the agreement by the Company, including specific listed breaches. In addition, UFRF may terminate the agreement immediately if the Company or its affiliates challenge the validity, patentability or enforceability of the licensed patents rights. If the challenge is brought by a sublicensee, UFRF may request that the Company terminate the sublicense.

Fondazione Salvatore Maugeri License Agreement

In connection with the Company's acquisition of Cardiogen, it acquired a license agreement previously entered into by Cardiogen with FSM, an Italian non-profit research organization. Under the license agreement, the Company obtained an exclusive worldwide license to certain intellectual property to develop, use and commercialize products related to recessive CPVT, as well as to several additional inherited arrhythmias.

The Company may terminate the license agreement with FSM for convenience upon prior written notice. Either party may terminate the agreement upon prior written notice for the uncured material breach of the agreement by the other party, or the other party's bankruptcy or liquidation.

Other License Agreements

The Company holds interests in other agreements providing the Company with licenses or options to acquire licenses to certain intellectual property rights. The Company has paid upfront fees of $0.3 million related to those agreements. Additionally, the Company is obligated to pay $0.3 million upon the occurrence of certain milestones or upon termination of the respective agreements. One agreement provides for a payment of $0.1 million within 18 months from the effective date, unless the agreement is cancelled prior to that date. The Company may terminate the agreements at any time upon prior written notice.

8. Convertible Preferred Stock

Convertible preferred stock consisted of the following:

	December 31, 2014				
	Shares Authorized	Original Issue Price per Share	Shares Issued and Outstanding	Net Carrying Value	Aggregate Liquidation Preference
	(in thousands, except share and per share amounts)				
Series Seed	1,400,000	$ 2.2300	627,867	$ 1,378	$ 1,400
Series A	11,199,876	5.9727	5,022,876	28,757	30,000
Series B	8,500,000	11.1942	3,796,635	42,268	42,500
Total convertible preferred stock	21,099,876		9,447,378	$ 72,403	$ 73,900

	December 31, 2015				
	Shares Authorized	Original Issue Price per Share	Shares Issued and Outstanding	Net Carrying Value	Aggregate Liquidation Preference
	(in thousands, except share and per share amounts)				
Series Seed	1,400,000	$ 2.2300	627,867	$ 1,378	$ 1,400
Series A	11,199,876	5.9727	5,022,876	28,757	30,000
Series B	8,570,366	11.1942	3,843,604	42,835	43,026
Series C	9,684,789	15.0256	4,325,954	62,780	65,000
Total convertible preferred stock	30,855,031		13,820,301	$ 135,750	$ 139,426

In November 2014, the Series A preferred stockholders elected to purchase shares pursuant to a third and final tranche option and the Company issued 2,511,441 shares for gross proceeds of $15.0 million. As of December 31, 2014, all tranche options had been fully exercised.

In November 2014, the Company entered into a preferred stock purchase agreement with existing and new investors and issued 3,796,635 shares of Series B convertible preferred stock at a price per shares of $11.1942 for net proceeds of approximately $42.3 million.

In October 2015, the Company entered into a preferred stock purchase agreement with existing and new investors and issued 4,325,954 shares of Series C convertible preferred stock at a price per share of $15.0256. Proceeds to the Company net of the placement agent fee and expenses were approximately $62.8 million.

The rights, privileges, and preferences of convertible preferred stock are summarized as follows:

Liquidation Preference

Upon liquidation, dissolution, or winding up of the Company (whether voluntary or involuntary), or Deemed Liquidation Event (as defined below), before any distribution or payment shall be made to the holders of common stock, each series of convertible preferred stock shall be entitled to be paid on a pari passu basis out of the funds and assets available for distribution, an amount equal to the Original Issue Price of $2.2300 for holders of Series Seed convertible preferred stock, $5.9727 for holders of Series A convertible preferred stock, $11.1942 for holders of Series B convertible preferred stock and $15.0256 for holders of Series C convertible preferred stock, plus any dividends declared but unpaid thereon. If upon any liquidation, dissolution, winding up or Deemed Liquidation Event of the Company, the assets of the Company available for distribution to shareholders is insufficient to pay the holders of shares of preferred stock in full, the holders of preferred stock will share ratably in any distribution.

After payment of all preferential amounts required to be paid to the holders of preferred stock, the remaining funds and assets available for distribution to the shareholders of the Company will be distributed among the holders of preferred stock and common stock, pro rata based on the number of shares held by each such holder.

The following events are defined as Deemed Liquidation Events unless the holders of at least 66.67% of the then outstanding shares of convertible preferred stock elect otherwise by written notice to the Company:

(i) a merger or consolidation; or

(ii) the sale, lease, transfer, exclusive license or other disposition, of all or substantially all the assets of the Company.

Voting

Each holder of shares of convertible preferred stock is entitled to the number of votes equal to the number of shares of common stock into which such shares of convertible preferred stock could be converted and has voting rights and powers equal to the voting rights and powers of the common stock, and except as provided by law or by other provisions of the Certificate of Incorporation, shall vote together with the common stock as a single class on an as-converted basis on all matters as to which holders of common stock have the right to vote.

The holders of convertible preferred stock, voting together as a single class, are entitled to elect four members of the Company's Board of Directors. The holders of common stock, exclusively and as a separate class, are entitled to elect one member of the Company's Board of Directors. The one remaining member of the Company's Board of Directors is elected by the holders of the common stock and any other series or class of voting stock, including the convertible preferred stock, exclusively and voting together as a single class.

Conversion

The holders of convertible preferred stock are subject to certain optional and mandatory conversion rights.

(i) *Optional Conversion Rights:* Each share of convertible preferred stock is convertible, at the option of the holder, into such number of fully paid shares of common stock as is determined by dividing

the original issuance price by the conversion price in effect at the time of conversion. As of December 31, 2015, the conversion ratio was 1:1 for all series of preferred stock.

(ii) *Mandatory Conversion Rights:* Upon either (a) the date and time, or the occurrence of a future event as determined by vote or written consent of holders of at least a 66.67% of the then outstanding shares of convertible preferred stock, or (b) the closing of the sale of shares of the Company's common stock to the public in a qualified initial public offering, all outstanding shares of convertible preferred stock will automatically be converted into shares of common stock, at the then effective conversion rate. A qualified initial public offering is defined as the closing of a firm commitment underwritten public offering with an offering price per share of not less than $24.0410, and at least $50.0 million in gross proceeds.

The conversion price for convertible preferred shares is subject to adjustment upon certain events including certain dilutive issuances of shares, share subdivisions such as stock splits and stock dividends. At December 31, 2015, Series Seed preferred shares had a conversion price of $2.2300 per share, Series A preferred shares had a conversion price of $5.9727 per share, Series B preferred shares had a conversion price of $11.1942 per share and Series C preferred shares had a conversion price of $15.0256 per share.

Dividends

The holders of the outstanding shares of convertible preferred stock are entitled to receive, when and if declared by the Board of Directors, a noncumulative cash dividend at the rate of 8% of the applicable original issue price per annum on each outstanding share of convertible preferred stock. Such dividends are payable in preference to any dividends for common stock declared by the Board of Directors. In the case of a dividend on common stock, the dividend per share of convertible preferred stock would also include the dividend payable on each share determined, if applicable, as if all convertible preferred stock had been converted to common stock. No dividends had been declared as of December 31, 2015.

