APPLIED GENETIC TECHNOLOGIES CORP
Form S-1/A
March 12, 2014
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As filed with the Securities and Exchange Commission on March 12, 2014.

Registration No. 333-193309

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Amendment No. 2

to

FORM S-1

REGISTRATION STATEMENT

under

THE SECURITIES ACT OF 1933

APPLIED GENETIC TECHNOLOGIES CORPORATION

(Exact Name of Registrant as Specified in Its Charter)

Delaware	2836	59-3553710
(State or Other Jurisdiction of Incorporation or Organization)	(Primary Standard Industrial Classification Code No.)	(I.R.S. Employer Identification No.)

11801 Research Drive, Suite D

Alachua, Florida 32615

(386) 462-2204

(Address, including zip code, and telephone number, including area code, of registrant s principal executive offices)

Susan B. Washer

President and Chief Executive Officer

11801 Research Drive, Suite D

Alachua, Florida 32615

(386) 462-2204

(Name, address, including zip code, and telephone number, including area code, of agent for service)

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Approximate date of commencement of proposed sale to the public: As soon as practicable after this Registration Statement is declared effective.

If any of the securities being registered on this form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, as amended (the Securities Act) please check the following box. ¨

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ¨

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ¨

If this Form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ¨

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of large accelerated filer, accelerated filer and smaller reporting company in Rule 12b2 of the Exchange Act.

Large accelerated filer ¨	Accelerated filer ¨
Non-accelerated filer x (Do not check if a smaller reporting company)	Smaller reporting company ¨

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.

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The information in this preliminary prospectus is not complete and may be changed. These securities may not be sold until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell nor does it seek an offer to buy these securities in any state where the offer or sale is not permitted.

Subject to Completion, dated March 12, 2014

PROSPECTUS

3,571,000 Shares

Common Stock

Applied Genetic Technologies Corporation

March , 2014.

This is the initial public offering of the common stock of Applied Genetic Technologies Corporation. We anticipate that the initial public offering price will be between $13.00 and $15.00 per share.

We have granted the underwriters the option to purchase up to 535,650 additional shares of common stock to cover over-allotments.

We have applied to list our shares of common stock on the NASDAQ Global Market under the symbol AGTC.

Investing in our common stock involves risks. See Risk Factors beginning on page 12 of this prospectus.

We are an emerging growth company as that term is used in the Jumpstart Our Business Startups Act of 2012 and, as such, we have elected to comply with certain reduced public company reporting requirements for this prospectus and future filings.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed on the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

	Per share	Total
Initial public offering price	$	$
Underwriting discounts and commissions (1)	$	$
Proceeds to AGTC (before expenses)	$	$

(1) We refer you to Underwriting beginning on page 158 of this prospectus for additional information regarding total underwriter compensation. Certain of our existing stockholders have indicated an interest in purchasing up to an aggregate of approximately $10.0 million in shares of our common stock in this offering 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, less or no shares in this offering to any of these stockholders, or any of these stockholders may determine to purchase more, less or no shares in this offering.

The underwriters expect to deliver the shares to purchasers on or about , 2014 through the book-entry facilities of The Depository Trust Company.

BMO Capital Markets Wedbush PacGrow Life Sciences

Cantor Fitzgerald & Co. Roth Capital Partners

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You should rely only on the information contained in this prospectus and any free writing prospectus prepared by us or on our behalf or to which we have referred you. We and the underwriters have not authorized anyone to provide you with information that is different. We and the underwriters are offering to sell shares of our common stock, and seeking offers to buy shares of our common stock, only in jurisdictions where such offers and sales are permitted. Regardless of the time of delivery of this prospectus or any free writing prospectus or any sale of our common stock, the information in this prospectus is accurate only as of the date of this prospectus, and the information in any free writing prospectus that we may provide you in connection with this offering is accurate only as of the date of that free writing prospectus. Our business, financial condition, results of operations and future growth prospects may have changed since those dates.

Until , 25 days after the date of this prospectus, all dealers that buy, sell or trade our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This requirement is in addition to the dealer s obligation to deliver a prospectus when acting as an underwriter and with respect to unsold allotments or subscriptions.

For investors outside the United States: Neither we nor any of the underwriters have taken any action to permit a public offering of the shares of our common stock or the possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than the United States. You are required to inform yourselves about and to observe any restrictions relating to this offering and the distribution of this prospectus and any free writing prospectus outside of the United States.

Estimates in this prospectus of the patient populations for the diseases that we are targeting are based on published estimates of the rates of incidence of the diseases from scientific and general publications and research, surveys and studies conducted by third parties that we consider to be reliable, although such publications do not guarantee the accuracy or completeness of such information. We assume populations of approximately 300 million persons in the United States and approximately 500 million persons in Europe.

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PROSPECTUS SUMMARY

This summary highlights information contained elsewhere in this prospectus. You should read the following summary together with the more detailed information appearing in this prospectus, including our financial statements and related notes and the risk factors beginning on page 12 before deciding whether to purchase shares of our common stock. Unless the context otherwise requires, we use the terms AGTC, Company, we, us and our in this prospectus to refer to Applied Genetic Technologies Corporation.

Overview

We are a clinical-stage biotechnology company that uses our proprietary gene therapy platform to develop products designed to transform the lives of patients with severe inherited orphan diseases in ophthalmology. Our lead product candidates, which are each in the preclinical stage, focus on rare diseases of the eye, caused by mutations in single genes, that significantly affect visual function and currently lack effective medical treatments. We have also obtained preliminary evidence of the safety and efficacy of our gene therapy approach in clinical-stage programs involving other diseases outside our current area of focus that we believe provide proof of concept for our gene therapy platform.

Our gene therapy approach uses a viral vector to deliver a functional copy of a gene to the patient s own cells through a variety of delivery methods. A viral vector is a virus that has been modified to carry a gene and deliver it to a cell. Our viral vectors utilize a modified version of a non-replicating strain of virus known as an adeno-associated virus, or AAV, which is incapable of causing disease in humans. When an AAV vector containing a functional copy of a gene is administered, the functional genetic material resides in the nucleus of the patient s cell, providing safe, sustained expression of the therapeutic protein to treat the disease without modifying the existing DNA of the patient.

We have developed extensive internal expertise in viral vector design, delivery and manufacturing that is supported by a broad intellectual property estate. Our proprietary AAV vector manufacturing process is both reproducible and scalable. We have assembled an experienced management team and a world-class group of scientific advisors, and we have strong collaborative relationships with key opinion leaders in the field of gene therapy. Combining these attributes, we have built a gene therapy platform that we believe will provide patients with treatments that may have life-long clinical benefits, potentially based on a one-time therapeutic administration.

Our product pipeline

Our lead product candidates are designed to treat:

X-linked retinoschisis, or XLRS. XLRS is an inherited retinal disease caused by mutations in the RS1 gene, which encodes the retinoschisin protein. It is characterized by abnormal splitting of the layers of the retina, resulting in poor visual acuity in young boys, which can progress to legal blindness in adult men. In preclinical studies, treatment by injection of our XLRS product candidate in mice improved responses to light in the retina and visual acuity. In late 2014, we plan to submit an Investigational New Drug Application, or IND, to the United States Food and Drug Administration, or FDA, and to initiate a Phase 1/2 clinical trial in XLRS, with initial clinical data expected in mid-2015.

Achromatopsia, or ACHM. ACHM is an inherited retinal disease, which is present from birth and is characterized by the lack of cone photoreceptor function. The condition results in markedly reduced visual acuity, light sensitivity, day blindness and complete loss of color discrimination. Best-corrected visual acuity in persons affected by ACHM, even under subdued light conditions, is usually about 20/200, a level at which people are considered legally blind. Preclinical studies in both mouse and dog models of our

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ACHM product candidate have shown the ability to restore photoreceptor function, improve visual acuity and mitigate light sensitivity and day blindness. In early 2015, we plan to submit an IND and to initiate a Phase 1/2 clinical trial in one form of ACHM, with initial clinical data expected in late 2015.

X-linked retinitis pigmentosa, or XLRP. XLRP is an inherited retinal dystrophy characterized by the progressive loss of vision, one form of which is caused by mutations in the RPGR gene, which encodes a protein essential for normal vision. It is commonly first observed in young men, who notice problems with vision under low light conditions, or night blindness, followed by tunnel vision, leading to poor central vision and eventual total blindness. A preclinical study in a dog model of XLRP caused by mutations in the RPGR gene demonstrated a delay in the rate of disease progression in dogs that received a subretinal injection of our XLRP product candidate.

We initially developed our gene therapy platform in clinical-stage proof-of-concept programs involving three other diseases:

Leber congenital amaurosis (type 2), or LCA2, an orphan eye disease caused by mutation in the RPE65 gene;

the wet form of age-related macular degeneration, or wet AMD, an eye disease affecting a large patient population; and

Alpha-1 antitrypsin deficiency, or AAT deficiency, an inherited orphan lung disease.

While not our principal focus at this time, these proof-of-concept programs are important because they have provided initial evidence of safety and efficacy of our gene therapy approach in both preclinical studies and clinical trials. They have also enabled us to develop substantial experience in vector design, delivery and manufacturing, clinical trial design and conduct, and in working with clinical investigators and regulatory agencies. In these proof-of-concept programs, our manufacturing process has been successfully vetted by regulatory agencies and partners and we have demonstrated our ability to produce clinical material for multiple studies.

In clinical trials conducted by our licensee Genzyme Corporation, or Genzyme, up to 34 patients with wet AMD were treated by intravitreal injection of an AAV vector, and in other trials conducted by us and others, more than 50 patients with LCA2 have been treated with subretinal injections of AAV vectors, in both cases without reports of serious adverse events attributed to the vector, and with promising indications of efficacy for LCA2 patients. See Business Strategic collaborations Our license to Genzyme. We believe our AAT deficiency program provides proof of concept for the use of our gene therapy platform in indications outside our focus area of orphan ophthalmology. We have conducted Phase 1 and Phase 2 clinical trials for our AAT deficiency product candidate in 30 patients and expect to start a Phase 2b trial in early 2015, with initial clinical data expected in mid-2015.

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The chart below summarizes our current gene therapy programs:

Our gene therapy platform

Our gene therapy platform is built on our core competencies in three key areas: vector selection and design, vector manufacturing and vector delivery:

Vector selection and design. The success of a gene therapy platform is highly dependent on the vector selected. Our gene therapy platform is based on viral vectors that utilize a modified version of the non-replicating adeno-associated virus to deliver a functional copy of a gene to the patient s own cells. We believe that AAV vectors are particularly well-suited for treating our target diseases and offer advantages including safety, stability and sustained expression compared with viral vectors such as adenovirus, herpes virus and lentivirus used by others. AAV vectors can carry genes of up to 4,000 base pairs in length, a carrying capacity sufficient to accommodate more than 90% of human genes.

One of our key capabilities is our understanding of the complex interplay between the clinical disease, the cells in the patient s body that need treatment, the selection of the protein shell, or capsid, and a promoter, the design of the gene construct and the physical administration method. We have spent years

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conducting research on the best combinations of these elements with the aim of developing safe and effective gene therapy treatments.

Vector manufacturing. We have developed a proprietary, high-yield vector manufacturing process using scalable technologies, which addresses problems of low productivity and low efficacy that have historically plagued efforts to manufacture AAV vectors and enables us to produce vectors with improved potency, efficiency and safety over processes previously used by us and others.

Our manufacturing process has been reviewed by both the FDA and the European Medicines Agency, or EMA, has been authorized for production of product candidates for use in clinical trials in the United States and Europe and has been transferred successfully to Genzyme and to our contract manufacturing organization. We hold or have licensed 79 issued and 28 pending patents covering our manufacturing technology. We believe that our core competency and intellectual property estate in vector manufacturing differentiate us competitively and provide a key element of our gene therapy platform.

Vector delivery. Our gene therapy platform allows for vector delivery by a variety of methods, and we select the method that is most beneficial for the disease we are targeting. In ophthalmology, the product candidate can best be delivered to cells in the eye by injection. For other indications, such as AAT deficiency, we plan to administer the product candidate by intramuscular injection or vascular delivery. These methods of administration are well-established for the safe and effective delivery of other drugs and protein products.

Because our AAV vectors can be used to introduce functional genes into many different cell types and by a variety of delivery methods and have a carrying capacity sufficient to accommodate most of the individual genes in the human genome, our gene therapy platform has the potential to provide treatments for many other diseases outside of our current focus on orphan ophthalmology, including those with large dosing requirements or in larger markets. We have already conducted preclinical proof-of-concept studies and Phase 1 and Phase 2 clinical trials of a treatment for AAT deficiency. We expect to explore other therapeutic areas selectively, either alone or through partnerships.

Our focus on orphan ophthalmology

We focus on orphan ophthalmology because we believe there is a significant unmet medical need in orphan eye diseases that provides an attractive business opportunity. The prevalence of the diseases we are pursuing is large by orphan standards, but small enough to permit clinical trials on a manageable scale and to provide markets that we believe can be served using a small, targeted commercial infrastructure. The eye diseases we are targeting are also of interest to us due to a number of factors that have enabled us to predict the potential safety and efficacy of our product candidates at an early stage of development:

- these diseases involve well-understood disease mechanisms;
- these are monogenic diseases, meaning they are caused by mutations in a single gene, which mitigates the uncertainty of disease biology;
- highly predictive animal models are available;
- local delivery of the therapeutic agent is possible via methods already widely used in ophthalmology;
- these diseases have clearly defined clinical endpoints that have been accepted by regulatory agencies in review of other ophthalmology products; and
- we anticipate a short time to meaningful clinical data.

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Our strategy

Our objective is to become the world leader in developing and commercializing gene therapy treatments for inherited orphan diseases in ophthalmology, for which there are no currently available treatments, and to thereby provide a better life for people with these diseases. Our strategy to accomplish this goal is to:

- develop and commercialize drugs in orphan ophthalmology;
- continue our leadership position in orphan ophthalmology;
- extend our expertise in AAV vector selection and design, delivery and manufacturing;
- pursue monogenic orphan indications with high unmet medical need and greater probability of clinical, regulatory and commercial success; and
- develop and partner selectively to expand the scope of our pipeline and the utilization of our gene therapy platform.

Risks associated with our business

Our ability to implement our business strategy is subject to numerous risks that you should be aware of before making an investment decision. These risks are described more fully in the section entitled Risk Factors beginning on page 12 of this prospectus. You are encouraged to read that section in its entirety before making an investment decision. These risks include, but are not limited to, the following:

- We have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future.
- Our ability to generate revenue from product sales is highly uncertain and we may never achieve or sustain profitability.
- In order to obtain regulatory approval for and commercialize our product candidates we will need to raise additional funding in the future, which may not be available on acceptable terms, or at all.
- All of our product candidates are in preclinical or clinical development. Clinical drug development is expensive, time consuming and uncertain, and we may ultimately not be able to obtain regulatory approvals for the commercialization of some or all of our product candidates.
- Our gene therapy product candidates are based on a novel technology, no gene therapy products have been approved in the United States and only one such product has been approved in Europe, which makes it difficult to predict the time and cost of product candidate development and regulatory approval.
- Success in animal studies or early clinical trials may not be indicative of results obtained in later trials.

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

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

We expect to rely on third parties to conduct, supervise and monitor our clinical trials and to conduct certain aspects of our product manufacturing and protocol development, and if these third parties perform in an unsatisfactory manner, it may harm our business.

The insurance coverage and reimbursement status of our product candidates is uncertain, and failure to obtain or maintain adequate coverage and reimbursement for new or current products could limit our ability to market those products and decrease our ability to generate revenue.

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

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

Corporate information

We were incorporated in Florida in January 1999 and reincorporated in Delaware in October 2003. Our principal executive offices are located at 11801 Research Drive, Suite D, Alachua, Florida 32615, and our telephone number is (386) 462-2204. Our corporate website address is www.agtc.com. Information contained on or accessible through 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.

We use AGTC and the double helix logo as trademarks in the United States and other countries. We have begun the registration process for these trademarks in the United States.

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

Implications of being an emerging growth company

As a company with less than $1 billion in revenue during our last fiscal year, we qualify as an emerging growth company as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. As an emerging growth company, we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include:

only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced Management s Discussion and Analysis of Financial Condition and Results of Operations disclosure;

reduced disclosure about our executive compensation arrangements;

no non-binding advisory votes on executive compensation or golden parachute arrangements; and

exemption from the auditor attestation requirement in the assessment of our internal controls over financial reporting.