9. Common Stock

Common Stock

Common stockholders are entitled to dividends when, as and if declared by the Board of Directors, subject to the liquidation preferences of the preferred stockholders. As of December 31, 2015, no dividends had been declared by the Board of Directors.

Common stock reserved for issuance was as follows:

	December 31,	
	2014	2015
Convertible preferred stock, on an as-converted basis	9,447,378	13,820,301
Options issued and outstanding	784,790	2,303,862
Options available for future grants	977,297	751,661
Total	11,209,465	16,875,824

Restricted Stock

Certain founding directors purchased 224,238 common shares that were subject to a repurchase right upon termination or cessation of services at the original purchase price of $0.02 per share. Compensation expense of such shares is remeasured at fair value over the vesting period at each reporting date. This repurchase right lapses as vesting occurs. At December 31, 2015, no amounts were recorded in liabilities related to restricted share sales as the repurchase right had lapsed.

A summary of restricted stock activity and related information follows:

	Number of Restricted Shares Outstanding
Unvested shares—December 31, 2014	42,978
Vested	(42,978)
Unvested shares—December 31, 2015	—

There were no restricted share awards granted during 2015.

10. Stock Compensation

In 2012, the Company adopted the 2012 Equity Incentive Plan, or Plan. Under the Plan, shares of the Company's common stock have been reserved for the issuance of stock options to employees, directors, and consultants under terms and provisions established by the Board of Directors. A total of 3,107,517 shares were reserved for issuance under the 2012 Plan at December 31, 2015, of which 751,661 shares are available for future grant. Under the terms of the Plan, options may be granted at an exercise price not less than fair market value. For employees holding more than 10% of the voting rights of all classes of stock, the exercise prices for incentive and non-statutory stock options may not be less than 110% of fair market value, as determined by the Board of Directors. The terms of options granted under the Plan may not exceed ten years. The vesting schedule of newly issued option grants is typically 25% one year from the vesting commencement date and 1/48th per month thereafter. The following summarizes option activity under the 2012 Plan:

	Shares Available for Grant	Number of Options Outstanding	Weighted-Average Exercise Price per Option	Weighted-Average Remaining Contract Term	Aggregate Intrinsic Value
Balance, December 31, 2014	977,297	784,790	$ 0.83	9.01	$ 1,074
Shares reserved for issuance	1,345,430	—	—		
Options granted	(1,687,484)	1,687,484	$ 4.64		
Options exercised	—	(51,994)	$ 1.34		
Options forfeited	104,787	(104,787)	$ 1.01		
Options canceled	11,631	(11,631)	$ 1.59		
Balance Outstanding, December 31, 2015	751,661	2,303,862	$ 3.60	9.18	$ 13,593
Exercisable, December 31, 2015		634,390	$ 1.31	8.36	$ 5,192
Vested and expected to vest, December 31, 2015		2,256,392	$ 3.52	9.16	$ 13,489

The aggregate intrinsic values of options outstanding, exercisable, vested and expected to vest were calculated as the difference between the exercise price of the options and the estimated fair value of the Company's common stock, as determined by the Board of Directors, as of December 31, 2015. During the year ended December 31, 2015, 51,994 options with an intrinsic value of approximately $59,000 were exercised.

Stock-Based Compensation Expense

Total stock-based compensation expense was as follows:

	Year Ended December 31,	
	2014	2015
	(in thousands)	
Research and development	$ 387	$ 932
General and administrative	128	359
Total stock-based compensation expense	$ 515	$ 1,291
Employees	$ 142	$ 413
Non-employees	373	878
Total stock-based compensation expense	$ 515	$ 1,291

The weighted average grant date fair value of employee options granted during the years ended December 31, 2014 and 2015 was $0.67 and $3.01 per share, respectively. As of December 31, 2015, the total unrecognized compensation expense related to unvested employee options, net of estimated forfeitures, was approximately $4.6 million which the Company expects to recognize over an estimated weighted-average period of 3.34 years. To the extent the actual forfeiture rate is different from what the Company has estimated, stock-based compensation related to these awards will be different from its expectations.

The fair value of stock options for employees was estimated using a Black-Scholes option pricing model with the following assumptions:

	Year Ended December 31,	
	2014	2015
Fair value of common stock	$0.78 – 1.05	$2.19 – $9.50
Expected term (in years)	6.1	5.8 – 6.1
Expected volatility	88% – 91%	66% – 85%
Risk-free interest rate	1.5% – 1.8%	1.3% – 1.9%
Expected dividend yield	—%	—%

There were no non-employee option grants during the year ended December 31, 2014. The weighted-average grant date fair value of non-employee options granted during the year ended December 31, 2015 was $1.61. As of December 31, 2015, the total unrecognized compensation expense related to unvested non-employee options, net of estimated forfeitures, was approximately $57,000, which includes $27,000 of expense for unvested common stock issued to Genethon (see Note 7). Research and development includes $0.3 million and $0.7 million of stock-based compensation expense related to remeasurement of Genethon shares during the years ended December 31, 2014 and 2015, respectively.

The fair value of stock options for non-employees was estimated using a Black-Scholes option pricing model with the following assumptions:

	Year Ended December 31,	
	2014	2015
Fair value of common stock	$0.78 – 2.19	$2.19 – 9.50
Expected term (in years)	8.7 – 9.5	7.7 – 10
Expected volatility	70% – 71%	69% – 72%
Risk-free interest rate	1.9% – 2.4%	1.6% – 2.3%
Expected dividend yield	—%	—%

In determining the fair value of the stock-based awards, the Company uses the Black-Scholes option pricing model and assumptions discussed below. Each of these inputs is subjective and generally requires significant judgment to determine.

Fair Value of Common Stock: Given the absence of a public trading market, the Board of Directors considered numerous objective and subjective factors to determine the fair value of common stock at each grant date. These factors included, but were not limited to, (i) contemporaneous valuations of common stock performed by unrelated third-party specialists; (ii) the prices for preferred stock sold to outside investors; (iii) the rights, preferences and privileges of preferred stock relative to common stock; (iv) the lack of marketability of common stock; (v) developments in the business; and (vi) the likelihood of achieving a liquidity event, such as an initial public offering or a merger or acquisition of the Company, given prevailing market conditions.

Expected Term: The Company's expected term represents the period that the Company's stock-based awards are expected to be outstanding and is determined using the simplified method (based on the mid-point between the vesting date and the end of the contractual term for employee options and based on the contractual term for non-employee options).

Expected Volatility: Since the Company is privately held and does not have any trading history for its common stock, the expected volatility was estimated based on the average volatility for comparable publicly traded biopharmaceutical companies over a period equal to the expected term of the stock option grants. The comparable companies were chosen based on their similar size, stage in the life cycle, or area of specialty.

Risk-Free Interest Rate: The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of option.

Expected Dividend Yield: The Company has never paid dividends on its common stock and has no plans to pay dividends on its common stock. Therefore, the Company used an expected dividend yield of zero.

11. Income Taxes

For financial reporting purposes, "loss before provision for income taxes," includes the following components:

	Year Ended December 31,	
	2014	2015
	(in thousands)	
Domestic	$(10,819)	$(26,458)
Total	$(10,819)	$(26,458)

Provision (Benefit) for Income Taxes

The provision (benefit) for income taxes for 2014 and 2015 was an immaterial amount.

Income tax provision (benefit) related to continuing operations differ from the amounts computed by applying the statutory income tax rate of 35% to pretax loss as follows:

	Year Ended December 31,	
	2014	2015
	(in thousands)	
U.S. federal provision (benefit):		
At statutory rate	$ (3,788)	$ (9,260)
State taxes	1	1
Change in valuation allowance	3,998	10,263
Tax credits	(345)	(1,347)
Stock-based compensation	60	160
Other	74	183
Total	$ —	$ —

Deferred Tax Assets and Liabilities

Deferred income taxes reflect the net tax effects of loss and credit carryforwards and temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Significant components of deferred tax assets for federal and state income taxes are as follows:

	Year Ended December 31,	
	2014	2015
	(in thousands)	
Deferred tax assets:		
Federal & state net operating loss carryforward	$ 5,125	$ 14,286
Research and other credits	487	2,858
Intangibles	296	1,211
Reserves and accruals	414	690
Stock-based compensation	6	22
Start-up costs	164	174
Other	44	141
Total gross deferred tax assets	6,536	19,382
Less valuation allowance	(6,531)	(19,350)
Total deferred tax assets	5	32
Deferred tax liabilities:		
Other intangibles	—	(3,260)
Property plant and equipment	(5)	(32)
Total gross deferred tax liability	(5)	(3,292)
Net deferred tax liability	$ —	$ (3,260)

Realization of deferred tax assets is dependent upon future earnings, if any, the timing and amount of which are uncertain. Due to the lack of earnings history, the net deferred tax assets have been fully offset by a valuation allowance.

Net Operating Loss and Tax Credit Carryforwards

As of December 31, 2014 and 2015, the Company had net operating loss carryforwards for federal income tax purposes of $11.7 million and $32.1 million, respectively, which will begin to expire in 2033. The Company had total state net operating loss carryforwards of approximately $11.8 million and $34.4 million, respectively, which will begin to expire in 2033. Utilization of some of the federal and state net operating loss and credit carryforwards are subject to annual limitations due to the "change in ownership" provisions of the Internal Revenue Code of 1986 and similar state provisions. The annual limitations may result in the expiration of net operating losses and credits before utilization. The Company has not performed an ownership change analysis.

The Company has federal credits of $3.1 million which will begin to expire in 2033 and state research credits of approximately $0.5 million which have no expiration date. These tax credits are subject to the same limitations discussed above.

Unrecognized Tax Benefits

The Company has incurred net operating losses since inception and has no significant unrecognized tax benefits. The Company's policy is to include interest and penalties related to unrecognized tax benefits, if any, within the provision for taxes in the statements of operations. If in the future the Company recognizes uncertain tax positions, the Company's effective tax rate will be reduced. Currently, the Company has a full valuation allowance against its net deferred tax asset which would impact the timing of the effective tax rate benefit should any of these uncertain tax positions be favorably settled in the future. Any adjustments to uncertain tax positions would result in an adjustment of net operating loss or tax credit carry forwards rather than resulting in a cash outlay.

Income tax returns are filed in the U.S. and California. The Company is not currently under examination. Due to net operating losses and research credit carryovers, all of the tax years remain open to examination.

Unrecognized tax benefits were as follows:

	Year Ended December 31,	
	2014	2015
	(in thousands)	
Beginning balance	$ 15	$ 121
Tax positions related to current year:		
Federal and state	106	593
Ending balance	$ 121	$ 714

Although it is reasonably possible that certain unrecognized tax benefits may increase or decrease within the next twelve months due to tax examination changes, settlement activities, expirations of statute of limitations, or the impact on recognition and measurement considerations related to the results of published tax cases or other similar activities, the Company does not anticipate any significant changes to unrecognized tax

benefits over the next 12 months. During the year ended December 31, 2015, no interest or penalties were recognized relating to unrecognized tax benefit.

12. Commitments and Contingencies

Facility Operating Leases

The Company leases its office location in San Francisco, California, under a non-cancelable operating lease for 4,996 square feet of office space. The original lease would have expired in January 2017 and included an initial rent-free period. The lease also provided for annual rent escalation throughout the term of the lease. In accordance with the lease agreement, the Company provided a security deposit of $67,500 to the landlord. In August 2014, the lease was amended to expand the available premises to 10,170 square feet under substantially similar terms as the original agreement and became effective beginning January 2015. In November 2015, the Company entered into an early lease termination agreement in exchange for $0.2 million, which accelerated the expiration date to April 2016. The lease termination fee was recorded as facilities expense as of the termination agreement date.

In December 2014, the Company entered into a facility lease agreement with Janssen Research & Development LLC (Janssen) whereby the Company gained access and use rights to office and laboratory space located in South San Francisco, California, effective January 2015. The agreement provides for successive renewable three-month lease terms that are cancelable by the Company upon 60 days written notice and annual rent escalation. In accordance with the Janssen agreement, the Company paid an initial security deposit of $35,800. During 2015, the Company entered into multiple lease amendments, each increasing the space available to the Company for research purposes and increasing the security deposit to $76,000 as of December 31, 2015. The Company recognizes rent expense on a straight-line basis over each non-cancelable lease term.

Solstice Sub-Lease

On July 30, 2015, the Company entered into a sub-lease agreement with Solstice Neurosciences LLC, or Solstice, to sub-lease 21,960 square feet of manufacturing space in South San Francisco, California with total minimum lease payments due of $0.9 million. The lease expires in May 2017. In November 2015, the Company entered into an option to extend the lease. The terms of the lease provide for a single rent escalation following the first twelve months of the lease. An initial deposit of $0.1 million and a standby letter of credit totaling $0.7 million was provided by the Company to Solstice, which is included within current restricted cash on the Company's consolidated balance sheet at December 31, 2015.

Under the Solstice lease agreement, the Company agreed to return the property to its original condition upon lease termination. The asset retirement obligation was estimated by the Company using expected future cash flows that reflect, to the extent possible, an assessment of the cost and timing of performing the required activities, which was then discounted using a credit adjusted risk free rate. The Company records rent expense to increase the asset retirement obligation to its full value of $0.6 million over the term of the lease agreement. The Company recognized $0.1 million of additional rent expense in 2015.

JCN Facility Lease Option

Effective November 10, 2015, the Company entered into a facility lease option agreement with JCN Partners, or JCN, that provides the Company an option to lease manufacturing space under the terms of a long-term lease for 22,000 square feet that is currently leased by the Company pursuant to the Solstice sub-lease agreement dated July 30, 2015, plus approximately 17,000 square feet of additional space. The Company has

until August 1, 2016 to exercise the option providing an initial lease term of 10 years with an option to further extend the lease for two additional five-year terms. The Company paid $10,000 to JCN in exchange for the lease option, which was recorded as research and development expense in 2015.