We may take advantage of these exemptions for up to five years or such earlier time that we are no longer an emerging growth company. We would cease to be an emerging growth company if we have more than $1 billion in annual revenue, we have more than $700 million in market value of our stock held by non-affiliates or we issue more than $1 billion of non-convertible debt over a three-year period. We may choose to take advantage of some, but not all, of the available exemptions. We have taken advantage of certain reduced reporting burdens in this prospectus. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock.

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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.

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The Offering

Common stock offered by AGTC	3,571,000 shares
Common stock to be outstanding after this offering	12,800,120 shares (13,335,770 shares in the event the underwriters elect to exercise in full their over-allotment option to purchase additional shares from us)
Use of proceeds	We estimate that the net proceeds from this offering will be approximately $44.8 million, or approximately $51.8 million if the underwriters exercise in full their over-allotment option, based on the assumed initial public offering price of $14.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We plan to use the net proceeds from this offering to extend development of our XLRS and ACHM product candidates beyond Phase 1/2 trials (which we believe are already adequately funded), and if successful to initiate pivotal Phase 3 trials for these product candidates, to continue preclinical studies of our XLRP product candidate and to explore in early preclinical studies potential applications of our gene therapy platform in other indications in orphan ophthalmology. We intend to use remaining amounts for working capital and other general corporate purposes. We may also use a portion of the net proceeds to in-license, acquire or invest in complementary gene therapy businesses, technologies, products or assets, as well as to selectively explore potential applications of our gene therapy platform in indications outside of orphan ophthalmology. See Use of Proceeds.
Risk factors	You should read the Risk Factors section and other information included in this prospectus for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
Proposed NASDAQ Global Market symbol	AGTC

The number of shares of our common stock to be outstanding after this offering set forth above is based on the 109,039 shares of our common stock outstanding as of December 31, 2013 and assumes the conversion of all outstanding shares of our preferred stock into 9,120,081 shares of common stock upon the closing of this offering.

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

- 59,247 shares of common stock issuable upon the exercise of preferred stock warrants outstanding as of December 31, 2013, at a weighted average exercise price of $8.16 per share;
- 782,947 shares of common stock issuable upon the exercise of stock options outstanding under our 2001 Stock Option Plan and our 2011 Stock Incentive Plan as of December 31, 2013, at a weighted average exercise price of $3.23 per share;

250,204 shares of common stock available for future issuance under our 2001 Stock Option Plan and our 2011 Stock Incentive Plan as of December 31, 2013; and

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an additional 1,279,999 shares of our common stock that will be made available for future issuance under our equity compensation plans upon the closing of this offering.

Except as otherwise noted, all information in this prospectus:

gives effect to a 1-for-35 reverse split of our common stock effected on March 4, 2014;

assumes no exercise of outstanding options or warrants described above;

assumes no exercise by the underwriters of their over-allotment option to purchase 535,650 additional shares of common stock from us;

gives effect to the automatic conversion of all outstanding shares of our preferred stock into 9,120,081 shares of our common stock upon the closing of this offering; and

gives effect to the amendment and restatement of our certificate of incorporation and bylaws upon the closing of this offering.

Certain of our existing stockholders have indicated an interest in purchasing up to an aggregate of approximately $10.0 million in shares of our common stock in this offering 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, less or no shares in this offering to any of these stockholders, or any of these stockholders may determine to purchase more, less or no shares in this offering.

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Summary Financial Data

The following summary financial data should be read together with our financial statements and accompanying notes and Management s Discussion and Analysis of Financial Condition and Results of Operations appearing elsewhere in this prospectus. Our summary statement of operations data for the fiscal years ended June 30, 2012 and 2013 and our summary balance sheet data as of June 30, 2012 and 2013 are derived from our audited financial statements included elsewhere in this prospectus. Our summary statement of operations data for the six months ended December 31, 2012 and 2013 and our summary balance sheet data as of December 31, 2013 have been derived from our unaudited financial statements included elsewhere in this prospectus. Our historical results are not necessarily indicative of results to be expected for any future period, and our interim results are not necessarily indicative of our results for the entire year or any future period. The summary financial data in this section are not intended to replace our financial statements and the related notes.

The pro forma balance sheet data as of December 31, 2013 gives effect to the conversion of all of our preferred stock into 9,120,081 shares of common stock upon the closing of this offering and the conversion of all outstanding warrants exercisable for shares of Series A-1, Series A-1A and Series B-1 preferred stock into warrants exercisable for shares of common stock, resulting in our preferred stock warrant liability being reclassified to additional paid-in capital. The pro forma as adjusted balance sheet data as of December 31, 2013 gives effect to (1) the pro forma adjustments described above and (2) our receipt of estimated net proceeds of $44.8 million from this offering, based on the assumed initial public offering price of $14.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and estimated offering expenses payable by us, as if each had occurred as of December 31, 2013. The pro forma as adjusted summary financial data are not necessarily indicative of what our financial position would have been if this offering had been completed as of the date indicated, nor are these data necessarily indicative of our financial position for any future date or period.

	Fiscal Year Ended June 30,		Six Months Ended December 31,	
	2012	2013	2012	2013
	(in thousands except per share data)			
Statement of Operations Data:				
Revenue:				
Grant revenue	$ 718	$ 439	$ 293	$ 466
Sponsored research revenue	364	503	156	307
Total revenue	1,082	942	449	773
Operating expenses:				
Research and development	2,354	3,133	1,156	3,673
General and administrative	787	1,403	649	1,971
Total operating expenses	3,141	4,536	1,805	5,644
Loss from operations	(2,059)	(3,594)	(1,356)	(4,871)

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	Fiscal Year Ended June 30,		Six Months Ended December 31,	
	2012	2013	2012	2013
	(in thousands except per share data)			
Other income (expense):				
Interest income		10		15
Interest expense	(69)	(191)	(152)	
Fair value adjustments to warrant liabilities (1)	204	(8)	5	(105)
Fair value adjustments to Series B purchase rights (1)		(1,207)	(437)	(2,838)
Total other income (expense), net	135	(1,396)	(584)	(2,928)
Net loss	$ (1,924)	$ (4,990)	$ (1,940)	$ (7,799)
Net loss per share, basic and diluted (2)	$ (17.65)	$ (45.78)	$ (17.80)	$ (71.55)
Weighted-average shares outstanding, basic and diluted (2)	109	109	109	109
Pro forma net loss per share, basic and diluted (unaudited) (2)		$ (1.20)		$ (1.13)
Weighted-average pro forma shares outstanding, basic and diluted (unaudited) (2)		4,146		6,885

	As of June 30,		As of December 31, 2013		
	2012	2013	Actual	Pro Forma	Pro Forma As Adjusted (3)
			(in thousands)		
Balance Sheet Data:					
Cash and cash equivalents	$ 774	$ 8,893	$ 8,998	$ 8,998	$ 53,842
Short-term investments	$	$ 14,000	$ 19,000	$ 19,000	$ 19,000
Working capital	$ (399)	$ 20,051	$ 28,106	$ 28,106	$ 72,950
Total assets	$ 2,824	$ 25,490	$ 31,709	$ 31,709	$ 76,553
Current liabilities	$ 1,494	$ 3,460	$ 1,644	$ 1,644	$ 1,644
Total stockholders (deficit) equity	$ (31,290)	$ (36,183)	$ (43,871)	$ 30,065	$ 74,909

(1) See note 6 of the notes to financial statements appearing elsewhere in this prospectus for a description of the fair value adjustments to our warrant liabilities and Series B purchase rights.

(2) See note 2 of the notes to financial statements appearing elsewhere in this prospectus for a description of the method used to calculate basic and diluted net loss per share and pro forma basic and diluted net loss per share.

(3) A $1.00 increase (decrease) in the assumed initial public offering price of $14.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, working capital, total assets and total stockholders (deficit) equity by approximately $3.3 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 estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, a one million share increase (decrease) in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) each of cash and cash equivalents, working capital, total assets and total stockholders equity (deficit) by approximately $13.0 million, assuming the initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus) remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

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RISK FACTORS

Investing in our common stock involves a high degree of risk. Before you decide to invest in our common stock, you should consider carefully the risks described below, together with the other information contained in this prospectus, including our financial statements and the related notes appearing at the end of this prospectus. We believe the risks described below are the risks that are material to us as of the date of this prospectus. If any of the following risks actually occur, our business, financial condition, results of operations and future growth prospects would likely be materially and adversely affected. In these circumstances, the market price of our common stock could decline, and you may lose part or all of your investment.

Risks related to our financial condition and capital requirements

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

We are a clinical-stage biotechnology company, and we have not yet generated revenues from product sales. We have incurred losses from operations in each year since our inception in 1999, and net losses of $1.9 million and $5.0 million for the years ended June 30, 2012 and 2013, respectively. As of December 31, 2013, we had an accumulated deficit of $56.2 million. Our prior losses, combined with expected future losses, have had and may continue to have an adverse effect on our stockholders equity and working capital.

We have devoted most of our financial resources to research and development, including our clinical and preclinical development activities. To date, we have financed our operations primarily through the sale of equity securities and, to a lesser extent, through research grants from third parties or milestone payments from a collaborator. The amount of our future net losses will depend, in part, on the rate of our future expenditures and our ability to obtain funding through equity or debt financings, strategic collaborations or additional grants. We have not begun clinical trials for our lead product candidates and it will be several years, if ever, before we have a product candidate ready for commercialization. Even if we obtain regulatory approval to market a product candidate, our future revenues will depend upon the size of any markets in which our product candidates have received approval, and our ability to achieve sufficient market acceptance, reimbursement from third-party payors and adequate market share for our product candidates in those markets.

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

- continue our research and preclinical and clinical development of our product candidates;
- expand the scope of our current clinical trials for our product candidates;
- initiate additional preclinical studies, clinical trials or other studies for our product candidates;
- further develop our gene therapy platform, including the process for design, delivery and manufacturing of our vectors for our product candidates;
- change or add additional manufacturers or suppliers;
- seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;

seek to identify and validate additional product candidates;

acquire or in-license other product candidates and technologies;

make milestone or other payments under any in-license agreements;

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maintain, protect and expand our intellectual property portfolio;

attract and retain skilled personnel;

create additional infrastructure to support our operations as a public company and our product development and planned future commercialization efforts; and

experience any delays or encounter issues with any of the above.

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

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

All of our revenue generated to date has come from research grants from third parties or license fees or milestone payments from a collaborator. Our ability to generate substantial revenue and achieve profitability depends on our ability, alone or with strategic collaboration partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our product candidates. We do not anticipate generating revenues from product sales for at least the next several years, if ever. If any of our product candidates fail in clinical trials or do not gain regulatory approval, or if any of our product candidates, if approved, fail to achieve market acceptance, we may never become profitable. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our ability to generate future revenues from product sales depends heavily on our success in:

completing research and preclinical and clinical development of our product candidates;

seeking and obtaining regulatory and marketing approvals for product candidates for which we complete clinical trials;

establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate (in amount and quality) products and services to support clinical development and the market demand for our product candidates, if approved;

launching and commercializing product candidates for which we obtain regulatory and marketing approval, either by collaborating with a partner or, if launched independently, by establishing a sales, marketing and distribution infrastructure;

obtaining and maintaining adequate coverage and reimbursement from third-party payors for our product candidates;

obtaining market acceptance of our product candidates and gene therapy as a viable treatment option;

addressing any competing technological and market developments;

implementing additional internal systems and infrastructure, as needed;

identifying and validating new gene therapy product candidates;

negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;

maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how; and

attracting, hiring and retaining qualified personnel.

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

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

All of our lead programs in orphan ophthalmology are currently in preclinical development. Developing gene therapy products is expensive, and we expect our research and development expenses to increase substantially as we advance our current product candidates in clinical trials and as we undertake preclinical studies of new product candidates.

Our operations have consumed substantial amounts of cash since inception. As of December 31, 2013, our cash and cash equivalents and short-term investments were $28.0 million. Our research and development expenses were $2.4 million and $3.1 million for the fiscal years ended June 30, 2012 and 2013, respectively, and $1.2 million and $3.7 million for the six months ended December 31, 2012 and 2013, respectively. We estimate that the net proceeds from this offering will be approximately $44.8 million, based on the assumed initial public offering price of $14.00 per share, which is the midpoint of the price range set forth on the cover of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. We expect that the net proceeds from this offering and our existing cash and cash equivalents will be sufficient to enable us to complete planned preclinical studies and clinical trials for our lead product candidates for at least the next 24 months. See Use of Proceeds. In order to complete the process of obtaining regulatory approval for our lead product candidates and to build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize our lead product candidates, if approved, we will require substantial additional funding. Also, our current operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements, or a combination of these approaches.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, financing may not be available to us in the future in sufficient amounts or on terms acceptable to us, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all of our stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and a portion of our operating cash flows, if any, being dedicated to the payment of principal and interest on such indebtedness, and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available, and we may be required to relinquish or license on unfavorable terms rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, financial condition, results of operations and prospects and cause the price of our common stock to decline.

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If we are unable to obtain needed funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition, results of operations and prospects and cause the price of our common stock to decline.

Risks related to the discovery and development of our product candidates

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

The research, testing, manufacturing, labeling, approval, selling, marketing and distribution of drug products are subject to extensive regulation by the FDA and other regulatory authorities, which regulations differ from country to country. Our product candidates are in various stages of development and are subject to the risks of failure typical of drug development. The development and approval process is expensive and can take many years to complete, and its outcome is inherently uncertain. We have not submitted an application for or received marketing approval for any of our product candidates. We have limited experience in conducting and managing the later stage clinical trials necessary to obtain regulatory approvals, including approval by the FDA. To receive approval, we must, among other things, demonstrate with substantial evidence from clinical trials that the product candidate is both safe and effective for each indication for which approval is sought, and failure can occur in any stage of development. Satisfaction of the approval requirements typically takes several years and the time needed to satisfy them may vary substantially, based on the type, complexity and novelty of the pharmaceutical product. We cannot predict if or when we might receive regulatory approvals for any of our product candidates currently under development.

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

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical or clinical significance required by the FDA or comparable foreign regulatory authorities for approval;
- the patients recruited for a particular clinical program may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- the results may not confirm the positive results from earlier preclinical studies or clinical trials;
- we may be unable to demonstrate that a product candidate s clinical and other benefits outweigh its safety risks;

the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;

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- the data collected from clinical trials of our product candidates may not be sufficient to the satisfaction of FDA or comparable foreign regulatory authorities to support the submission of a biologics license application, or BLA, or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;

- regulatory agencies might not approve or might require changes to our manufacturing processes or facilities; or

- regulatory agencies may change their approval policies or adopt new regulations in a manner rendering our clinical data insufficient for approval.

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

We are not permitted to market our product candidates in the United States or in other countries until we receive approval of a BLA from the FDA or marketing approval from applicable regulatory authorities outside the United States. Obtaining approval of a BLA can be a lengthy, expensive and uncertain process. If we fail to obtain FDA approval to market our product candidates, we will be unable to sell our product candidates in the United States, which will significantly impair our ability to generate any revenues. In addition, failure to comply with FDA and non-U.S. regulatory requirements may, either before or after product approval, if any, subject our company to administrative or judicially imposed sanctions, including:

- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;

- restrictions on the products, manufacturers or manufacturing process;

- warning letters;

- civil and criminal penalties;

- injunctions;

- suspension or withdrawal of regulatory approvals;

- product seizures, detentions or import bans;

- voluntary or mandatory product recalls and publicity requirements;

- total or partial suspension of production;

- imposition of restrictions on operations, including costly new manufacturing requirements; and

refusal to approve pending BLAs or supplements to approved BLAs.

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

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Our gene therapy product candidates are based on a novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. At the moment, no gene therapy products have been approved in the United States and only one such product has been approved in Europe.

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

In addition, the clinical trial requirements of the FDA, the EMA and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidates. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates. At the moment, only one gene therapy product, uniQure B.V. s Glybera, which received marketing authorization from the EMA in 2012, has been approved in Europe but has not yet been launched for commercial sale, 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 either the United States or Europe. Approvals by the EMA may not be indicative of what the FDA may require for approval.

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

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected for orphan ophthalmology product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue to maintain our business.