In connection with the lease option agreement, the Company also entered into a separate agreement for restoration of premises between JCN, Solstice and a previous sub-tenant, whereby the Company agreed to accept liability to restore the leased premises to its original warehouse condition upon expiration of the final lease term. The Company estimates that the cost to restore the premises without additional improvements would be approximately $0.6 million. Upon entry into the restoration agreement, the Company issued a stand-by letter of credit to JCN for $1.0 million, which is recorded in long-term restricted cash on the consolidated balance sheets.

MEPT Lease Agreement

On September 21, 2015, the Company entered into a lease agreement with MEPT 600 California Street LLC, or MEPT, to occupy 21,596 square feet of office space in San Francisco, California beginning February 2016. The Company intends to relocate its corporate headquarters to this location. The lease agreement provides for an initial three month rent-free period and provides for annual rent escalation with a lease term through June 2022. The Company provided a standby letter of credit in the amount of $1.9 million, which is recorded as long-term restricted cash in the accompanying consolidated balance sheets, following execution of the agreement.

The agreement also provides for up to $1.6 million of leasehold improvements to be paid by MEPT. The Company reports its leasehold improvement expenditures as a miscellaneous receivable on a pro-rata basis with the offsetting amount recorded as deferred rent.

Rent expense under non-cancelable operating leases was approximately $0.3 million and $1.5 million for the years ended December 31, 2014 and 2015, respectively, including approximately $0.2 million for lease termination payments and lease options. Future minimum lease payments under non-cancelable operating leases as of December 31, 2015 were as follows:

	Year ended December 31, Amount
	(in thousands)
2016	$ 1,603
2017	1,794
2018	1,643
2019	1,691
2020	1,740
Thereafter	2,591
	$ 11,062

Guarantees and Indemnifications

The Company indemnifies each of its directors and officers for certain events or occurrences, subject to certain limits, while the director is or was serving at the Company's request in such capacity, as permitted under Delaware law, and in accordance with its certificate of incorporation and bylaws. The term of the indemnification period lasts as long as a director may be subject to any proceeding arising out of acts or omissions of such director in such capacity. The maximum amount of future indemnification is unlimited; however, the Company currently holds director liability insurance. This insurance allows the transfer of risk associated with the

Company's exposure and may enable it to recover a portion of any future amounts paid. The Company believes that the fair value of these indemnification obligations is minimal. Accordingly, it has not recognized any liabilities relating to these obligations for any period presented.

13. Net Loss per Share and Pro Forma Net Loss per Share

Basic net loss per share is calculated by dividing net loss by the weighted-average number of common shares outstanding during the period and excludes any dilutive effects of share-based awards. Diluted net loss per share is computed giving effect to all potential dilutive common shares, including common stock issuable upon exercise of stock options, convertible preferred stock, and unvested restricted common stock. As the Company had net losses for the years ended December 31, 2014 and 2015, all potential common shares were determined to be anti-dilutive.

The following table sets forth the computation of basic and diluted net loss per share of common stock during the years ended December 31, 2014 and 2015:

	Year Ended December 31,	
	2014	2015
	(in thousands, except share and per share data)	
Net loss	$ (10,819)	$ (26,458)
Weighted-average shares used in computing net loss per share	501,707	1,148,827
Net loss per share, basic and diluted	$ (21.56)	$ (23.03)

The following outstanding shares of common stock equivalents were excluded from the computation of diluted net loss per share of common stock for the periods presented because including them would have been anti-dilutive:

	Year Ended December 31,	
	2014	2015
Convertible preferred stock (on an as-if-converted basis)	9,447,378	13,820,301
Stock options to purchase common stock	784,790	2,303,862
Restricted stock subject to future vesting	217,909	95,595
Total	10,450,077	16,219,758

The Company has presented unaudited pro forma basic and diluted net loss per common share, which has been computed to give effect to the conversion of all outstanding shares of convertible preferred stock into shares of common stock as if such conversion had occurred as of the beginning of the period presented or as of the date of issuance for convertible preferred stock issued during 2015. The following table sets forth the computation of the Company's pro forma basic and diluted net loss per common share for the year ended December 31, 2015 (in thousands, except share and per share amounts):

Pro Forma Net Loss Per Share

Net loss per share—basic and diluted	$ (26,458)
Shares used in computing pro forma net loss per share:	
Weighted-average shares used in computing net loss per share—basic and diluted	1,148,827
Pro forma adjustment to reflect assumed conversion of convertible preferred stock	10,472,422
Weighted-average shares used in computing pro forma net loss per share—basic and diluted	11,621,249
Pro forma net loss per share, basic and diluted	$ (2.28)

14. Related Party Transactions

Aggregate payments in connection with related party transactions totaled approximately $47,000 and $32,000 during the years ended December 31, 2014 and 2015, respectively, and consisted primarily of cost reimbursements to certain investors.

15. Subsequent Events

In March 2016, the Company granted options to purchase 200,239 shares of its common stock at an exercise price of $7.54 per share.

In July 2016, the Company's board of directors approved an amendment to the Company's amended and restated certificate of incorporation to effect a reverse split of the Company's issued and outstanding common stock and convertible preferred stock at a 2.22977-to-1 ratio, which was effected on July 7, 2016. The par value and authorized shares of common stock and convertible preferred stock were not adjusted as a result of the reverse split. All issued and outstanding common stock and convertible preferred stock, options to purchase common stock and per share amounts contained in the financial statements have been retroactively adjusted to reflect the reverse stock split for all periods presented.

AUDENTES THERAPEUTICS, INC.
Condensed Consolidated Balance Sheets
(in thousands, except shares and per share data)