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Success in animal studies or early clinical trials may not be indicative of results obtained in later trials.

Trial designs and results from animal studies or previous clinical trials are not necessarily predictive of our future clinical trial designs or results, and interim results of a clinical trial are not necessarily indicative of final results. Our product candidates may also fail to show the desired safety and efficacy in clinical development despite demonstrating positive results in animal studies or having successfully advanced through initial clinical trials. For example, our animal studies of our AAT product candidate resulted in evidence of significant production of AAT levels, but early clinical trials of our product candidate showed significantly lower levels of AAT production in treated patients. There can be no assurance that the success we achieved in the animal studies for our lead product candidates will result in success in our clinical trials of those product candidates.

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

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

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on the speed at which we can recruit patients to participate in testing our product candidates as well as completion of required follow-up periods. If patients are unwilling to participate in our gene therapy studies because of negative publicity from adverse events in the biotechnology or gene therapy industries or for other reasons, including competitive clinical trials for similar patient populations, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of our product candidates may be delayed. For example, trials using early versions of lentiviral vectors, which integrate with, and thereby alter, the host cell s DNA, have led to several well-publicized adverse events, including reported cases of leukemia. If there are delays in accumulating the required number of clinical events in trials for our product candidates where clinical events are a primary endpoint, there may be delays in completing the trial. These delays could result in increased costs, delays in advancing our product candidates, delays in testing the effectiveness of our technology or termination of the clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a trial, to complete our clinical trials in a timely manner. In particular, each of the conditions for which we plan to evaluate our product candidates are rare genetic disorders with limited patient pools from which to draw for clinical trials. The eligibility criteria of our clinical trials will further limit the pool of available trial participants.

Patient enrollment is affected by factors including:

- severity of the disease under investigation;
- design of the clinical trial protocol;
- size and nature of the patient population;
- eligibility criteria for the trial in question;
- perceived risks and benefits of the product candidate under trial;
- proximity and availability of clinical trial sites for prospective patients;

availability of competing therapies and clinical trials;

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clinicians and patients perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating;

efforts to facilitate timely enrollment in clinical trials;

patient referral practices of physicians; and

our ability to monitor patients adequately during and after treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may be forced to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business. We could encounter delays if physicians encounter unresolved ethical issues associated with enrolling patients in clinical trials of our product candidates in lieu of prescribing existing treatments that have established safety and efficacy profiles.

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

difficulty in establishing or managing relationships with contract research organizations, or CROs, and physicians;

different standards for conducting clinical trials;

our inability to locate qualified local consultants, physicians and partners; and

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

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

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of such product candidates in humans. Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

delays in raising, or inability to raise, sufficient capital to fund the planned clinical trials;

inability to generate sufficient preclinical, toxicology, or other data to support the initiation of human clinical trials;

delays in reaching a consensus with regulatory agencies on trial design;

identifying, recruiting and training suitable clinical investigators;

delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;

delays in obtaining required IRB approval at each clinical trial site;

delays in recruiting suitable patients to participate in our clinical trials;

delays due to changing standard of care for the diseases we are targeting;

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adding new clinical trial sites;

imposition of a clinical hold by regulatory agencies, after review of an IND application or equivalent application or an inspection of our clinical trial operations or trial sites;

failure by our CROs, other third parties or us to adhere to clinical trial requirements;

loss of product due to shipping delays or delays in customs in connection with delivery to foreign countries for use in clinical trials;

failure to perform in accordance with the FDA s good clinical practices, or GCP requirements or applicable regulatory guidelines in other countries;

inability to manufacture, test, release, import or export for use sufficient quantities of our product candidates for use in clinical trials;

failure to manufacture our product candidate in accordance with the FDA s good manufacturing practice, or GMP, requirements or applicable regulatory guidelines in other countries;

delays in the testing, validation and delivery of our product candidates to the clinical trial sites;

delays in having patients complete participation in a trial or return for post-treatment follow-up;

clinical trial sites deviating from trial protocol or clinical trial sites or patients dropping out of a trial;

occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;

changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;

the costs of clinical trials of our product candidates may be greater than we anticipate; or

clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon drug development programs.

Further, a clinical trial may be suspended or terminated by us, our collaborators, the IRBs, in the institutions in which such trials are being conducted, the Data Safety Monitoring Board, or DSMB, for such trial, or by the FDA or other regulatory authorities due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience termination of, or delays in the completion of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenues from any of these product candidates will be delayed. Furthermore, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

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

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

- be delayed in obtaining marketing approval for our product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes with the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

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

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

As with many pharmaceutical and biological products, treatment with our product candidates may produce undesirable side effects or adverse reactions or events. These adverse events may occur despite our belief that our AAV vectors have an improved safety profile over prior such treatments.

Known adverse side effects that could occur with treatment with AAV vectors include an immunologic reaction to the capsid protein or gene at early timepoints after administration. In previous clinical trials involving AAV viral vectors for gene therapy, some subjects experienced serious adverse events, including the development of T-cell response due to immune response against the vector capsid proteins. If our vectors demonstrate a similar effect, or other adverse events, we may be required to halt or delay further clinical development of our product candidates. In addition, theoretical adverse side effects of AAV vectors include replication and spread of the virus to other parts of the body and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, also known as cancer, which could potentially enhance the risk of malignant transformation. Potential procedure-related events, including inflammation or injury to the eye, are similar to those associated with standard ophthalmic intervention procedures. There is also the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material.

If any such adverse events occur, our clinical trials could be suspended or terminated and the FDA, the EMA or other foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. The product-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial. If we elect or are required to delay, suspend or terminate any clinical trial of any of our product candidates, the commercial prospects of such product candidates will be harmed and our ability to generate product revenues from any of these product candidates will be delayed or eliminated. Any of these occurrences may harm our business, financial condition and prospects significantly.

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

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

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

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

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals diagnosed annually in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, the EMA s Committee for Orphan Medicinal Products, or COMP, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than 5 in 10,000 persons in the European Union Community. Additionally, designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biological product. Our product candidates for the treatment of LCA2, XLRS, ACHM (in the form caused by mutations in the CNGB3 gene) and AAT deficiency have been granted orphan drug designations by the FDA, but at this time we have neither requested nor obtained orphan drug designation for any of our other product candidates. Even if we request orphan drug designation for our other product candidates, there can be no assurances that the FDA will grant any of our product candidates such designation. Additionally, the designation by the FDA of any of our product candidates as an orphan drug does not guarantee that the FDA will accelerate regulatory review of or ultimately approve that product candidate.

Generally, if a product candidate with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the EMA or the FDA from approving another marketing application for the same drug and indication for that time period, except in limited circumstances. The applicable period is seven years in the United States and 10 years in Europe. The European exclusivity period can be reduced to six years if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or EMA determines

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that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

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

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

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

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

Even if we obtain regulatory approval in a jurisdiction for our product candidates, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, and submission of safety and other post-market information. Any regulatory approvals that we receive for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. FDA guidance advises that patients treated with some types of gene therapy undergo follow-up observations for potential adverse events for as long as 15 years. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with GMP requirements and adherence to commitments made in the BLA or foreign marketing application. If we or a regulatory agency discovers previously unknown problems with a product such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured or disagrees with the promotion, marketing or labeling of that product, a regulatory agency may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

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

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

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

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

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

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries must also approve the manufacturing and marketing of the product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our products, if approved, is also subject to approval. We intend to submit a marketing authorization application to the EMA for approval in the EEA, but obtaining such approval is a lengthy and expensive process and the EMA has its own procedures for approval of product candidates.

Even if a product candidate is approved, the FDA or the EMA, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the EEA also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries.

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

Risks related to our reliance on third parties

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

We do not expect to independently conduct all aspects of our vector production, product manufacturing, protocol development, and monitoring and management of our ongoing and planned preclinical and clinical programs. We currently rely, and expect to continue to rely, on third parties for the production of our clinical trial materials. In such cases, we expect to control only certain aspects of their activities.

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

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

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

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

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

All parties involved in the preparation of therapeutics for clinical trial or commercial sale, including our existing contract manufacturer for our product candidates, SAFC Pharma, are subject to extensive regulation.

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

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

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

Additionally, if supply from an approved manufacturer is interrupted, there could be a significant disruption in commercial supply of our products. We do not currently have a backup manufacturer of our product candidate supply for clinical trials or commercial sale. An alternative manufacturer would need to be qualified through a BLA supplement which could result in further delay. The regulatory agencies may also require additional trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

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

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

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

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

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

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

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

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

We entered into a collaboration with Genzyme relating to a wet AMD product candidate, which subsequently was modified to take the form of a license to Genzyme. Under our modified relationship, Genzyme became responsible for all future clinical and commercial development of the licensed wet AMD product candidate. Genzyme recently informed us that it no longer intends to use our HSV-based manufacturing technology to produce the AAV vector being used for the wet AMD product. Our license agreement with Genzyme was further amended in December 2013 to reflect this fact. We do not currently expect to derive substantial revenue from our license arrangement with Genzyme, but an unsuccessful outcome in pending and future clinical trials for which Genzyme is responsible could be harmful to the public perception and prospects of our gene therapy platform. Our license relationship with Genzyme, and any future collaboration we enter into in the future, may pose a number of risks, including the following:

collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;

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collaborators may not perform their obligations as expected;

collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;

collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;

collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;

product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;

a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;

disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development of any product candidates, might cause delays or termination of the research, development or commercialization of such product candidates, might lead to additional responsibilities for us with respect to such product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;

collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;

collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and

collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

If our collaborations do not result in the successful development and commercialization of products or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of our gene therapy platform and product candidates could be delayed and we may need additional resources to develop product candidates and gene therapy platform. All of the risks relating to product development, regulatory approval and commercialization described in this prospectus also apply to the activities of our therapeutic program collaborators, if any.

Our license to Genzyme contains a restriction on our engaging in activities that are the subject of that collaboration. However, as a result of the December 2013 amendment of our agreement with Genzyme, these restrictions no longer apply to the field of treatments for ocular neovascularization disorders, including AMD. In addition, under that collaboration agreement, Genzyme has options, which expire in 2015 and 2017, to license our manufacturing technology as it existed at the time of the license for specified genes implicated in diseases outside our current area of focus. These restrictions, and any similar restrictions contained in future collaborations, may have the effect of preventing us

from undertaking development and other efforts that may appear to be attractive to us.

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

We may in the future determine to collaborate with pharmaceutical and biotechnology companies for development and potential commercialization of our product candidates. These relationships or those like them may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement will depend, among other things, upon our assessment of the collaborator s resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator s evaluation of a number of factors. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for our product candidates because our research and development pipeline may be insufficient, our product candidates may be deemed to be at too early of a stage of development for collaborative effort and third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy. If we license product candidates, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. We cannot be certain that, following a strategic transaction or license, we will achieve the revenues or specific net income that justifies such transaction.

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

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

Because we rely on third parties to manufacture our viral vectors and our product candidates, and because we collaborate with various organizations and academic institutions on the advancement of our gene therapy platform, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor s discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our collaborators, advisors, employees and consultants to publish data potentially relating to our trade secrets. Our academic collaborators typically have rights to publish data, provided that we are notified in advance and may delay publication for a specified time in order to secure our intellectual property rights arising from the collaboration. In other cases, publication rights are controlled exclusively by us, although in some cases we may share these rights with other parties. We also

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conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets in cases where we do not have proprietary or otherwise protected rights at the time of publication. A competitor s discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

Risks related to commercialization of our product candidates

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

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

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

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

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

We believe a number of companies are working on AAV-based gene therapy technology, including Genzyme and its parent company Sanofi S.A., BioMarin Pharmaceutical Inc., uniQure B.V., Celladon Corp., Audentes Therapeutics, GenSight Biologics, ReGenX Biosciences, LLC, or ReGenX, Avalanche Biotechnologies, Inc., Spark Therapeutics, LLC, or Spark, Voyager Therapeutics, Inc., Dimension Therapeutics, Inc. and Sangamo Biosciences, Inc. We believe that companies developing gene therapies in the field of orphan ophthalmology on which we are currently focused include Genzyme and Spark, whose programs are at the clinical stage, and GenSight, Neurotech Pharmaceuticals, Inc. and ReGenX, as well as two smaller, early-stage

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companies, RetroSense Therapeutics, LLC and Eos Neuroscience, Inc., all of whose programs we believe are in the pre-clinical stage. Other companies could also seek to enter this field.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of treatments and the commercialization of those treatments. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

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

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

a covered benefit under its health plan;

safe, effective and medically necessary;

appropriate for the specific patient;

cost-effective; and

neither experimental nor investigational.

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

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. In the United States, third-party payors, including private and governmental payors, such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. The Medicare and Medicaid programs increasingly are used as models for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs and biologics. Currently, no gene therapy products have been approved for coverage and reimbursement by the Centers for Medicare & Medicaid Services, or CMS, the agency responsible for administering the Medicare program, and it is difficult to predict what CMS will decide with respect to coverage and reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products. Moreover, reimbursement agencies in Europe may be more conservative than CMS. For example,

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a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada, and other countries has and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products 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 abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and 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.

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

Gene therapy remains a novel technology, with no gene therapy product approved to date in the United States and only one gene therapy product approved to date in Europe. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians specializing in the treatment of those diseases that our product candidates target prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments they are already familiar with and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, trials using early versions of lentiviral vectors, which integrate with, and thereby alter, the host cell s DNA, have led to several well-publicized adverse events, including reported cases of leukemia. Although none of our current product candidates utilize lentiviral vectors, our product candidates use a viral delivery system. Adverse events in our clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

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

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or PPACA, was passed, which substantially changes the way health care is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The PPACA, among other things, subjects biologic products to potential competition by

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lower-cost biosimilars, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations, establishes annual fees and taxes on manufacturers of certain branded prescription drugs, subjects additional drugs to lower pricing under the 340B drug pricing program by adding new entities to the program and establishes 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 manufacturer s outpatient drugs to be covered under Medicare Part D.

In addition, other legislative changes have been proposed and adopted in the United States since the PPACA was enacted. On August 2, 2011, the Budget Control Act of 2011 among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers up to 2% per fiscal year, which went into effect on April 1, 2013. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

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

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

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

the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;

the strength of marketing and distribution support;

the timing of market introduction of competitive products;

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publicity concerning our products or competing products and treatments; and

sufficient third-party insurance coverage and reimbursement.

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

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

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

different regulatory requirements for approval of drugs and biologics in foreign countries;

the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;

challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;

unexpected changes in tariffs, trade barriers and regulatory requirements;

economic weakness, including inflation, or political instability in particular foreign economies and markets;

compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;

foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;

difficulties staffing and managing foreign operations;

workforce uncertainty in countries where labor unrest is more common than in the United States;

potential liability under the Foreign Corrupt Practices Act of 1977 or comparable foreign regulations;

production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and

business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

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

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

The success of our business depends primarily upon our ability to identify, develop and commercialize product candidates based on our gene therapy platform. Although certain of our product candidates are currently in clinical or preclinical development, we may fail to identify other potential product candidates for clinical development for a number of reasons. For example, our research methodology may be unsuccessful in

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identifying potential product candidates or our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

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

Risks related to our business operations

We 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 and corporate governance practices.

As a public company, and 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. We will be subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, which will require, among other things, that we file with the Securities and Exchange Commission, or the SEC, annual, quarterly and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the NASDAQ Global Market and other applicable securities rules and regulations impose 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. In particular, we will or may be required to:

- prepare and distribute periodic public reports in compliance with our obligations under the federal securities laws;
- establish an investor relations function;
- establish new internal policies, such as those relating to disclosure controls and procedures and insider trading;
- expand the roles and duties of our board of directors, our board committees and management;
- institute a more comprehensive financial reporting and disclosure compliance function;
- hire additional financial and accounting personnel and other experienced accounting and finance staff with the expertise to address the complex accounting matters applicable to public companies; and
- establish an internal audit function.

We may not be successful in complying with these obligations, and compliance with these obligations could be time-consuming and expensive. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, which in turn could make it more difficult for us to attract and retain qualified members of our board of directors. The increased costs will decrease our net income or increase our consolidated net loss, and may require us to reduce costs in other areas of our business or increase the prices of our products or services.

We are evaluating these rules and regulations, and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their

application in practice may evolve over time

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as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we will be required to furnish a report by our management on our internal control over financial reporting. 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.