	December 31, 2015	March 31, 2016 (unaudited)	Pro Forma Stockholder Equity as of March 31, 2016 (unaudited)
Assets			
Current assets:			
Cash and cash equivalents	$ 72,058	$ 59,713	
Short-term investments	23,169	20,559	
Restricted cash	730	730	
Prepaid expenses and other current assets	3,682	5,001	
Total current assets	99,639	86,003	
Restricted cash-long-term	2,930	2,930	
Property and equipment, net	2,968	9,690	
Goodwill	3,631	3,631	
Intangible assets	8,000	8,000	
Other assets	301	301	
Total assets	$117,469	$110,555	
Liabilities and Stockholders' Equity			
Current liabilities:			
Accounts payable	$ 2,789	$ 548	
Accrued liabilities	4,797	8,950	
Facility lease obligations	137	355	
Total current liabilities	7,723	9,853	
Facility lease obligations	519	1,432	
Contingent acquisition consideration payable	4,278	4,368	
Deferred tax liability, net	3,260	3,260	
Total liabilities	15,780	18,913	
Stockholders' equity:			
Convertible preferred stock, Series Seed, $0.00001 par value; 1,400,000 shares authorized as of December 31, 2015 and March 31, 2016; 627,867 shares issued and outstanding as of December 31, 2015 and March 31, 2016, aggregate liquidation preference of $1,400 as of December 31, 2015 and March 31, 2016	1,378	1,378	$ —
Convertible preferred stock, Series A, $0.00001 par value; 11,199,876 shares authorized as of December 31, 2015 and March 31, 2016; 5,022,876 shares issued and outstanding as of December 31, 2015 and March 31, 2016, aggregate liquidation preference of $30,000 as of December 31, 2015 and March 31, 2016	28,757	28,757	—
Convertible preferred stock, Series B, $0.00001 par value; 8,570,366 shares authorized as of December 31, 2015 and March 31, 2016; 3,843,604 shares issued and outstanding as of December 31, 2015 and March 31, 2016, aggregate liquidation preference of $43,026 as of December 31, 2015 and March 31, 2016	42,835	42,835	—
Convertible preferred stock, Series C, $0.00001 par value; 9,684,789 shares authorized as of December 31, 2015 and March 31, 2016; 4,325,954 shares issued and outstanding as of December 31, 2015 and March 31, 2016, aggregate liquidation preference of $65,000 as of December 31, 2015 and March 31, 2016	62,780	62,780	—
Common stock, $0.00001 par value, 50,000,000 shares authorized as of December 31, 2015 and March 31, 2016; 2,106,152 and 2,200,077 shares issued and outstanding as of December 31, 2015 and March 31, 2016, respectively; 16,020,378 shares issued and outstanding, pro forma (unaudited)	—	—	—
Additional paid-in capital	6,692	7,095	142,845
Accumulated deficit	(40,743)	(51,207)	(51,207)
Accumulated other comprehensive income (loss)	(10)	4	4
Total stockholders' equity	101,689	91,642	$ 91,642
Total liabilities and stockholders' equity	$117,469	$110,555	

See accompanying notes to unaudited interim condensed consolidated financial statements.

AUDENTES THERAPEUTICS, INC.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(unaudited)
(in thousands, except shares and per share data)

	Three Months Ended March 31,	
	2015	2016
Operating expenses:		
Research and development	$ 3,080	$ 7,906
General and administrative	1,083	2,632
Total operating expenses	4,163	10,538
Loss from operations	(4,163)	(10,538)
Interest income	61	97
Other income (expense), net	47	(23)
Net loss	(4,055)	(10,464)
Unrealized gains on available-for-sale securities	3	14
Net loss and comprehensive loss	$ (4,052)	$ (10,450)
Net loss per share, basic and diluted	$ (6.63)	$ (4.85)
Shares used in computing net loss per share, basic and diluted	612,039	2,159,081
Pro forma net loss per share, basic and diluted		$ (0.65)
Shares used in computing pro forma net loss per share, basic and diluted		15,979,382

See accompanying notes to unaudited interim condensed consolidated financial statements.

AUDENTES THERAPEUTICS, INC.
Condensed Consolidated Statements of Cash Flows
(unaudited)
(in thousands)

	Three Months Ended March 31,	
	2015	2016
Cash flows from operating activities:		
Net loss	$ (4,055)	$(10,464)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	23	78
Stock-based compensation	116	327
Accretion of asset retirement obligation	69	82
Amortization of discount on investments	—	133
Non-cash change in fair value of contingent acquisition consideration payable	—	90
Other	—	31
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(287)	(1,402)
Other assets	(70)	—
Accounts payable	(69)	(2,323)
Accrued liabilities	(573)	(450)
Facility lease obligations	(3)	1,049
Net cash used in operating activities	(4,849)	(12,849)
Cash flows from investing activities:		
Purchases of property and equipment	(24)	(2,145)
Proceeds from sales and maturities of marketable securities	2,000	17,229
Purchases of marketable securities	(26,134)	(14,656)
Net cash (used in) provided by investing activities	(24,158)	428
Cash flows from financing activities:		
Proceeds from exercise of stock options	—	76
Net cash provided by financing activities	—	76
Net decrease in cash and cash equivalents	(29,007)	(12,345)
Cash and cash equivalents at beginning of period	45,599	72,058
Cash and cash equivalents at end of period	$ 16,592	$ 59,713
Noncash investing and financing activities:		
Increase (decrease) in accounts payable, facility lease obligations and accrued liabilities related to property and equipment purchases	$ (88)	$ 3,415

See accompanying notes to unaudited interim condensed consolidated financial statements.

AUDENTES THERAPEUTICS, INC.
Notes to Unaudited Interim Condensed Consolidated Financial Statements

1. Organization and Basis of Presentation

Audentes Therapeutics, Inc., or the Company, was incorporated in the State of Delaware on November 13, 2012. The Company is a biotechnology company focused on developing and commercializing gene therapy products for patients suffering from serious, life-threatening rare diseases caused by single gene defects. The Company's principal operations are located in San Francisco, California, and it operates in one business segment.

The accompanying condensed consolidated financial statements include the accounts of Audentes Therapeutics, Inc., and its wholly owned subsidiary, Audentes Therapeutics UK Ltd. All intercompany balances and transactions have been eliminated in consolidation.

Liquidity

In the course of its development activities, the Company has sustained operating losses and expects such losses to continue over the next several years. The Company's ultimate success depends on the outcome of its research and development activities. The Company has incurred net losses from operations since inception and as of March 31, 2016, had an accumulated deficit of $51.2 million. The Company intends to raise additional capital through the issuance of additional equity, and potentially through strategic alliances with partner companies. If financing is not available at adequate levels, the Company may need to reevaluate its operating plans. Management believes its currently available resources will provide sufficient funds to enable the Company to meet its operating plans for at least the next twelve months. However, if the Company's anticipated operating results are not achieved in future periods, planned expenditures may need to be reduced in order to extend the time period over which the then-available resources would be able to fund the Company's operations.

2. Summary of Significant Accounting Policies

Unaudited Interim Condensed Consolidated Financial Statements

The interim condensed consolidated financial statements as of March 31, 2016 and for the three months ended March 31, 2015 and 2016 are unaudited. The unaudited interim financial statements have been prepared on the same basis as the annual financial statements and reflect, in the opinion of management, all adjustments of a normal and recurring nature that are necessary for the fair presentation of the Company's consolidated financial position as of March 31, 2016 and its consolidated results of operations and cash flows for the three months ended March 31, 2015 and 2016. The financial data and the other information disclosed in these notes to consolidated financial statements related to the two periods are unaudited. The results of operations for the three months ended March 31, 2016 are not necessarily indicative of the results to be expected for the year ending December 31, 2016 or for any other future annual or interim period. The balance sheet as of December 31, 2015 included herein was derived from the audited financial statements as of that date. These financial statements should be read in conjunction with the Company's audited financial statements.

Unaudited Pro Forma Stockholder's Equity

The unaudited pro forma stockholders' equity as of March 31, 2016 presents the Company's stockholders' equity as though all the Company's outstanding convertible preferred stock had converted into shares of common stock upon the completion of an initial public offering, or IPO, of the Company's common stock. The Company's certificate of incorporation provides for automatic conversion of outstanding preferred shares in the event of an IPO provided that certain minimum offering conditions are met.

Use of Estimates

The accompanying consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles, or GAAP. The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities, as of the date of the financial statements, and the reported amounts of any expenses during the reporting period. On an ongoing basis, management evaluates its estimates, including those related to accrued liabilities, fair value of assets, common stock, income taxes, and stock-based compensation. Management bases its estimates on historical experience, and on various other market-specific relevant assumptions that management believes to be reasonable, under the circumstances. Actual results may differ from those estimates.