We have identified material weaknesses in our internal control over financial reporting, and if we are unable to achieve and maintain effective internal control over financial reporting, investors could lose confidence in our financial statements and our company which could have a material adverse effect on our business and our stock price.

In the course of preparing the financial statements that are included in this prospectus, our management has determined that we have material weaknesses in our internal control over financial reporting, which relate to the design and operation of our closing and financial reporting processes and our accounting for debt, equity and convertible instruments. We have concluded that these material weaknesses in our internal control over financial reporting are due to the fact that we do not have the appropriate resources with the appropriate level of experience and technical expertise to oversee our closing and financial reporting processes and to address the accounting and financial reporting requirements related to our issuances of convertible notes, preferred stock warrants, stock options, preferred stock and preferred stock purchase rights.

In order to remediate these material weaknesses, we are taking the following actions:

- we have hired two additional accounting and finance staff members, including a permanent chief financial officer;
- we are actively seeking additional accounting and finance staff members, including a senior accounting officer with public company reporting experience, to augment our current staff and to improve the effectiveness of our closing and financial reporting processes; and
- we are formalizing our accounting policies and internal controls documentation and strengthening supervisory reviews by our management.

If we fail to fully remediate these material weaknesses or fail to maintain effective internal controls in the future, it could result in a material misstatement of our financial statements that would not be prevented or detected on a timely basis, which could cause investors to lose confidence in our financial information or cause our stock price to decline. Our independent registered public accounting firm has not assessed the effectiveness of our internal control over financial reporting and, under the JOBS Act, will not be required to provide an attestation report on the effectiveness of our internal control over financial reporting so long as we qualify as an emerging growth company, which may increase the risk that weaknesses or deficiencies in our internal control over financial reporting go undetected.

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

If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial and other systems and resources to manage our operations, continue our research and development activities, and, in the longer term, build a sales force and commercial infrastructure to support commercialization

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of any of our product candidates that are approved for sale. Future growth would impose significant added responsibilities on members of management. It is possible that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and products requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

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

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

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

Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us. The loss of the services of any of our executive officers or other key employees and our inability to find suitable replacements could potentially harm our business, prospects, financial condition or results of operations. We do not maintain key man insurance policies on the lives of these individuals or any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level, and senior managers as well as junior, mid-level, and senior scientific and medical personnel.

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

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

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

- the laws and regulations of the FDA and non-U.S. regulators, including those laws requiring the reporting of true, complete and accurate information to such regulatory bodies;

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manufacturing standards we have established;

healthcare fraud and abuse laws and regulations in the United States and similar foreign laws; or

laws requiring the accurate reporting of financial information or data or the disclosure of unauthorized activities to us.

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

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

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations may be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws, including, without limitation, the federal Health Care Program Anti-Kickback Statute, the federal civil and criminal False Claims Acts and Physician Payments Sunshine Act and regulations. These laws may impact, among other things, our proposed sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include, but are not limited to:

the federal Health Care Program Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind in return for, the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;

federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other government payers that are false or fraudulent;

the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act; Other Modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and health care providers;

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federal transparency laws, including the federal Physician Payment Sunshine Act that requires disclosure of payments and other transfers of value provided to physicians and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations;

the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the Affordable Care Act, and its implementing regulations, which may impact, among other things, reimbursement rates by federal health care programs and commercial insurers; and

state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the industry s voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government, or otherwise restrict certain payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states.

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

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

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

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

impairment of our business reputation;

withdrawal of clinical trial participants;

initiation of investigations by regulators;

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costs due to related litigation;

distraction of management s attention from our primary business;

substantial monetary awards to trial participants, patients or other claimants;

loss of revenue;

exhaustion of any available insurance and our capital resources;

the inability to commercialize our product candidates; and

decreased demand for our product candidates, if approved for commercial sale.

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop. While we believe our product liability insurance coverage is sufficient in light of our current clinical programs, The amount of the product liability coverage that we carry varies from time to time, depending on a number of factors, the most significant of which are the nature and scope of the clinical trials in which we are engaged and the number of patients being treated with our product candidates in these trials. The amount of our product liability coverage as of December 31, 2013 was $10.0 million. This amount may increase or decrease in the future. We may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability and any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. If and when we obtain marketing approval for product candidates, we intend to expand our insurance coverage to include the commercial sale of our products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business.

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

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

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

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

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

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

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

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

Our product candidates are manufactured using technically complex processes requiring specialized facilities, highly specific raw materials and other production constraints. The complexity of these processes, as well as strict company and government standards for the manufacture and storage of our products, subjects us to production risks. While product batches released for use in clinical trials or for commercialization undergo sample testing, some defects may only be identified following product release. In addition, process deviations or unanticipated effects of approved process changes may result in these intermediate products not complying with stability requirements or specifications. Most of our product candidates must be stored and transported at temperatures within a certain range. If these environmental conditions deviate, our product candidates remaining shelf-lives could be impaired or their efficacy and safety could become adversely affected, making them no longer suitable for use. The occurrence or suspected occurrence of production and distribution difficulties can lead to lost inventories, and in some cases product recalls, with consequential reputational damage and the risk of product liability. The investigation and remediation of any identified problems can cause production delays, substantial expense, lost sales and delays of new product launches. Any interruption in the supply of finished products or the loss thereof could hinder our ability to timely distribute our products and satisfy customer

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demand. Any unforeseen failure in the storage of the product or loss in supply could delay our clinical trials and, if our product candidates are approved, result in a loss of our market share and negatively affect our revenues and operations.

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

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

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

Under Section 382 of the Internal Revenue Code of 1986, as amended, substantial changes in our ownership may limit the amount of net operating loss carryforwards that could be utilized annually in the future to offset our taxable income. Specifically, this limitation may arise in the event of a cumulative change in ownership of our company of more than 50% within a three-year period. Any such annual limitation may significantly reduce the utilization of our net operating loss carryforwards before they expire. The closing of this offering, alone or together with transactions in our stock that have occurred in the past and may occur in the future, may trigger an ownership change pursuant to Section 382, which could limit the amount of net operating loss carryforwards that could be utilized annually in the future to offset our taxable income, if any. Any such limitation, whether as the result of this offering, sales of common stock by our existing stockholders or additional sales of common stock by us after this offering, could potentially result in increased tax liability in future years. We have not completed a study to assess whether an ownership change has occurred, or whether there have been multiple ownership changes since our inception, due to the significant costs and complexities associated with such study. However, we believe it is likely that transactions that have occurred in the past, alone or together with the closing of this offering and other transactions that may occur in the future, would trigger an ownership change pursuant to Section 382, which could limit the amount of net operating loss carryforwards that could be utilized annually in the future to offset our taxable income, if any.

Risks related to our intellectual property

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

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

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too

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late to obtain patent protection. Moreover, in some circumstances, we do not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

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

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

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

In addition, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions and *inter partes* reexamination proceedings before the

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United States Patent and Trademark Office and corresponding foreign patent offices. Numerous United States and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire.

Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, methods for manufacture or methods of use, including combination therapy, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

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

Presently we have rights to the intellectual property to develop our gene therapy product candidates. Because our programs may involve additional product candidates that may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights. In addition, our product candidates may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

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

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In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to required third-party intellectual property rights, our business, financial condition and prospects for growth could suffer.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues and is complicated by the rapid pace of scientific discovery in our industry. We are a party to intellectual property license agreements with the University of Florida Research Foundation, an affiliate of the University of Florida, Johns Hopkins University and the UAB Research Foundation, an affiliate of The University of Alabama at Birmingham, each of which is important to our business, and we expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license.

We may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we have done so from time to time. It is possible that we may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current product candidates or future products, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

In many cases, patent prosecution of our licensed technology is controlled solely by the licensor. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. In certain cases, we control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

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

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

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

Competitors may infringe our patents or other intellectual property or the patents or other intellectual property of our licensors. In response, we may be required to file infringement claims, which can be expensive and time-consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us, alleging that we infringe their patents. In addition, in a patent infringement proceeding, a court may decide that a patent of ours or our licensors is invalid or unenforceable, in whole or in part, construe the patent s claims narrowly or refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

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

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

If we or one of our licensing partners initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the United States Patent and Trademark Office, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions. Such proceedings could result in the revocation of or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of the patent protection on one or more of our product candidates. Such a loss of patent protection could have a material adverse impact on our business.

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

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

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

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

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

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

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

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the United States Patent and Trademark Office and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We rely on our outside counsel to pay these fees due to non-U.S. patent agencies. The United States Patent and Trademark Office and various non-U.S. governmental patent agencies require compliance with a number of

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procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could have a material adverse effect on our business.

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

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

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

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

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

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

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

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States,

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or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Risks related to this offering and ownership of our common stock

There has been no public market for our common stock prior to this offering, and you may not be able to resell our shares at or above the price you paid, or at all.

Prior to this offering, there has been no public market for our common stock. We have applied to list our common stock on the NASDAQ Global Market, but an active trading market for our common stock may never develop or be sustained following this offering. If an active trading market for our common stock does not develop after this offering, the market price and liquidity of our common stock will be materially and adversely affected. You may not be able to sell your shares quickly or at the market price if trading in our common shares is not active. The offering price for our common stock will be determined by negotiations between us and the underwriters and may bear no relationship to the market price for our common stock after this offering. An active trading market for our common stock may not develop and the market price of our common stock may decline below the offering price. Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic partnerships or acquire companies or products by using our shares of common stock as consideration.

The market price for our common stock may be volatile, which could contribute to the loss of your investment.

Fluctuations in the price of our common stock could contribute to the loss of all or part of your investment. Prior to this offering, there has not been a public market for our common stock. Accordingly, the initial public offering price for the shares of our common stock may not be indicative of the price that will prevail in the trading market, if any, that develops following this offering. If an active market for our common stock develops and continues, the trading price of our common stock following this offering is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. Any of the factors listed below could have a material adverse effect on your investment in our common stock and our common stock may trade at prices significantly below the initial public offering price. In such circumstances the trading price of our common stock may not recover and may experience a further decline.

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

- our failure to develop and commercialize our product candidates;

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actual or anticipated fluctuations in our quarterly financial results or the quarterly financial results of companies perceived to be similar to us;

changes in the market s expectations about our operating results;

adverse results or delays in preclinical studies or clinical trials;

our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;

adverse regulatory decisions, including failure to receive regulatory approval for any of our product candidates;

success of competitive products;

adverse developments concerning our collaborations and our manufacturers;

inability to obtain adequate product supply for any product candidate for clinical trials or commercial sale or inability to do so at acceptable prices;

the termination of a collaboration or the inability to establish additional collaborations;

unanticipated serious safety concerns related to the use of any of our product candidates;

our ability to effectively manage our growth;

the size and growth, if any, of the orphan ophthalmology and other targeted markets;

our operating results failing to meet the expectation of securities analysts or investors in a particular period or failure of securities analysts to publish reports about us or our business;

changes in financial estimates and recommendations by securities analysts concerning our company, the gene therapy market, or the biotechnology and pharmaceutical industries in general;

operating and stock price performance of other companies that investors deem comparable to us;

overall performance of the equity markets;

announcements by us or our competitors of acquisitions, new product candidates or programs, significant contracts, commercial relationships or capital commitments;

our ability to successfully market our product candidates;

changes in laws and regulations affecting our business, including but not limited to clinical trial requirements for approvals;

disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our product candidates and gene therapy platform;

commencement of, or involvement in, litigation involving our company, our general industry, or both;

changes in our capital structure, such as future issuances of securities or the incurrence of additional debt;

the volume of shares of our common stock available for public sale;

additions or departures of key scientific or management personnel;

any major change in our board or management;

changes in accounting practices;

ineffectiveness of our internal control over financial reporting;

sales of substantial amounts of common stock by our directors, executive officers or significant stockholders or the perception that such sales could occur; and

general economic and political conditions such as recessions, interest rates, fuel prices, international currency fluctuations and acts of war or terrorism.

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

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

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

The concentration of our capital stock ownership with insiders upon the closing of this offering will likely limit your ability to influence corporate matters.

We anticipate that our executive officers, employees, directors, current 5% or greater stockholders, and their respective affiliates will together beneficially own or control, in aggregate, approximately 71% of the shares of our outstanding common stock, after giving effect to the conversion of all outstanding preferred stock and assuming no exercise of outstanding options or warrants following the closing of this offering (assuming no exercise of the underwriters over-allotment option). As a result, these executive officers, directors and principal stockholders, acting together, will have substantial influence over most matters that require approval by our stockholders, including the election of directors, any merger, consolidation or sale of all or substantially all or of our assets or any other significant corporate transaction. Corporate action might be taken even if other stockholders, including those who purchase shares in this offering, oppose such action. These stockholders may delay or prevent a change of control or otherwise discourage a potential acquirer from attempting to obtain control of our company, even if such change of control would benefit our other stockholders. This concentration of stock ownership may adversely affect investors perception of our corporate governance or delay, prevent or cause a change in control of our company, any of which could adversely affect the market price of our common stock. Certain of our existing stockholders have indicated an interest in purchasing up to an aggregate of approximately $10.0 million in shares of our common stock in this offering at the initial public offering price. The previously discussed ownership percentage upon completion of this offering does not reflect the potential purchase of any shares in this offering by such stockholders. If these stockholders purchase an aggregate of $10.0 million in shares of our common stock in this offering at $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus), upon completion of this offering, our executive officers, employees, directors, 5% stockholders, and their respective affiliates will together beneficially own approximately 77% of our outstanding common stock (assuming no exercise of the underwriters over-allotment option).

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

We are an emerging growth company, as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or the

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Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds $700.0 million as of any December 31 before that time or if we have total annual gross revenue of $1.0 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following June 30 or, if we issue more than $1.0 billion in non-convertible debt during any three-year period before that time, we would cease to be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a smaller reporting company which would allow us to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, changes in rules of U.S. generally accepted accounting principles or their interpretation, the adoption of new guidance or the application of existing guidance to changes in our business could significantly affect our financial position and results of operations.

Sales of a substantial number of shares of our common stock by our existing stockholders in the public market may cause our stock price to decline.

Sales of our common stock in the public market after this offering, or the perception that these sales may occur, could cause the market price of our common stock to decline. Based on our shares of common stock outstanding as of December 31, 2013, upon the closing of this offering, we will have 12,800,120 shares of common stock outstanding, assuming no exercise of the underwriters over-allotment option. Of these, only the 3,571,000 shares of our common stock sold in this offering, plus any shares sold upon exercise of the underwriters over-allotment option, will be freely transferable without restriction or additional registration under the Securities Act of 1933, as amended, or the Securities Act. The remaining shares outstanding after this offering will be available for sale, upon the expiration of the 180-day lock-up period beginning from the date of this prospectus, if applicable, subject to volume and other restrictions as applicable under Rule 144 under the Securities Act. Any or all of these shares may be released prior to expiration of the lock-up period at the discretion of the lead underwriter for this offering. After the lock-up agreements expire, up to an additional 9,229,120 shares of common stock will be eligible for sale in the public market, 8,536,272 of which shares are held by directors, executive officers, 5% stockholders and other affiliates and will be subject to volume limitations under Rule 144 under the Securities Act, assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus). In addition, 59,247 shares of common stock issuable upon exercise of outstanding preferred stock warrants and 2,313,150 shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. To the extent these shares are sold, or if it is perceived that they will be sold, into the market, the market price of our common stock could decline. See Shares Eligible for Future Sale for a more detailed description of the restrictions applicable to the sale of shares of our common stock after this offering.

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

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

You will experience immediate and substantial dilution in the net tangible book value of the shares you purchase in this offering.

If you purchase shares of our common stock in this offering, you will experience immediate and substantial dilution, as the initial public offering price of our common stock will be substantially greater than the net tangible book value per share of our common stock. Based on an assumed initial offering price of $14.00 per share, which is the midpoint of the price range on the cover page of this prospectus, if you purchase our common stock in this offering, you will suffer immediate and substantial dilution of approximately $8.27 per share. Further, investors purchasing common stock in this offering will contribute approximately 38.1% of the total amount invested by stockholders since our inception, but will own only approximately 27.9% of the shares of common stock outstanding after giving effect to this offering. If the underwriters exercise their over-allotment option, or if outstanding options and warrants to purchase our common stock are exercised, you will experience additional dilution. For a further description of the dilution that you will experience immediately after this offering, see the section entitled Dilution.

Our board of directors and 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 board of directors and 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 board of directors and 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. While we have not allocated these estimated net proceeds for any specific purposes, we expect to use the net proceeds from this offering to develop our product candidates and for general corporate purposes, including working capital. We may also use a portion of the proceeds to repay outstanding indebtedness or in acquisitions of businesses, products and technologies that are complementary to our business. Although we have from time to time evaluated possible acquisitions, we currently have no commitments or agreements to make any material acquisition, and we may not make any acquisitions in the future. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in short-term, investment-grade, interest-bearing securities. 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.

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

We have never declared or paid any cash dividends on our common stock. We currently intend to retain any future earnings to fund our future growth and do not expect to declare or pay any dividend on shares of our

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common stock in the foreseeable future. As a result, you may only receive a return on your investment in our common stock if the market price of our common stock appreciates and you sell your shares at a price above your cost. The price of our common stock may not appreciate in value or ever exceed the price that you paid for shares of our common stock in this offering.

We could be subject to securities class action litigation.

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

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

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

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

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

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

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

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Exchange Act. In some cases, you can identify these statements by forward-looking words such as may, could, should, would, intend, will, expect, anticipate, believe, estimate, continue, plan, potential predict, project or the negative of thos Any statements contained herein that are not statements of historical facts may be deemed to be forward-looking statements. You should read these statements carefully because they discuss our future expectations, contain projections of our future results of operations or of our financial condition or state other forward-looking information. These forward-looking statements include, among other things, statements about:

- the anticipated timing, costs and conduct of our planned clinical trials for our ACHM and XLRS product candidates;
- the anticipated timing, costs and conduct of our planned preclinical studies of our XLRP product candidate;
- our plans to explore potential applications of our gene therapy platform in other indications in orphan ophthalmology;
- our expectations regarding the clinical effectiveness of our product candidates;
- our beliefs regarding the scalability and commercial viability of our HAVE manufacturing method;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position;
- our competitive position;
- our expectations related to the use of proceeds from this offering; and
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing.

These forward-looking statements reflect our management s beliefs and views with respect to future events and are based on estimates and assumptions as of the date of this prospectus and are subject to risks and uncertainties. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this prospectus, particularly in the Risk Factors section, that could cause actual results or events to differ materially from the forward-looking statements that we make. Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments that we may make.

You should read this prospectus, the documents that we reference in this prospectus and the documents that we have filed as exhibits to the registration statement of which this prospectus is a part completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

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USE OF PROCEEDS

We estimate that the net proceeds from our issuance and sale of 3,571,000 shares of our common stock in this offering will be approximately $44.8 million, assuming an initial public offering price of $14.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise their over-allotment option in full, we estimate that the net proceeds from this offering will be approximately $51.8 million, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

A $1.00 increase or decrease in the assumed initial public offering price of $14.00 per share would increase or decrease the net proceeds from this offering by approximately $3.3 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, a one million share increase (decrease) in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) the net proceeds to us by approximately $13.0 million, assuming an initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus) remains the same, and after deducting estimated underwriting discounts and commissions.

We expect to use the net proceeds from this offering, together with cash and cash equivalents on hand, to continue the clinical trials of our XLRS and ACHM product candidates beyond the Phase 1/2 stages (which we believe are already adequately funded), expand preclinical studies of our XLRP product candidate, and explore potential applications of our gene therapy platform in other indications in orphan ophthalmology.

Specifically, we intend to apply the net proceeds of this offering as follows:

- approximately $10.6 million to fund a Phase 1/2 clinical trial and, if that is successful, to initiate a pivotal Phase 3 trial of our XLRS product candidate;
- approximately $17.9 million to fund a Phase 1/2 clinical trial and, if that is successful, to initiate a pivotal Phase 3 trial of our ACHM product candidate;
- approximately $5.0 million to fund additional preclinical studies of our XLRP product candidate;
- approximately $2.3 million to explore, through early preclinical studies, potential applications of our gene therapy platform in other indications in orphan ophthalmology; and
- the remainder for working capital and other general corporate purposes.

We may also use a portion of the net proceeds to in-license, acquire or invest in complementary businesses, technologies, products or assets, though we have no present plans to make any such acquisition or investment. Pending the use of the net proceeds from this offering, we intend to invest the net proceeds in short-term, interest-bearing, investment-grade securities, certificates of deposit or government securities.

Our expected use of net proceeds from this offering represents our current intentions based upon our present plans and business condition. As of the date of this prospectus, we cannot predict with certainty all of the particular uses for the net proceeds to be received upon the closing of this offering or the amounts that we will actually spend on the uses set forth above. Due to the many variables inherent in the development of gene therapy products at this time, such as the timing of patient enrollment, the timing and results of preclinical animal studies and clinical trials and the timing of regulatory submissions and evolving regulatory requirements, the amount and timing of our actual expenditures will depend upon such variables and we cannot currently predict the stage of development we expect the net proceeds of this offering to achieve for our clinical studies and product candidates.

As a result, we will have broad discretion over the use of the net proceeds from this offering, and investors will be relying on our judgment regarding the application of the net proceeds of this offering. In addition, we might decide to postpone or not pursue certain clinical trials or preclinical activities if the net proceeds from this offering and the other sources of cash are less than expected.

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DIVIDEND POLICY

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and future earnings, if any, to finance the growth and development of our business. We do not expect to pay any cash dividends on our common stock in the foreseeable future. Payment of future dividends, if any, will be at the discretion of our board of directors and will depend on our financial condition, results of operations, capital requirements, restrictions contained in any future financing instruments, provisions of applicable law and other factors the board deems relevant. See Risk Factors Risks related to this offering and ownership of our common stock We do not currently intend to pay dividends on our common stock and, consequently, your ability to achieve a return on your investment will depend on the appreciation in the price of our common stock.

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CAPITALIZATION

The following table sets forth our cash and cash equivalents and capitalization as of December 31, 2013 on:

An actual basis;

A pro forma basis, giving effect to the conversion of all of our preferred stock into 9,120,081 shares of common stock upon the closing of this offering and the conversion of all outstanding warrants exercisable for shares of Series A-1, Series A-1A and Series B-1 preferred stock into warrants exercisable for shares of common stock, resulting in the preferred stock warrant liability being reclassified to additional paid-in capital, each upon the closing of this offering; and

A pro forma as adjusted basis, giving additional effect to the sale of 3,571,000 shares of our common stock offered in this offering, assuming an initial public offering price of $14.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us and the filing and effectiveness of a restated certificate of incorporation upon the closing of this offering.

The pro forma information below is illustrative only and our capitalization following the closing of this offering will be adjusted based on the actual initial public offering price and other terms of this offering determined at pricing. You should read the following table in conjunction with our financial statements and related notes, Selected Financial Data and Management s Discussion and Analysis of Financial Condition and Results of Operations appearing elsewhere in this prospectus.

	As of December 31, 2013		
	Actual	Pro Forma	Pro Forma as Adjusted
		(in thousands, except share and per share data)	
Cash and cash equivalents	$ 8,998	$ 8,998	$ 53,842
Short-term investments	$ 19,000	$ 19,000	$ 19,000
Convertible preferred stock and stockholders equity:			
Convertible preferred stock, $0.001 par value; Series A-1 to B-3; shares issued and outstanding: 281,660,161 actual; none pro forma or pro forma as adjusted	73,721		
Common stock, $0.001 par value; 410,000,000 shares authorized; shares issued: 109,039 actual; 9,229,120 pro forma; 12,800,120 pro forma as adjusted		9	13
Additional paid-in capital	12,354	86,281	131,121
Accumulated deficit	(56,225)	(56,225)	(56,225)
Total stockholders (deficit) equity	$ (43,871)	$ 30,065	$ 74,909
Total capitalization	$ (43,871)	$ 30,065	$ 74,909

A $1.00 increase (decrease) in the assumed initial public offering price of $14.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents and total stockholders (deficit) equity by approximately $3.3 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 estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, a one million share increase (decrease) in the number of shares offered by us, as set forth on the cover page of this

prospectus, would increase (decrease) each of cash and

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cash equivalents and total stockholders equity (deficit) and total capitalization by approximately $13.0 million, assuming the initial public offering price of $14.00 per share (the midpoint of the price range set forth on the cover page of this prospectus) remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

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DILUTION

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

Our pro forma historical net tangible book value (deficit) as of December 31, 2013 was $28.5 million, or $3.08 per share of common stock, taking into account the expected conversion of all shares of our preferred stock outstanding as of that date into 9,120,081 shares of common stock and the conversion of all outstanding warrants exercisable for shares of Series A-1, Series A-1A and Series B-1 preferred stock into warrants exercisable for shares of common stock, resulting in the preferred stock warrant liability being reclassified to additional paid-in capital, each upon closing of this offering. Without giving effect to the conversion of our outstanding preferred stock into common stock and the conversion of our outstanding warrants exercisable for preferred stock into warrants exercisable for common stock, we had a historical net tangible book value (deficit) of $(45.5) million, or $(417.04) per share of common stock, as of December 31, 2013. Historical net tangible book value per share is equal to our total tangible assets, less total liabilities and preferred stock, divided by the number of outstanding shares of our common stock.

After giving effect to our issuance and sale of 3,571,000 shares of common stock in this offering at an assumed initial public offering price of $14.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, the pro forma as adjusted net tangible book value (deficit) as of December 31, 2013 would have been $73.3 million, or $5.73 per share. This represents an immediate increase in pro forma net tangible book value to existing stockholders of $2.65 per share. The initial public offering price per share will significantly exceed the pro forma as adjusted net tangible book value per share. Accordingly, new investors who purchase shares of common stock in this offering will suffer an immediate dilution of their investment of $8.27 per share. The following table illustrates this per share dilution to the new investors purchasing shares of common stock in this offering without giving effect to the over-allotment option granted to the underwriters:

Assumed initial public offering price		$ 14.00
Historical net tangible book value (deficit) per share	$ (417.04)	
Increase per share attributable to conversion of outstanding preferred stock and preferred stock warrants	$ 420.13	
Pro forma historical net tangible book value (deficit) per share as of December 31, 2013	$ 3.08	
Increase per share attributable to sale of shares of common stock in this offering	$ 2.65	
Pro forma as adjusted historical net tangible book value per share		$ 5.73
Dilution per share to new investors		$ 8.27

Each $1.00 increase or decrease in the assumed initial public offering price of $14.00 per share would increase or decrease the pro forma net tangible book value (deficit) by $3.3 million, the pro forma net tangible book value (deficit) per share after this offering by $0.26 per share and the dilution in pro forma net tangible book value (deficit) per share to investors in this offering by $0.74 per share, 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 discount and offering expenses payable by us.

Similarly, a one million share increase in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase the pro forma as adjusted net tangible book value per share after this offering by approximately $0.53 and decrease the dilution per share to investors participating in this offering by approximately $0.53, assuming the assumed initial public offering price of $14.00 per share remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. A one million share decrease in the number of shares offered by us, as set forth on the cover page of this prospectus, would decrease the pro forma as adjusted net tangible book value per share after this offering by approximately $0.62 and increase the dilution per share to investors participating in this offering by approximately $0.62, assuming the

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assumed initial public offering price of $14.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

If the underwriters exercise their over-allotment option in full, the pro forma as adjusted net tangible book value (deficit) will increase to $6.02 per share, representing an immediate increase to existing stockholders of $2.94 per share and an immediate dilution of $7.98 per share to new investors. If any shares are issued upon exercise of outstanding options or warrants, you will experience further dilution.

The following table summarizes, on a pro forma as adjusted basis as of December 31, 2013, after giving effect to the conversion of all of our outstanding preferred stock into common stock, the differences between the number of shares of common stock purchased from us, the total consideration paid to us and the average price per share paid by existing stockholders and by new investors purchasing shares of common stock in this offering. The calculation below is based on an assumed initial public offering price of $14.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, before the deduction of the estimated underwriting discounts and commissions and estimated offering expenses payable by us:

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	%	Amount	%	
Existing stockholders	9,229,120	72.1%	$ 81,164,054	61.9%	$ 8.79
New investors	3,571,000	27.9	49,994,000	38.1	$ 14.00
Total	12,800,120	100%	$ 131,158,054	100%	

The number of shares purchased from us by existing stockholders is based on 9,229,120 shares of our common stock outstanding as of December 31, 2013, which gives effect to the conversion of all outstanding shares of our preferred stock into 9,120,081 shares of common stock upon the closing of this offering, and excludes:

- 59,247 shares of common stock issuable upon the exercise of preferred stock warrants outstanding and exercisable as of December 31, 2013, at a weighted average exercise price of $8.16 per share;
- 782,947 shares of common stock issuable upon the exercise of stock options outstanding under our 2001 Stock Option Plan and our 2011 Stock Incentive Plan as of December 31, 2013, at a weighted average exercise price of $3.23 per share;
- 250,204 shares of common stock available for future issuance under our 2001 Stock Option Plan and our 2011 Stock Incentive Plan as of December 31, 2013; and
- an additional 1,279,999 shares of our common stock that will be made available for future issuance under our equity compensation plans upon the closing of this offering.

If the underwriters exercise their option to purchase additional shares from us in full, the number of shares held by new investors will increase to 4,106,650, or 30.8% of the total number of shares of common stock outstanding after this offering and the percentage of shares held by existing stockholders will decrease to 69.2% of the total shares outstanding.

Certain of our existing stockholders have indicated an interest in purchasing up to an aggregate of approximately $10.0 million in shares of our common stock in this offering at the initial public offering price. The foregoing discussion does not reflect the potential purchase of any shares in this offering by these existing stockholders.

In addition, the initial share reserves under our 2013 Equity and Incentive Plan will be subject to automatic annual increases in accordance with the terms of that plan. Furthermore, we may choose to raise additional capital through the sale of equity or convertible debt securities due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. To the extent that any of the currently outstanding options are exercised, new options are issued under our equity incentive plans or we issue additional shares of common stock or other equity or convertible debt securities in the future, there will be further dilution to investors participating in this offering.

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SELECTED FINANCIAL DATA

The following selected financial data should be read together with our financial statements and accompanying notes and Management s Discussion and Analysis of Financial Condition and Results of Operations appearing elsewhere in this prospectus. Our selected statement of operations data for the fiscal years ended June 30, 2012 and 2013 and our selected balance sheet data as of June 30, 2012 and 2013 are derived from our audited financial statements included elsewhere in this prospectus. Our selected statement of operations data for the six months ended December 31, 2012 and 2013 and our selected balance sheet data as of December 31, 2013 have been derived from our unaudited financial statements included elsewhere in this prospectus. Our historical results are not necessarily indicative of results to be expected for any future period, and our interim results are not necessarily indicative of our results for the entire year or any future period. The selected financial data in this section are not intended to replace our financial statements and the related notes.

	Fiscal Year Ended June 30,		Six Months Ended December 31,	
	2012	2013	2012	2013
	(in thousands except per share data)			
Statement of Operations Data:				
Revenue:				
Grant revenue	$ 718	$ 439	$ 293	$ 466
Sponsored research revenue	364	503	156	307
Total revenue	1,082	942	449	773
Operating expenses:				
Research and development	2,354	3,133	1,156	3,673
General and administrative	787	1,403	649	1,971
Total operating expenses	3,141	4,536	1,805	5,644
Loss from operations	(2,059)	(3,594)	(1,356)	(4,871)
Other income (expense):				
Interest income		10		15
Interest expense	(69)	(191)	(152)	
Fair value adjustments to warrant liabilities (1)	204	(8)	5	(105)
Fair value adjustments to Series B purchase rights (1)		(1,207)	(437)	(2,838)
Total other income (expense), net	135	(1,396)	(584)	(2,928)
Net loss	$ (1,924)	$ (4,990)	$ (1,940)	$ (7,799)
Net loss per share, basic and diluted (2)	$ (17.65)	$ (45.78)	$ (17.80)	$ (71.55)
Weighted-average shares outstanding, basic and diluted (2)	109	109	109	109
Pro forma net loss per share, basic and diluted (unaudited) (2)		$ (1.20)		$ (1.13)
Weighted-average pro forma shares outstanding, basic and diluted (unaudited) (2)		4,146		6,885

	As of June 30,		As of December 31,
	2012	2013	2013
	(in thousands)		
Balance Sheet Data:			
Cash and cash equivalents	$ 774	$ 8,893	$ 8,988
Short-term investments	$	$ 14,000	$ 19,000
Working capital	$ (399)	$ 20,051	$ 28,106

Total assets	$ 2,824	$ 25,490	$ 31,709
Current liabilities	$ 1,494	$ 3,460	$ 1,644
Total stockholders (deficit) equity	$ (31,290)	$ (36,183)	$ (43,871)

(1) See note 6 of the notes to financial statements appearing elsewhere in this prospectus for a description of the fair value adjustments to our warrant liabilities and Series B purchase rights.