Fair Value Measurements

Fair value is defined as the price at which an asset could be exchanged in a current transaction between knowledgeable, willing parties. A liability's fair value is defined as the amount that would be paid to transfer the liability to a new obligor, not the amount that would be paid to settle the liability with the creditor. Where available, fair value is based on observable market prices, or parameters derived from such prices. Where observable prices or inputs are not available, valuation models are applied. These valuation techniques involve some level of management estimation and judgment. The degree of management estimation and judgment is dependent on the price transparency for the instruments, or market, and the instruments' complexity. The authoritative accounting guidance describes a fair value hierarchy based on three levels of inputs that may be used to measure fair value, of which the first two are considered observable and the last is considered unobservable. These levels of inputs are as follows:

Level 1—Observable inputs such as unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date.

Level 2—Inputs (other than quoted prices included in Level 1) are either directly or indirectly observable for the asset or liability. These include quoted prices for similar assets or liabilities in active markets and quoted prices for identical or similar assets or liabilities in markets that are not active.

Level 3—Unobservable inputs that reflect management's best estimate of what market participants would use in pricing the asset or liability at the measurement date. Consideration is given to the risk inherent in the valuation technique and the risk inherent in the inputs to the model.

Money market funds are valued using quoted market price, and are included in cash equivalents on the Company's balance sheets. Marketable securities are valued using quoted prices for similar assets or liabilities in active markets and quoted prices for identical or similar assets or liabilities in markets that are not active, and are included in cash equivalents and short-term investments on the Company's consolidated balance sheets.

Deferred Offering Costs

Deferred offering costs, consisting of legal, accounting, printer and filing fees related to the proposed IPO are capitalized. The deferred offering costs will be offset against proceeds from the IPO upon completion of the offering. In the event the offering is terminated, all capitalized deferred offering costs will be immediately expensed. As of December 31, 2015 and March 31, 2016, the Company had $2.3 million and $3.0 million, respectively, of deferred offering costs, which are included in prepaid expenses and other current assets in the accompanying condensed consolidated balance sheets.

Accrued Research and Development Costs

The Company accrues for estimated costs of research and development activities conducted by third-party service providers, which include the conduct of preclinical studies and clinical trials, and contract manufacturing activities. The Company records the estimated costs of research and development activities based upon the estimated amount of services provided, and includes these costs in accrued liabilities in the consolidated balance sheets and within research and development expense in the consolidated statements of operations and comprehensive loss. These costs are a significant component of the Company's research and development expenses. The Company accrues for these costs based on factors such as estimates of the work completed and in accordance with agreements established with its third-party service providers. The Company makes significant judgments and estimates in determining the accrued liabilities balance in each reporting period.

Research and Development Costs

Research and development costs are expensed as incurred and consist primarily of personnel and consultant costs, lab supplies, allocated facility and other costs, fees paid to third parties to conduct research and development activities on the Company's behalf and expenses incurred in connection with license agreements.

Facility Lease Obligations

Rent expense is recognized on a straight-line basis over the non-cancelable term of the Company's operating leases and, accordingly, the Company records the difference between cash rent payments and the recognition of rent expense as a deferred rent asset or liability. Incentives granted under the Company's facility leases, including any allowances to fund leasehold improvements, are deferred and recognized as adjustments to rent expense on a straight-line basis over the term of the lease.

Under the terms of its sublease for manufacturing facilities, the Company assumed a restoration obligation from the previous tenant. The liability is being accreted to rent expense through the end of the lease term. In addition, upon execution of the sublease in July 2015, the Company received approximately $0.2 million of laboratory equipment for de minimis consideration. This amount has been recorded in property and equipment and will be depreciated when placed in service. The related liability will be amortized over the remaining lease term as a reduction to rent expense.

Recent Accounting Pronouncements

In August 2014, the FASB issued ASU 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*, or ASU 2014-15, which requires management to evaluate whether there is substantial doubt that the Company is able to continue operating as a going concern within one year after the date the financial statements are issued or available to be issued. If there is substantial doubt, additional disclosure is required, including the principal condition or event that raised the substantial doubt, the Company's evaluation of the condition or event in relation to its ability to meet its obligations and the Company's plan to alleviate (or, which is intended to alleviate) the substantial doubt. ASU 2014-15 is effective for interim and annual reporting periods beginning after December 15, 2016. Early adoption is permitted. The Company is currently assessing what impact, if any, the adoption of this ASU will have on its consolidated financial statements and related disclosure.

In January 2016, the FASB issued ASU No. 2016-01, *Financial Instruments - Overall* (Subtopic 825-10): *Recognition and Measurement of Financial Assets and Financial Liabilities*, or ASU 2016-01. ASU 2016-01 addresses certain aspects of recognition, measurement, presentation, and disclosure of financial instruments.

ASU 2016-01 is effective for fiscal years, and interim periods within those years, beginning after December 15, 2017, which for the Company is January 1, 2018. The Company is currently assessing what impact, if any, the adoption of this ASU will have on its consolidated financial statements and related disclosure.

In February 2016, the FASB issued ASU No. 2016-02, *Leases* (Topic 842), or ASU 2016-02. Under the new guidance, (with the exception of short-term leases) at the commencement date, lessees will be required to recognize a lease liability and a right-of-use asset. Lessor accounting is largely unchanged, while lessees will no longer be provided with a source of off-balance sheet financing. Public business entities should apply the amendments in ASU 2016-02 for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years (January 1, 2019, for the Company). Early application is permitted. Lessees (for capital and operating leases) must apply a modified retrospective transition approach for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements. The modified retrospective approach would not require any transition accounting for leases that expired before the earliest comparative period presented. The Company is currently assessing what impact the adoption of this ASU will have on its consolidated financial statements and related disclosure.

In March 2016, the FASB Issued ASU No. 2016-09, *Compensation-Stock Compensation: Improvements to Employee Share-Based Payment Accounting*, or ASU 2016-09. The updated guidance changes how companies account for certain aspects of share-based payment awards to employees, including the accounting for income taxes, forfeitures, and statutory tax withholding requirements, as well as classification in the statement of cash flows. The update to the standard is effective for fiscal years and interim periods within those years beginning after December 15, 2016, with early application permitted. The Company is currently assessing what impact, if any, the adoption of this ASU will have on its consolidated financial statements and related disclosure.

3. Cash Equivalents and Available for Sale Securities

The fair value and amortized cost of cash equivalents and available-for-sale debt securities by major security type as of December 31, 2015 and March 31, 2016 are presented in the tables that follow:

	December 31, 2015			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Market Value
	(in thousands)			
Money market funds	$19,787	$ —	$ —	$19,787
Commercial paper	3,996	—	—	3,996
Corporate debt securities	16,548	—	(8)	16,540
U.S. government agency Securities	4,016	—	(2)	4,014
Total cash equivalents and available-for sale securities	$44,347	$ —	$ (10)	$44,337

	March 31, 2016			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Market Value
	(in thousands)			
Money market funds	$17,856	$ —	$ —	$17,856
Commercial paper	9,233	—	—	9,233
Corporate debt securities	7,717	1	—	7,718
U.S. government agency Securities	6,007	3	—	6,010
Total cash equivalents and available-for sale securities	$40,813	$ 4	$ —	$40,817

Realized gains and losses on the sale of marketable securities during the three months ended March 31, 2015 and 2016 were not material.