(2) See note 2 of the notes to financial statements appearing elsewhere in this prospectus for a description of the method used to calculate basic and diluted net loss per share and pro forma basic and diluted net loss per share.

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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 our financial statements and related notes appearing in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the Risk Factors section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. See Cautionary Note Regarding Forward-Looking Statements.

Overview

We are a clinical-stage biotechnology company that uses our proprietary gene therapy platform to develop products designed to transform the lives of patients with severe inherited orphan diseases in ophthalmology. Our lead product candidates, which are each in the preclinical stage, are treatments for X-linked retinoschisis, or XLRS, achromatopsia, or ACHM, and X-linked retinitis pigmentosa, or XLRP. These rare diseases of the eye are caused by mutations in single genes, significantly affect visual function and currently lack effective medical treatments. For our XLRS product candidate, we expect to file an IND and initiate Phase 1/2 clinical trials in the United States in late 2014 with initial clinical data expected in mid-2015. For our ACHM product candidate, we expect to file an IND and initiate Phase 1/2 clinical trials in the United States in early 2015, with clinical data expected in late 2015. We have also begun preclinical studies for our product candidate addressing XLRP, a disease characterized by progressive degeneration of the retina, leading to total blindness in adult men. In the longer term, we will seek opportunities to take advantage of the adaptability of our gene therapy platform to address a range of genetic diseases, both within and beyond our initial focus area of orphan ophthalmology.

Since our inception in 1999, we have devoted substantially all of our resources to our development efforts relating to our proof-of-concept programs in ophthalmology and alpha-1 antitrypsin deficiency, or AAT deficiency, an inherited orphan lung disease, including activities to manufacture product in compliance with good manufacturing practices, preparing to conduct and conducting clinical trials of our product candidates, providing general and administrative support for these operations and protecting our intellectual property. We do not have any products approved for sale and have not generated any revenue from product sales. We have funded our operations primarily through the private placement of preferred stock, common stock, convertible notes and warrants to purchase preferred stock. We have also been awarded grant funding aggregating $10.6 million between our inception and December 31, 2013, either independently or with our collaborators. Most recently, in May 2013, we and the University of Florida, or UF, were jointly awarded an $8.3 million dollar grant from the National Eye Institute, or NEI, of the National Institutes of Health, or NIH, to support development of our ACHM product candidate. As a sub-awardee, as of December 31, 2013, we had received $0.2 million and we expected to receive an additional $3.7 million over four years under this grant.

We have incurred losses from operations in each year since inception. Our net losses were $1.9 million and $5.0 million for the fiscal years ended June 30, 2012 and 2013, respectively. Substantially all our net losses resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. We expect our expenses will increase substantially in connection with our ongoing activities, as we:

- conduct preclinical studies and clinical trials for our XLRS, ACHM and XLRP product candidates;
- continue our research and development efforts, including exploration through early preclinical studies of potential applications of our gene therapy platform in other indications in orphan ophthalmology;
- manufacture clinical trial materials and develop large-scale manufacturing capabilities;

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seek regulatory approval for our product candidates;

further develop our gene therapy platform;

add personnel to support our product development and commercialization efforts; and

operate as a public company.

We do not expect to generate revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years and which we believe is subject to significant uncertainty. We expect that the net proceeds from this offering and our existing cash and cash equivalents will be sufficient to enable us to advance planned preclinical studies and clinical trials for our lead product candidates for at least the next 24 months. In order to complete the process of obtaining regulatory approval for our lead product candidates and to build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize our lead product candidates, if approved, we will require substantial additional funding. Also, our current operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements as and when needed would have a negative impact on our financial condition and our ability to develop our products.

We expect to incur losses for the foreseeable future, and we expect these losses to increase as we continue our development of, and seek regulatory approvals for, our product candidates and begin to commercialize any approved products. Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Our ability to generate revenue from product sales will depend on a number of factors, including, among others, obtaining and maintaining adequate coverage and reimbursement from third-party payors for our product candidates and for gene therapy as a viable treatment option. Even if we are able to generate revenues from the sale of our products, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

Financial operations overview

Revenue

Our ability to generate product revenue and become profitable depends upon our ability to successfully commercialize products. To date, we have not generated any revenues from the sales of products. In the two fiscal years ended June 30, 2012 and 2013, and the six months ended December 31, 2013, all our revenues were derived from grants. Our grant revenue is primarily generated through research and development grant programs offered by federal, state, and local governments and agencies, including the United States Food and Drug Administration, or FDA, and by patient advocacy groups such as the Foundation Fighting Blindness, or FFB, and the Alpha-1 Foundation. Grant revenue is recognized when there is reasonable assurance that the grant will be received and we have complied with the terms of the grant. Prior to fiscal year 2012, we also derived revenue from collaboration and license fees received under our agreement with Genzyme Corporation, or Genzyme. We currently do not expect to derive substantial additional revenue from our agreement with Genzyme.

Research and development expenses

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

employee-related expenses, including salaries, benefits, travel and share-based compensation expense;

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- expenses incurred under agreements with academic research centers, contract research organizations, or CROs, and investigative sites that conduct our clinical trials;
- the cost of acquiring, developing, and manufacturing clinical trial materials; and
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance, and other supplies.

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

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

- the scope, rate of progress, and expense of our ongoing as well as any additional clinical trials and other research and development activities;
- the countries in which trials are conducted;
- future clinical trial results;
- uncertainties in clinical trial enrollment rates or drop-out or discontinuation rates of patients;
- potential additional safety monitoring or other studies requested by regulatory agencies;
- significant and changing government regulation; and
- the timing and receipt of any regulatory approvals.

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

From inception through June 30, 2013, we have incurred approximately $46.4 million in research and development expenses. Our research and development expenses, categorized by product candidate or program, in fiscal years 2012 and 2013 were as follows:

Product candidate or program	Fiscal year ended June 30, 2012	2013

	(in thousands)	
XLRS	$ 633	$ 929
ACHM	151	685
XLRP		
LCA2	265	312
Other orphan ophthalmology indications		70
General research and process development	711	746
AAT deficiency	594	391
Total	$ 2,354	$ 3,133

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We plan to increase our research and development expenses for the foreseeable future as we continue the development of our XLRS, ACHM and XLRP product candidates and explore potential applications of our gene therapy platform in other indications in orphan ophthalmology. Our current planned research and development activities include the following:

- we expect to file an IND and initiate in late 2014 Phase 1/2 clinical trials in the United States to examine the feasibility, safety and efficacy of our XLRS product candidate;
- we expect to file an IND and initiate in early 2015 Phase 1/2 clinical trials in the United States to examine the feasibility, safety and efficacy of our ACHM product candidate;
- we are currently designing preclinical studies to further evaluate the ability of an AAV vector to delay disease progression in animal models of XLRP. If these studies are successful, we will conduct additional preclinical studies required for submission of an IND to the FDA; and
- we will continue to manufacture clinical trial materials in support of our clinical trials.

General and administrative expenses

General and administrative expenses consist primarily of salaries and related costs for personnel, including share-based compensation and travel expenses for our employees in executive, operational, finance and human resource functions. Other general and administrative expenses include facility-related costs and professional fees for directors, accounting and legal services and expenses associated with obtaining and maintaining patents.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research and development and potential commercialization of our product candidates. We also anticipate increased expenses related to audit, legal, regulatory, and tax-related services associated with maintaining compliance with NASDAQ listing and Securities and Exchange Commission requirements, director and officer insurance premiums, and investor relations costs associated with being a public company. Additionally, if and when we believe a regulatory approval of the first product candidate appears likely, we anticipate an increase in payroll and related expenses as a result of our preparation for commercial operations, especially as it relates to the sales and marketing of our product candidates.

Other income (expense), net

Other income and expense consists primarily of interest earned on cash and cash equivalents and short-term investments, interest incurred on our bridge and bank loans, loss on disposal of property and equipment and re-measurement gain or loss associated with the change in the fair value of our Series B purchase rights liability and our preferred stock warrant liability.

We use the Black-Scholes option pricing model to estimate the fair value of our Series B purchase rights liability and preferred stock warrant liability. We base the estimates in the Black-Scholes option pricing model, in part, on subjective assumptions, including stock price volatility, risk-free interest rate, dividend yield, and the fair value of the preferred stock underlying the purchase rights and the warrants. The re-measurement gain or loss associated with the changes in the fair value of our Series B purchase rights liability and preferred stock warrant liability in each reporting period is recognized as a component of other income (expense), net.

Critical accounting policies and significant judgments and estimates

Our management s discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related

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to accrued expenses and share-based compensation. We base our estimates on historical experience, known trends and events, and various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this prospectus, we believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our financial statements.

Revenue recognition

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

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue in our balance sheets. Amounts expected to be recognized as revenue within the twelve months following the balance sheet date are classified as current liabilities. We recognize revenue for reimbursements of research and development costs under collaboration agreements as the services are performed. We record these reimbursements as revenue and not as a reduction of research and development expenses, as we have the risks and rewards as the principal in the research and development activities.

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

Research and development costs and expenses

Research and development costs are charged to expense as incurred. We recognize costs for certain development activities based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and our clinical sites. When outside contracts for research products or testing require advance payments, they are recorded on the balance sheet as a prepaid item and expensed when the service is provided or reaches a specific milestone outlined in the contract.

Share-based compensation

We account for our share-based compensation in accordance with ASC 718, *Compensation Stock Compensation*. ASC 718 establishes accounting for share-based awards exchanged for employee services. Under the fair value recognition provisions of ASC 718, share-based compensation cost is measured at the grant date based on the fair value of the award and is recognized as expense over the requisite service/vesting period. Determining the appropriate fair value model and calculating the fair value of share-based payment awards require the use of highly subjective assumptions, including the expected life of the share-based payment awards and stock price volatility.

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We estimate the grant date fair value of stock options and the related compensation expense using the Black-Scholes option valuation model. This option valuation model requires the input of subjective assumptions including: (1) estimated period of time outstanding, or expected term, of the options granted, (2) volatility, (3) risk-free interest rate and (4) expected dividend yield. Because share-based compensation expense is based on awards ultimately expected to vest, it is reduced for estimated forfeitures. ASC 718 requires forfeitures to be estimated at the time of grant and revised, if necessary, in subsequent periods if actual forfeiture rates differ from those estimates. We have estimated expected forfeitures of stock options based on our historical turnover rate and used these rates in developing a future forfeiture rate. If our actual forfeiture rate varies from our estimates, additional adjustments to compensation expense may be required in future periods. The assumptions used in calculating the fair value of share-based payment awards represent management s best estimates, but the estimates involve inherent uncertainties and the application of management judgment. As a result, if facts change and we use different assumptions, our share-based compensation expense could be materially different in the future.

Exercise price and fair value of common stock

All options have been granted at exercise prices determined by our board of directors to be not less than the fair value of the underlying shares on the date of grant. The fair value of the shares of common stock that underlie the stock options we have granted has historically been estimated by our board of directors based upon information available to it at the time of grant, as further discussed below.

Information pertaining to the Black-Scholes valuation of common stock options granted to employees during fiscal years 2012 and 2013 and the six months ended December 31, 2013 is as follows:

	Fiscal Year Ended June 30,		Six Months Ended December 31,	
	2012	2013	2012	2013
Options granted (number of shares)	3,934	193,066		371,718
Weighted-average exercise price	$ 3.50	$ 0.35		$ 4.90
Weighted-average grant date fair value of common stock options	$ 1.75	$ 0.35		$ 4.90
Assumptions:				
Expected volatility	65.02%	63.23%		60.00%
Expected term in years	6.25	6.25		6.25
Risk-free interest rate	1.39%	1.37% to 1.40%		2.05%
Expected dividend yield	0.00%	0.00%		0.0%

The dividend yield is based upon the assumption that we will not declare a dividend over the life of the options. Since adopting ASC 718, we have been unable to use historical employee exercise and option expiration data to estimate the expected term assumption for the Black-Scholes grant-date valuation. We have therefore utilized the simplified method, as prescribed by the SEC s Staff Accounting Bulletin No. 107, Share-Based Payment, to estimate on a formula basis the expected term of our stock options considered to have plain vanilla characteristics. The risk-free interest rate is based on the U.S. Treasury yield curve on the date of the grant. We compute volatility under the calculated value method of ASC 718 by utilizing the average of a peer group comprised of publicly-traded companies and expect to continue to do so until we have adequate historical data regarding the volatility of our traded stock price. The peer group was determined based upon companies considered to be direct competition or having been presented by independent parties as a comparable company based upon market sector. In determining a comparable, we have excluded large-cap entities. Forfeitures are estimated at the time of the grant and revised, if necessary, in subsequent periods if actual forfeitures differ from those estimates. Share-based compensation expense recognized in the statement of operations for the years ended June 30, 2012 and 2013 and the six months ended December 31, 2012 and 2013 does not record tax related effects on stock-based compensation given our historical and anticipated operating losses and offsetting changes in its valuation allowance that fully reserves against potential deferred tax assets.

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Stock option grants during fiscal years 2012 and 2013

The following table presents the grant dates, number of underlying shares and related exercise prices of all stock options granted to employees between July 1, 2011 and December 31, 2013, along with the fair value per share utilized to calculate share-based compensation expense for each grant:

Date of grant	Number of shares	Exercise price per share	Common stock fair value per share on grant date
August 25, 2011	3,999	$ 3.50	$ 3.50
November 2, 2011	3,934	3.50	3.50
January 6, 2013	192,067	0.35	0.35
April 19, 2013	999	0.35	0.35
September 18, 2013	371,718	4.90	4.90

Share-based compensation totaled $24,445 and $25,237 for fiscal years 2012 and 2013, and $9,981 and $111,247 for the six months ended December 31, 2012 and 2013, respectively. We expect the amount of our share-based compensation expense for stock options granted to employees and non-employees to increase in future periods due to increases in headcount and, potentially, to increases in the value of our common stock.

The intrinsic value of all vested and unvested options outstanding at December 31, 2013 was $8.4 million, representing the difference between the aggregate value of the shares subject to such options, assuming a value per share equal to the midpoint of the price range on the cover page of this prospectus, and the aggregate exercise price of the such options, which have a weighted average exercise price equal to $3.23 per share.

Significant factors used in determining the fair value of our common stock

The fair value of the shares of common stock that underlie the stock options we have granted has historically been determined by our board of directors based upon information available to it at the time of grant. The board of directors considered numerous objective and subjective factors in the assessment of fair value, including reviews of our business and financial condition, the conditions of the industry in which we operate and the markets that we serve and general economic, market and United States and global capital market conditions, the lack of marketability of our common stock, the likelihood of achieving a liquidity event for the shares of common stock underlying these stock options, the preferences and privileges of the preferred stock over the rights of the common stock, the status of the clinical trials and preclinical studies relating to our product candidates and third-party valuations of our common stock. The board has generally considered the most persuasive evidence of fair value to be the prices at which our securities were sold in actual arms length transactions.

Background: awards prior to fiscal year 2012

On six occasions in fiscal years 2004 through 2010, we issued shares of our preferred stock to venture capital investors. Our most recent preferred stock financing before fiscal year 2012 was on February 23, 2010, at which time we issued shares of our Series A-1 preferred stock for $0.9658 per share (or $33.80 per share on an as-converted to common stock basis). On November 4, 2010, we issued stock options for 28 shares of our common stock at an exercise price equal to $3.50 per share, or approximately 10% of the as-converted purchase price of the Series A-1 preferred stock issued in February 2010, which our board determined to be not less than the fair value of our common stock. These were our last option grants prior to fiscal year 2012.

In estimating the fair value of our common stock as of November 4, 2010 and determining that this 10-to-1 ratio between the arms length price paid for our Series A-1 preferred stock and the estimated fair value of our common stock was reasonable, we took into account the early status of the clinical and preclinical studies relating to our product candidates, the lack of marketability of our common stock, the preferences and privileges of the preferred stock over the rights of the common stock, the lack of voting control on the part of the holders of

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the common stock and our assessment that there was a low likelihood of achieving a liquidity event that would result in the receipt of value by the holders of common stock underlying the stock options in the near term.

We also considered a retrospective third-party valuation of our common stock, dated as of June 30, 2010. In conducting its valuation, the valuation firm determined that the market approach was appropriate for a valuation of our equity, given the then-current stage of our development and the nature of our company. In applying the market approach, the valuation firm estimated our enterprise value on a marketable, control basis, based upon the $0.9658 per share price paid for the Series A-1 preferred stock that we issued on February 23, 2010. After subtracting the liquidation preferences of our preferred stock, the valuation firm determined the equity value attributable to our common stock and, after applying a marketability discount of 40% and a control discount of 20%, concluded that the fair value of our common stock as of June 30, 2010 was $3.50 per share.

Stock option grants on August 25, 2011

In the first option award in fiscal year 2012, on August 25, 2011, our board awarded options for 3,999 shares of common stock at an exercise price of $3.50 per share, which it determined to be not less than the fair value of our common stock.

In estimating the fair value of our common stock as of August 25, 2011, we took into account the lack of marketability of our common stock, the preferences and privileges of the preferred stock over the rights of the common stock, the lack of voting control on the part of the holders of the common stock and our assessment that there was a low likelihood of achieving a liquidity event for the shares of common stock underlying the stock options in the near term. We also considered developments in the preclinical and clinical trials of our product candidates, including the fact that we had been awarded a $1.5 million grant from the Foundation Fighting Blindness to fund animal studies on our XLRS program, which was a positive development, but also noted that we had not yet received data from the pending clinical trial of our most advanced proof-of-concept product candidate for the treatment of AAT deficiency.

We also considered a retrospective third-party valuation of our common stock, dated as of June 30, 2011. In conducting its valuation, the valuation firm determined that the market approach was appropriate for a valuation of our equity, given the then-current stage of our development and the nature of our company. In applying the market approach, the valuation firm estimated our enterprise value on a marketable, control basis, based upon the most recent arms length transaction, namely the $0.9658 per share price paid for the Series A-1 preferred stock that we issued on February 23, 2010. After subtracting the liquidation preferences of our preferred stock, the valuation firm determined the equity value attributable to our common stock and, after applying a marketability discount of 40% and a control discount of 20%, concluded that the fair value of our common stock as of June 30, 2010 was $3.50 per share.

Balancing these factors, we considered that there was no reason to increase or decrease our estimate of the fair value of our common stock from the previous estimate of $3.50 per share.

Stock option grants on November 2, 2011

On November 2, 2011, our board awarded options for 3,934 shares of common stock at an exercise price of $3.50 per share, which it determined to be not less than the fair value of our common stock.

In estimating the fair value of our common stock as of November 2, 2011, we primarily considered that in November we received data from the clinical trials of our AAT deficiency product candidate, which was our most advanced program. The data were encouraging, in that they provided evidence of safety, dose response and sustained expression. However, in these trials the protein expression did not reach therapeutic levels. As a result, we concluded that additional development and clinical trials would be necessary for this product, and that it would be advisable for us to seek a partner with whom to share the cost of this effort.

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Balancing these factors, we considered that there was no reason to increase or decrease our estimate of the fair value of our common stock from the previous estimate of $3.50 per share.

Stock option grants on January 6, 2013

On January 6, 2013, our board awarded options for 192,067 shares of common stock at an exercise price of $0.35 per share, which it determined to be not less than the fair value of our common stock.

The principal factor influencing our estimate of the fair value of our common stock as of January 6, 2013 was the fact that in November 2012, we entered into definitive agreements with respect to a $37.5 million Series B preferred stock financing and sold 66,147,709 shares of our Series B-1 preferred stock at a price of $0.1297 per share (or $4.54 on an as-converted to common stock basis). The Series B-1 investment, which was led by a new investor, reflected a valuation of our company that was 87% lower than that reflected in the most recent Series A-1 financing in 2010.

We believe that the primary factors that influenced this lower valuation were the failure of our AAT deficiency product candidate to achieve serum AAT expression levels of 11 μM or more in its clinical trials; the fact that we had not identified a partner to help fund continued development of our AAT deficiency program; the fact that our next most advanced product candidate, for the treatment of LCA2, while generating encouraging clinical data, addressed a disease with a small population estimated at only 600 patients in the United States and Europe; the fact that the commercial terms on which we had licensed our wet AMD product candidate to Genzyme were such that we no longer expected to receive substantial revenue from the wet AMD program; and the fact that our lead ophthalmology programs in XLRS and ACHM were still in an early and uncertain preclinical stage of development.

In connection with our Series B preferred stock financing, we obtained a contemporaneous, independent third-party valuation of our common stock, as of November 30, 2012, from the same firm that had previously performed annual valuations of our common stock. In conducting its valuation, the valuation firm again determined that the market approach was appropriate for a valuation of our equity, given the then-current stage of our development and the nature of our company. In applying the market approach, the valuation firm estimated our enterprise value on a marketable, control basis, based upon the most recent arms length transaction, namely the $0.1297 per share price paid for the Series B preferred stock that we issued in November 2012. After subtracting the liquidation preferences of our preferred stock, the valuation firm determined the equity value attributable to our common stock was zero.

In estimating the fair value of our common stock, we recognized, as did the independent valuation firm, that any sale or other exit scenario at a valuation less than the accumulated liquidation preference (other than an initial public offering that resulted in mandatory conversion to common stock of our outstanding preferred stock), would result in the receipt by common stockholders of no consideration. In January 2013, conditions in the United States economy remained uncertain and the investment climate for biotechnology companies in general was not favorable, with investment in biotech companies in the United States, Europe and Canada declining in 2012 compared to 2011. We also considered that our business development efforts had not identified any strategic partner or collaborator for our programs other than the wet AMD program licensed to Genzyme. The initial public offering market in the United States was weak, no gene therapy company had ever reached the public markets, and we did not consider an initial public offering by our company to be a realistic option in the foreseeable future. We therefore considered the likelihood of any near term exit scenario at a valuation that would result in receipt of consideration by our common stockholders to be extremely low.

However, our board also did not regard as reasonable the independent valuation firm s assignment of zero value to our common stock. After considering the preferences and privileges of the preferred stock over the rights of the common stock and other factors bearing upon the relative values of our preferred stock and our common stock, we determined that a reasonable estimate of the fair value of our common stock at January 6, 2013 was $0.35 per share. This represented approximately 8% of the as-converted price of the Series B-1 preferred stock

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we issued in November 2012, generally consistent with the discount we applied in determining the fair value of our common stock in relation to our previous preferred stock issuances.

Stock option grants on April 19, 2013

On April 19, 2013, our board of directors awarded options for 999 shares of common stock at an exercise price of $0.35 per share, which it determined to be not less than the fair value of our common stock.

In estimating the fair value of our common stock on April 19, 2013, we considered the positive development that in March 2013 we received initial funding under a $0.3 million grant from the Alpha-1 Foundation to support our clinical trials of our product candidate addressing AAT deficiency. In March 2013, we also obtained primate data demonstrating the ability to deliver our product candidate addressing XLRS by intravitreous injection, satisfying a milestone that resulted in the funding of a second tranche of our Series B financing, at a price equal to $0.1485 per share (or $5.20 on an as-converted to common stock basis). However, after taking into account the uncertain investment climate for gene therapy companies and what we still considered to be the very low likelihood of any near term exit scenario at a valuation that would result in receipt of consideration by our common stockholders, we concluded that there was no reason to increase or decrease our estimate of the fair value of our common stock from our previous estimate of $0.35 per share as of January 6, 2013.

Stock option grants on September 18, 2013

On September 18, 2013, our board of directors authorized the grant of options for 371,718 shares of common stock at an exercise price of $4.90 per share, which it determined to be not less than the fair value of our common stock on that date. Share-based compensation expense attributable to these awards is accounted for in our statement of operations for the six months ended December 31, 2013.

In connection with establishing the exercise price for the September 18, 2013 option awards and estimating the fair value of our common stock as of September 18, 2013, we obtained a contemporaneous third-party valuation by an independent valuation firm other than that which had previously performed valuations of our common stock. This valuation, dated as of September 18, 2013, was conducted in accordance with the framework of the American Institute of Certified Public Accountants Technical Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, or the Practice Aid, utilizing the probability weighted expected return method, or PWERM.

Using the PWERM method, the value of an enterprise s common stock is estimated based upon an analysis of future values for the company assuming various possible future liquidity events. Share value is based upon the probability-weighted present value of expected future net cash flows, considering each of the possible future events, as well as the rights and preferences of each share class.

As part of this valuation, we considered various scenarios involving the consummation of an initial public offering and our remaining a private company. We utilized the following probability-weighted scenarios:

Scenario	Probability
IPO by first calendar quarter of 2014	40%
IPO by second calendar quarter 2014	10%
Remain private through late 2015	50%

In assigning probabilities to the two IPO scenarios and to the remain private scenario, we considered the uncertainties affecting the public securities markets and the risk that we would be unable to successfully complete an initial public offering. We also considered the fact that as a result of our Series B financing in November 2012 and committed grants, we believe we have funding sufficient to enable us to complete planned preclinical studies and Phase 1/2 clinical trials for our lead product candidates, and therefore do not need to raise capital to reach a value inflection point. We believed that these factors made it less likely that we would complete a public offering, particularly if market conditions were unfavorable, and more likely that we would remain independent as a private company.

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We used the market approach, in addition to considering the preliminary valuation indications that we received from various investment banking groups, to estimate our future exit values in connection with an assumed initial public offering of our common stock occurring in the first quarter of 2014, or IPO scenario 1, and the second quarter of 2014, or IPO scenario 2. In making this estimate, we considered the current stage of development of our various product candidates, analysis of pre-money valuations in recent IPOs by other companies of similar size at similar stages of clinical development, the strength of the current market for initial public offerings in the biotechnology industry and the preliminary valuations provided to us by various investment banking groups with which we met in August 2013.

In considering the remain private scenario, we applied the option-pricing model, or OPM, back-solve method to solve for the equity value and corresponding value of common stock based on the $5.20 price per share of common stock issuable upon the conversion of Series B-2 preferred stock sold in April 2013. The OPM pricing method treats preferred and common stock as call options on the enterprise s value, with exercise prices based on the liquidation and conversion preferences of the preferred stock, and based on the common equity per-share values equal to outstanding option and warrant exercise prices. The option pricing method relies on a number of inputs, including the expected time to a liquidity event, the risk free rate, volatility and expected dividend yield.

We utilized the following assumptions as inputs in the option-pricing method:

Assumption	Value
Expected time to liquidity	2.25 years
Risk-free interest rate	0.36%
Volatility	60.0%
Expected dividend yield	0.0%

We assumed an expected time to a liquidity event of approximately 2.25 years, which equates to a liquidity event occurring at December 31, 2015. In arriving at this estimate, we considered our cash position and burn rate, the stage of clinical development of our product candidates and upcoming clinical milestones. We selected a risk free rate equal to the yield on the U.S. Treasury bond, stripped principal, with a maturity date approximating the expected liquidity date. Based on an expected liquidity date of December 31, 2015, we utilized a risk-free interest rate of 0.36%. We estimated volatility equal to 60.0%, which approximates the third quartile of the re-levered volatilities from a group of guideline companies that we considered similar to us in size and diversification. Because we have never declared a dividend on our common stock and do not expect to do so in the foreseeable future, we utilized an assumed 0.0% dividend yield.

After applying the probability weightings described above, we determined the probability-weighted marketable value of the common stock based on the three scenarios to equal $6.30 per share on a marketable minority interest basis.

We then applied a discount for lack of marketability, or DLOM, of our common stock. We utilized the Black-Scholes standard put option model and the average-strike put option pricing model to estimate the DLOM. Based upon these methods, we considered an appropriate DLOM to be 20%. Taking this into account, we determined the fair value of our common stock to be $4.90 per share as of September 18, 2013.

We believe the increase in our estimate of fair value at September 18, 2013, compared to our most recent previous valuation of $0.35 per share as of April 19, 2013, is due to the following principal factors:

- By the time of these September 18, 2013 awards, conditions in the securities markets and the prospects for our industry in general, and our company in particular, had changed dramatically. A growing body of clinical data providing evidence of efficacy and safety of gene therapy in a variety of diseases, improvements in vector design and manufacturing processes by us and others and the establishment of regulatory guidelines for the development and approval of gene therapy products had led to increased investment from the biopharmaceutical industry.

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In November 2012, the first gene therapy treatment to be approved by any regulatory authority in the Western world had been approved by the European Commission, and in July 2013 the developer of the product announced that it had entered into a collaboration to commercialize the product.

We also regarded the recent preclinical data demonstrating the feasibility of intravitreal delivery of AAV vectors in primates to be an important confirmation of the potential of our gene therapy approach in ophthalmic disease.

Meanwhile, the number of IPOs completed in the United States in the second calendar quarter of 2013 almost doubled compared to the first calendar quarter of 2013. More relevant to us, beginning in the second calendar quarter of 2013 and particularly in the third and fourth calendar quarters of 2013, the volume of initial public offerings by biotechnology companies accelerated significantly. Even more importantly, for the first time, these included offerings by companies in the early stages of developing treatments in various disease areas, including one based on gene therapy. As a result of these developments, we believed that investors had developed significant interest in the area of gene therapy.

Further, in May 2013, we and the University of Florida were jointly awarded an $8.3 million grant from the NEI to support development of our ACHM product, of which we expect to receive approximately $4.0 million over the next five years. During the summer of 2013, we continued to make progress to complete the design and construction of vectors for our ACHM product candidate and in August 2013, we commenced preclinical animal studies of that product.

At a board meeting in August 2013, our board of directors and management reviewed recent developments in the IPO market for early-stage gene therapy companies and for the first time began to consider conducting an underwritten public offering of our common stock. We began interviewing investment banks, and by early September 2013, we had selected underwriters for a proposed initial public offering. The organizational meeting for the offering contemplated by this prospectus occurred on September 12, 2013.

Option awards since September 18, 2013

We have not granted any stock options or other form of equity-based compensation since September 18, 2013. In connection with the employment of our new chief financial officer, we agreed to issue to him, subject to approval by our board of directors, stock options to purchase a number of shares of our common stock representing 1.0% of our fully diluted shares, vesting over four years, at an exercise price equal to the fair market value of our common stock on the date of grant. We expect that our board of directors will authorize the grant of this option contemporaneously with the offering contemplated by this prospectus, at an exercise price equal to the initial public offering price.

Initial public offering price

We estimate that the initial public offering price will be between $13.00 and $15.00 per share. In comparison, our most recent previous estimate of the fair value of our common stock, made in connection with our grant of options on September 18, 2013, was $4.90 per share. We note that, as in most initial public offerings, the actual initial public offering price will not be derived using a formal determination of fair value, but will be determined by negotiation between us and the underwriters.

Among the factors that were considered in making the estimate of our initial public offering price were the following:

an analysis of the typical valuation ranges seen in recent initial public offerings for companies in our industry;

the general condition of the securities markets and the recent market prices of, and the demand for, publicly traded common stock of generally comparable companies;

an assumption that there would be a receptive public trading market for pre-commercial gene therapy companies such as us; and

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an assumption that there would be sufficient demand for our common stock to support an offering of the size contemplated by this prospectus.

The estimated initial public offering price reflects a significant increase over the estimated valuation as of September 18, 2013 of $4.90 per share. Investors should be aware of this difference and recognize that the estimated initial public offering price is in excess of our prior valuations. We believe the difference may be due to the following factors:

Whereas the estimated initial public offering price necessarily assumes that this offering has occurred, the valuation as of September 18, 2013 was based upon several probability-weighted scenarios, as follows:

Scenario	Probability
IPO by first calendar quarter of 2014	40%
IPO by second calendar quarter 2014	10%
Remain private through late 2015	50%

The use of these alternative liquidity scenarios as inputs accounts for a significant portion, but not all, of the difference between the estimated initial public offering price and the valuation as of September 18, 2013.