The following tables summarize the amortized cost and estimated fair value of investments in marketable securities designated as available-for-sale and classified by the contractual maturity date of the security as of December 31, 2015 and March 31, 2016:

	December 31, 2015			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Market Value
	(in thousands)			
Less than one year	$44,347	$ —	$ (10)	$44,337
Total cash equivalents and available-for sale securities	$44,347	$ —	$ (10)	$44,337

	March 31, 2016			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Market Value
	(in thousands)			
Less than one year	$40,813	$ 4	$ —	$40,817
Total cash equivalents and available-for sale securities	$40,813	$ 4	$ —	$40,817

4. Fair Value Measurements

Assets Measured at Fair Value

The Company's financial instruments are valued using quoted prices in active markets or based upon other observable inputs. The following tables set forth the fair value of the Company's financial assets as of December 31, 2015 and March 31, 2016:

	December 31, 2015			
		Fair Value Measurements Using		
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$19,787	$19,787	$ —	$ —
Commercial paper	3,996	—	3,996	—
Corporate debt securities	16,540	—	16,540	—
U.S. government agency securities	4,014	—	4,014	—
Total financial assets	$44,337	$19,787	$24,550	$ —

	March 31, 2016			
		Fair Value Measurements Using		
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$17,856	$17,856	$ —	$ —
Commercial paper	9,233	—	9,233	—
Corporate debt securities	7,718	—	7,718	—
U.S. government agency securities	6,010	—	6,010	—
Total financial assets	$40,817	$17,856	$22,961	$ —

Liabilities Measured at Fair Value

The Company's financial liabilities are valued based upon observable inputs when available or upon estimates made by management. The following tables set forth the fair value of the Company's financial liabilities as of December 31, 2015 and March 31, 2016:

	December 31, 2015			
		Fair Value Measurements Using		
	Total	Level 1	Level 2	Level 3
		(in thousands)		
Contingent acquisition consideration payable	$4,278	$ —	$ —	$4,278
Asset retirement obligation	136	—	—	136
Total financial liabilities	$4,414	$ —	$ —	$4,414

	March 31, 2016			
		Fair Value Measurements Using		
	Total	Level 1	Level 2	Level 3
		(in thousands)		
Contingent acquisition consideration payable	$4,368	$ —	$ —	$4,368
Asset retirement obligation	218	—	—	218
Total financial liabilities	$4,586	$ —	$ —	$4,586

The Company's contingent acquisition consideration payable, resulting from the acquisition of Cardiogen Sciences, Inc. in August 2015, is estimated using a probability-based income approach utilizing an appropriate discount rate. Key assumptions used by management to estimate the fair value of contingent acquisition consideration payable include estimated probability of occurrence, the estimated timing of when the milestone may be attained and assumed discount period and discount rate. Subsequent changes in the fair value of the contingent acquisition consideration payable, resulting from management's revision of key assumptions will be recorded in research and development expense in the condensed consolidated statement of operations and comprehensive loss. The probability-based income approach used by management to estimate the fair value of the contingent acquisition consideration is most sensitive to changes in the estimated probability of occurrence.

The following is a summary of the contingent acquisition consideration payable, recorded as a non-current liability in the accompanying consolidated balance sheets:

	Amount
	(in thousands)
Balance, December 31, 2015	$4,278
Change in fair value of contingent acquisition consideration payable	90
Balance, March 31, 2016	$4,368

Under the terms of its sublease for manufacturing facilities, the Company assumed an asset restoration obligation from the previous tenant. The liability is being accreted to rent expense through the end of the lease term. The asset retirement obligation is included in facilities lease obligations in the accompanying consolidated balance sheets.

	Amount
	(in thousands)
Balance, December 31, 2015	$136
Asset retirement obligation accretion expense	82
Balance, March 31, 2016	$218

5. Balance Sheet Components

Property and Equipment, Net

Property and equipment, net, consist of the following:

	December 31, 2015	March 31, 2016
	(in thousands)	
Furniture and office equipment	$ 168	$ 171
Computer equipment	67	67
Software	87	91
Leasehold improvements	64	64
Laboratory equipment	723	723
Construction in progress	2,063	8,856
Total property and equipment	3,172	9,972
Less accumulated depreciation and amortization	(204)	(282)
Property and equipment, net	$2,968	$9,690

A portion of the Company's construction in progress is subject to mechanics' lien encumbrances while the assets remain under construction. Property and equipment depreciation and amortization expense for the three months ended March 31, 2015 and 2016 was $23,000 and $78,000, respectively.

Accrued Liabilities

Accrued liabilities consist of the following:

	December 31, 2015	March 31, 2016
	(in thousands)	
Accrued payroll and related expenses	$1,152	$ 914
Accrued research and development expenses	2,682	2,749
Accrued construction in progress	—	3,312
Accrued professional services	740	874
Other	223	1,101
Total accrued liabilities	$4,797	$8,950

Facility Lease Obligations

Facility lease obligations consist of the following as of December 31, 2015 and March 31, 2016:

	December 31, 2015			March 31, 2016		
	Long-term	Current	Total	Long-term	Current	Total
			(in thousands)			
Equipment purchase obligation	$ 44	$ 107	$ 151	$ 18	$ 107	$ 125
Asset retirement obligation	136	—	136	218	—	218
Deferred rent	339	30	369	1,195	249	1,444
	$ 519	$ 137	$ 656	$ 1,431	$ 356	$ 1,787

6. Stock-based Compensation

Under the Company's 2012 Equity Incentive Plan, 3,107,517 shares were reserved for issuance as of March 31, 2016. The plan requires that options are granted at an exercise price not less than fair market value. The following table summarizes option activity for the year ended December 31, 2015 and the three months ended March 31, 2016:

	Shares Available for Grant	Number of Options Outstanding	Weighted-Average Exercise Price Per Option	Weighted-Average Remaining Contract Term (Years)	Aggregate Intrinsic Value
					(in thousands)
Balance, December 31, 2015	751,661	2,303,862	$3.60	9.2	$ 13,593
Options granted	(200,239)	200,239	$7.54		
Options exercised	—	(93,925)	$0.82		
Options forfeited	130,796	(130,796)	$4.72		
Options cancelled	294	(294)	$2.19		
Balance, March 31, 2016	682,512	2,279,086	$3.99	9.0	$ 8,071
Exercisable, March 31, 2016		784,219	$1.84	8.3	$ 4,536
Vested and expected to vest, March 31, 2016		2,280,241	$3.84	9.0	$ 9,335

Stock-based compensation expense by category was as follows for the three months ended March 31, 2015 and 2016:

	Three Months Ended March 31,	
	2015	2016
Research and development	$ 38	$ 210
General and administrative	42	217
Total stock-based compensation expense	$ 80	$ 427
Employees	$ 48	$ 295
Non-employees	32	132
Total stock-based compensation expense	$ 80	$ 427

As of March 31, 2016, the Company had total unrecognized stock-based compensation expense related to unvested options, net of estimated forfeitures, of $4.8 million, which it expects to recognize over an estimated weighted-average period of 3.16 years.

During the three months ended March 31, 2016, 93,925 options with an intrinsic value of $707,000 were exercised. On the exercise dates, the Company received cash payments of $76,000. No options were exercised during the three months ended March 31, 2015.

The fair value of stock options for employees was estimated using a Black-Scholes option pricing model with the following assumptions:

	Three Months Ended March 31,	
	2015	2016
Fair value of employee options	$ 1.56	$ 4.64
Fair value of common stock	$ 2.19	$ 7.54
Expected term (in years)	6.1	6.1
Expected volatility	84% – 85%	68%
Risk-free interest rate	1.3% – 1.5%	1.5%
Expected dividend yield	0%	0%

The fair value of stock options for non-employees was estimated using a Black-Scholes option pricing model with the following assumptions:

	Three Months Ended March 31,	
	2015	2016
Fair value of non-employee options	$1.63 – $1.85	$5.66 – $8.21
Fair value of common stock	$2.19	$7.54 – $8.72
Expected term (in years)	8.5 – 10	7.7 – 10
Expected volatility	69% – 70%	69% – 71%
Risk-free interest rate	1.6% – 2.0%	1.7% – 2.1%
Expected dividend yield	0%	0%

7. Income Taxes

For the three months ended March 31, 2015 and 2016, the Company did not record an income tax provision. The U.S. federal deferred tax assets generated from the Company's net operating losses have been fully reserved, as the Company believes it is not more likely than not that the benefit will be realized.

8. Net Loss per Share and Pro Forma Net Loss per Share

The following table sets forth the computation of basic and diluted net loss per share of common stock during the three months ended March 2015 and 2016:

	Three Months Ended March 31,	
	2015	2016
	(in thousands, except share and per share data)	
Net loss	$ (4,055)	$ (10,464)
Shares used in computing net loss per share	612,039	2,159,081
Net loss per share, basic and diluted	$ (6.63)	$ (4.85)

The following outstanding shares of common stock equivalents were excluded from the computation of diluted net loss per share of common stock for the periods presented because including them would have been anti-dilutive:

	March 31,	
	2015	2016
Convertible preferred stock (on an as-if-converted basis)	9,447,378	13,820,301
Stock options to purchase common stock	1,121,139	2,279,086
Restricted stock subject to future vesting	124,838	—
	10,693,355	16,099,387

The following table sets forth the computation of the Company's pro forma basic and diluted net loss per common share for the three months ended March 31, 2016 (in thousands, except share and per share amounts):

Net loss	$ (10,464)
Shares used in computing pro forma net loss per share:	
Shares used in computing net loss per share, basic and diluted	2,159,081
Pro forma adjustment to reflect assumed conversion of convertible preferred stock	13,820,301
Shares used in computing pro forma net loss per share, basic and diluted	15,979,382
Pro forma net loss per share, basic and diluted	$ (0.65)

9. Related Party Transactions

Aggregate payments in connection with related party transactions totaled approximately $16,000 and $8,000 during the three months ended March 31, 2015 and 2016, respectively, and consisted of cost reimbursements paid to certain investors.

10. Subsequent Events

Solazyme Sublease

In April 2016, the Company entered into a sublease agreement with Solazyme, Inc. to sublease approximately 8,983 square feet of research and development laboratory space in South San Francisco, California with total minimum lease payments of $0.6 million over an approximately two year term.

Manufacturing Lease Option Exercise

In May 2016, the Company exercised its option to enter into a ten-year lease for its existing 22,000 square feet of manufacturing space in South San Francisco plus approximately 17,000 additional square feet of manufacturing space; the ten-year lease will become effective in June 2017.

The University of Pennsylvania Collaboration Agreement

In May 2016, the Company entered into a license and collaboration agreement with The Trustees of the University of Pennsylvania, or the University of Pennsylvania. Under the agreement, the University of Pennsylvania granted the Company an exclusive worldwide license under certain patent rights to research, develop, use, sell, offer for sale, have sold, make, have made and import licensed products for the treatment of Crigler-Najjar.

As consideration for the licensed rights, the Company paid the University of Pennsylvania an upfront fee of $0.5 million, as well as $3.0 million for certain preclinical development activities. The Company is obligated to pay the University of Pennsylvania (i) up to an aggregate of $6.0 million for preclinical development activities agreed upon by both parties, subject to adjustment based on the work plan, which amount includes the $3.0 million already paid in May 2016, (ii) up to an aggregate of $13.7 million in development, regulatory and net sales milestone payments for the first licensed product; (iii) low to mid single-digit royalty percentages on tiered annual net sales of the licensed products sold by the Company, its affiliates or sublicensees and (iv) mid single-digit to low double-digit percentages of any sublicense fees the Company receives from third parties for the grant of sublicenses to any licensed patent rights.

Under the agreement, the Company is obligated to use commercially reasonable efforts to develop, pursue regulatory approval for, market and commercialize at least one licensed product. The University of Pennsylvania will be responsible for conducting preclinical development activities according to a work plan, including all IND-enabling non-clinical studies and research grade manufacturing. The Company will be responsible for regulatory strategy and operations, clinical development, GMP manufacture and commercialization of the licensed products.

The agreement will continue on a country-by-country basis and expire upon the later of the expiration of the last valid claim of the licensed patent rights that covers the exploitation of such licensed patent rights in such country, or ten years after first commercial sale of such licensed product in such country. The Company may terminate the agreement upon 60 days' prior written notice. Either party may terminate the agreement for material breach that is not cured within a specified number of days.

Upon execution of the license and collaboration agreement with the University of Pennsylvania, the Company met the conditions of a contractual milestone pursuant to its license agreement with REGENXBIO Inc. relating to Crigler-Najjar. Subsequently, the Company made a payment of $0.4 million to REGENXBIO Inc.

Option Grants

In April 2016, the Company granted options to employees to purchase an aggregate of 82,739 shares of common stock at an exercise price of $7.54 per share, pursuant to its 2012 Equity Incentive Plan.

Reverse Split

In July 2016, the Company's board of directors approved an amendment to the Company's amended and restated certificate of incorporation to effect a reverse split of the Company's issued and outstanding common stock and convertible preferred stock at a 2.22977-to-1 ratio, which was effected on July 7, 2016. The par value and authorized shares of common stock and convertible preferred stock were not adjusted as a result of the reverse split. All issued and outstanding common stock and convertible preferred stock, options to purchase common stock and per share amounts contained in the financial statements have been retroactively adjusted to reflect the reverse stock split for all periods presented.

Through and including , 2016 (the 25th day after the date of this prospectus), all dealers effecting transactions in the Common Stock, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.

5,000,000 Shares

Common Stock

PROSPECTUS

BofA Merrill Lynch

Cowen and Company

Piper Jaffray

Wedbush PacGrow

, 2016