In November 2013, we announced that we had expanded our management team by hiring our new vice president and chief business officer and our new project director, and in February 2014 we announced that we had hired our new chief financial officer, each of whom has significant prior leadership experience in the pharmaceutical and biotech industries.

In November 2013, we also reported data from a Phase 2 clinical trial using our proprietary AAV gene therapy vector system that demonstrated that intramuscular administration of AAV-based therapies supports sustained expression of a therapeutic protein for up to a year following a single dose.

In December 2013, we were awarded an additional $658,000 in funding by the NEI, to support expansion of our preclinical development work for our product candidate for achromatopsia. We also completed a study in non-human primates that supported the feasibility of delivering AAV-based therapies to all cone types in humans, a critical development in our ability to develop treatments for a range of ophthalmology indications.

We believe that since September 2013, new research published in the scientific literature providing further evidence of the safety and efficacy of AAV vectors generally, and the announcement of venture capital investments in several early stage gene therapy companies and of a number of licensing and partnering transactions by biopharmaceutical companies in this field, have all provided further validation for the emergence of gene therapy as an important therapeutic modality.

Capital market conditions for companies in our industry also improved since September 18, 2013, as evidenced by a recent substantial increase in the number of public offerings by biotech companies, including other gene therapy companies, and in the initial public offering valuations of such companies compared to the valuations in their most recent pre-IPO equity financing.

The estimated initial public offering price assumes that this offering has occurred, a public market for our common stock has been created and that our preferred stock has converted into common stock in connection with this offering and, therefore, excludes the marketability or illiquidity discounts associated with the timing or likelihood of an initial public offering, the superior rights and preferences of our preferred stock and the alternative scenarios considered in the contemporaneous valuations since the beginning of fiscal year 2012. By contrast, our September 18, 2013 valuation included a discount for lack of marketability of 20%.

We believe that investors in the public markets may apply more qualitative and subjective valuation criteria to our product candidates and business than the quantitative valuation methods we applied,

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although there can be no assurance that this is the case. Further, the initial public offering price will not be derived using a quantitative determination of fair value, but rather will be determined by negotiation between us and the underwriters. In particular, our estimate of the fair value of our common stock as of September 18, 2103 was not considered by us or the underwriters as a factor in estimating the initial public offering price.

The price that investors are willing to pay in this offering may take into account other things that have not been expressly considered in our prior valuations, that are not objectively determinable and that valuation models are not able to quantify.

The valuations described above were made solely for the purposes of valuing the common stock underlying our stock option grants for financial reporting purposes and involved significant judgments and estimates, including assumptions regarding our future performance and the success of our pre-clinical studies and planned clinical trials. If we had made different assumptions, our stock-based compensation expense could have been different. The valuation methodologies we have historically used in estimating the fair value of our common stock are not the only methodologies available and they will not be used to value our common stock once this offering is complete. We cannot predict or offer any assurance with regard to the future value of our common stock. Accordingly, investors are cautioned not to place undue reliance on the valuation methodologies we describe above as an indicator of our future stock prices. Before investing in our common stock, you should carefully read this entire prospectus and consider, among other things, the matters described under Risk Factors.

Warrant liability

As of June 30, 2012 and 2013 and December 31, 2013, we had warrants outstanding to purchase shares of our Series A-1, Series A-1A and Series B-1 preferred stock. Because our Series A-1, Series A-1A and Series B-1 preferred stock are subject to redemption under circumstances outside of our control, the outstanding shares of these series of preferred stock are presented as temporary equity. Consequently, the warrants to purchase shares of Series A-1, Series A-1A and Series B-1 preferred stock are accounted for as liabilities and adjusted to fair value at the end of each reporting period. The fair value of the warrants classified as liabilities is estimated using the Black-Scholes option pricing model. The estimates in the Black-Scholes option pricing model are based, in part, on subjective assumptions, including stock price volatility, term of the warrants, risk free interest rate, dividend yield, and fair value of the preferred stock underlying the warrants. Such assumptions could differ materially in the future. The gain or loss associated with the change in the fair value of the preferred stock warrant liability from the prior period is recognized as a component of other (expense) income, net.

Series B purchase rights

In November 2012, we entered into a Series B-1, B-2 and B-3 Preferred Stock Purchase Agreement, or Series B Purchase Agreement, which authorized the sale of up to 290,781,972 shares of convertible preferred stock in three separate tranches of Series B-1, Series B-2 and Series B-3 preferred stock, respectively. Simultaneously with the execution of the Series B Purchase Agreement, we issued and sold an aggregate of 66,147,709 shares of Series B-1 preferred stock at a price per share of $0.1297. The Series B Purchase Agreement provided that the holders of the Series B-1 shares, or Series B holders, were also entitled to purchase up to an aggregate of 140,542,178 shares of Series B-2 preferred stock for an aggregate purchase price equal to $18.2 million, or second tranche, and up to an aggregate of 82,670,167 shares of Series B-3 preferred stock for an aggregate purchase price equal to $10.7 million, or third tranche. The price per share and number of shares to be issued in exchange for such amount was to be determined separately for each tranche by reference to which, if any, of three milestones specified in the agreement had been satisfied by us.

The purchase rights were legally separable and exercisable apart from the Series B-1 shares and, because representatives of the Series B holders hold a majority of the seats on the board of directors, the decision to complete the second and third tranche was deemed to be outside our control. We therefore recorded, at the time

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of entry into the Series B Purchase Agreement, a Series B purchase rights liability of $1.7 million for the fair value of our obligation to sell the Series B-2 and Series B-3 preferred stock in the second and third tranche. The Series B purchase rights liability was valued separately for each series using the Black-Scholes option-pricing method to assign a value to the purchase right relating to that series under each of the possible applicable valuation scenarios, depending on which milestones were met, with each scenario being assigned an estimated probability as of the valuation date. The aggregate of these probability-weighted valuations was assigned as the value of the purchase right for each tranche. The initial fair value of the Series B purchase rights liability was estimated to be $0.6 million for the second tranche and $1.1 million for the third tranche. The total value allocated to the Series B purchase rights reduced the amount allocated to the carrying value of the Series B-1 preferred stock on our balance sheet.

The most significant and judgmental inputs driving the fair value of our Series B purchase rights are the assumptions regarding the fair value of the underlying preferred shares and the volatility factor. With all other inputs constant, an increase or decrease in the assumed fair value of the preferred shares would result in a higher or lower estimate of the fair value of the Series B purchase rights, respectively, although there would not be a direct correlation. Similarly, an increase or decrease in the assumed volatility factor would result in a higher or lower estimate of the fair value of the Series B purchase rights, respectively.

In April 2013, following the satisfaction by us of the first milestone, the Series B holders exercised their rights with respect to the second tranche and purchased an aggregate of 122,749,639 shares of Series B-2 preferred stock at a price per share of $0.1485, for gross cash proceeds of $18.2 million. During fiscal year 2013, we recorded a change in value of the Series B purchase rights liability of $1.2 million to other expense and the $0.8 million balance of the value allocated to the Series B-2 purchase rights liability immediately prior to the closing of the second tranche was recorded as proceeds from the issuance of the Series B-2 preferred stock.

In October 2013, the Series B holders exercised their rights with respect to the third tranche and on November 5, 2013, we sold to the Series B holders an aggregate of 58,816,897 shares of our Series B-3 preferred stock at a price per share of $0.1823 (or $6.38 on an as-converted to common stock basis), for gross cash proceeds of $10.7 million. In connection with the closing of the third tranche, we and the Series B holders amended the terms of the Series B purchase agreement to provide that if the two remaining milestones specified in the Series B Purchase Agreement are not satisfied by September 2014, the Series B holders who continue to hold shares of Series B-3 preferred stock will be entitled to receive an aggregate of approximately 13.4 million additional shares of Series B-3 preferred stock. This right will be extinguished upon the conversion to common stock of all outstanding shares shares of our preferred stock upon the closing of this offering.

During the six months ended December 31, 2013, we recorded a change in value of the Series B purchase right liability of $2.8 million to other expense, and $4.9 million allocated to the Series B-3 purchase right immediately prior to the closing of the third tranche was reallocated to the carrying value of the Series B-3 preferred stock.

The significant assumptions used as inputs in the Black-Scholes valuation were as follows:

Assumption	Year Ended June 30, 2013	Six Months Ended December 31, 2013
Exercise price	$0.1297 to $0.1823	$0.1485 to $0.1823
Years to maturity	0.37 to 1.87	0.00 to 1.00
Risk-free interest rate	0.04% to 0.25%	0.06% to 0.10%
Volatility	40.0% to 60.0%	55% to 85.0%

Income taxes

We recognize deferred taxes for temporary differences between the basis of assets and liabilities for financial statement and income tax purposes. At June 30, 2013, we had net operating losses of approximately

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$46.9 million that may be applied against future taxable income and expire in various years from 2022 to 2033. At June 30, 2013, we also had research and development tax credits of approximately $0.9 million that may provide future tax benefits and expire from 2027 to 2042.

We periodically evaluate the positive and negative evidence bearing upon the realizability of our deferred tax assets. Based on our history of operating losses, we have concluded that it is more likely than not that the benefit of our deferred tax assets will not be realized. Therefore, any tax benefits to be realized in future years as a result of the utilization of our net operating loss carry forwards as of June 30, 2013, computed based on statutory federal and state rates, are completely offset by valuation allowances.

Under Section 382 of the Internal Revenue Code of 1986, as amended, substantial changes in our ownership may limit the amount of net operating loss carryforwards that could be utilized annually in the future to offset our taxable income. Specifically, this limitation may arise in the event of a cumulative change in ownership of our company of more than 50% within a three-year period. Any such annual limitation may significantly reduce the utilization of our net operating loss carryforwards before they expire. We have not completed a study to assess whether an ownership change has occurred, or whether there have been multiple ownership changes since our inception, due to the significant costs and complexities associated with such study. However, we believe it is likely that transactions that have occurred in the past, alone or together with the closing of this offering and other transactions that may occur in the future, would trigger an ownership change pursuant to Section 382, which could limit the amount of net operating loss carryforwards that could be utilized annually in the future to offset our taxable income, if any.

For all years through June 30, 2013, we generated research credits but we have not conducted a study to document the qualified activities. When completed, this study may result in an adjustment to our research and development credit carry forwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position for these two years. A full valuation allowance has been provided against our research and development credits and, if an adjustment is required, this adjustment would be offset by an adjustment to the deferred tax asset established for the research and development credit carry forwards and the valuation allowance.

Our policy is to recognize interest and penalties related to uncertain tax positions in income tax expense. As of June 30, 2012 and 2013 and at December 31, 2013, we had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in our statements of operations.

Internal control over financial reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting for our company. Internal control over financial reporting is a process to provide reasonable assurance regarding the reliability of our financial reporting and the preparation of financial statements for external purposes in accordance with accounting principles generally accepted in the United States. Internal control over financial reporting includes maintaining records that in reasonable detail accurately and fairly reflect our transactions; providing reasonable assurance that transactions are recorded as necessary for preparation of our financial statements; providing reasonable assurance that receipts and expenditures of our assets are made in accordance with management s authorization; and providing reasonable assurance that unauthorized acquisition, use or disposition of our assets that could have a material effect on the financial statements would be prevented or detected on a timely basis. Because of its inherent limitations, internal control over financial reporting is not intended to provide absolute assurance that a misstatement of our financial statements would be prevented or detected. Furthermore, our controls and procedures can be circumvented by the individual acts of some persons, by collusion of two or more people or by management override of the control, and misstatements due to error or fraud may occur and not be detected on a timely basis.

Our management has determined that we have material weaknesses in our internal control over financial reporting which relate to the design and operation of our closing and financial reporting processes and our

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accounting for debt, equity and convertible instruments. We have concluded that these material weaknesses in our internal control over financial reporting are due to the fact that we do not have the appropriate resources with the appropriate level of experience and technical expertise to oversee our closing and financial reporting processes and to address the accounting and financial reporting requirements related to our issuances of convertible notes, preferred stock warrants, stock options, preferred stock and preferred stock purchase rights.

In order to remediate these material weaknesses, we are taking the following actions:

- we have hired two additional accounting and finance staff, including a permanent chief financial officer;
- we are actively seeking additional accounting and finance staff members, including a senior accounting officer with public company reporting experience, to augment our current staff and to improve the effectiveness of our closing and financial reporting processes; and
- we are formalizing our accounting policies and internal controls documentation and strengthening supervisory reviews by our management.

Notwithstanding the material weaknesses that existed as of June 30, 2012 and 2013, our management has concluded that the consolidated financial statements included elsewhere in this prospectus present fairly, in all material respects, our financial position, results of operation and cash flows in conformity with U.S. generally accepted accounting principles.

If we fail to fully remediate these material weaknesses or fail to maintain effective internal controls in the future, it could result in a material misstatement of our financial statements that would not be prevented or detected on a timely basis, which could cause investors to lose confidence in our financial information or cause our stock price to decline. Our independent registered public accounting firm has not assessed the effectiveness of our internal control over financial reporting and, under the Jumpstart our Business Startups Act of 2012, or the JOBS Act, will not be required to provide an attestation report on the effectiveness of our internal control over financial reporting so long as we qualify as an emerging growth company, which may increase the risk that weaknesses or deficiencies in our internal control over financial reporting go undetected.

Emerging growth company status

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

Results of operations

Comparison of the fiscal years ended June 30, 2012 and 2013

Revenue

	Fiscal year ended June 30,			%
	2012	2013	Increase (Decrease)	Increase (Decrease)
	(dollars in thousands)			
Grant revenue	$ 718	$ 439	$ (279)	(39)%
Sponsored research revenue	364	503	139	38%
Total revenue	$ 1,082	$ 942	$ (140)	(13)%

Grant revenue decreased by $0.3 million from $0.7 million to $0.4 million from fiscal year 2012 to fiscal year 2013. The decrease was primarily the result of the timing of the release of funding under our FDA orphan grants relating to our LCA2 and AAT deficiency product candidates. Sponsored research revenue increased by

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$0.1 million from $0.4 million to $0.5 million from fiscal year 2012 to fiscal year 2013. The increase was primarily the result of increased activity under our sponsored research arrangement with FFB related to the development of our XLRS product candidate.

Research and development expense

	Fiscal Year Ended June 30,			%
	2012	2013	Increase (Decrease)	Increase (Decrease)
	(dollars in thousands)			
Research and development expense	$ 2,354	$ 3,133	$ 779	33%

Research and development expense increased by $0.8 million from $2.4 million for fiscal 2012 to $3.1 million for fiscal 2013. The increase was the result of increased activity relating to our XLRS and ACHM product candidates, including increased facilities costs relating to new laboratory expansion, increased personnel costs relating to new hires and the acquisition of related laboratory supplies.

General and administrative expense

	Fiscal Year Ended June 30,			%
	2012	2013	Increase (Decrease)	Increase (Decrease)
	(dollars in thousands)			
General and administrative expense	$ 787	$ 1,403	$ 616	78%

General and administrative expense increased by $0.6 million from $0.8 million to $1.4 million for fiscal year 2012 to fiscal year 2013. The increase was primarily the result of increased overhead and personnel costs.

Other income (expense), net

Other income (expense), net decreased from income of $0.1 million in fiscal year 2012 to expense of $(1.4) million in fiscal year 2013, due to the following factors. Interest expense increased by 177% from $69,000 for fiscal year 2012 to $0.2 million for fiscal year 2013, primarily a result of the recognition of unamortized debt discount on our May 2012 convertible notes as interest expense in connection with the conversion of the notes to shares of Series B-1 preferred stock during fiscal year 2013. Other expense also increased by the $1.2 million fair value adjustments to our Series B purchase rights and our warrant liabilities that are described in note 11 to our financial statements appearing elsewhere in this prospectus.

Comparison of the six months ended December 31, 2012 and 2013

Revenue

	Six Months Ended December 31,		Increase	% Increase
	2012	2013	(Decrease)	(Decrease)
	(dollars in thousands)			
Grant revenue	$ 293	$ 466	$ 173	59%
Sponsored research revenue	$ 156	$ 307	$ 151	97%

Grant revenue for the six months ended December 31, 2013 increased by $0.2 million to $0.5 million from $0.3 million for the six months ended December 31, 2012. The change was primarily the result of the inception of new grant-funded projects related to our ACHM product candidate, which was partly offset by decreased activity of grant-funded projects relating to our AAT deficiency and LCA2 product candidates. Sponsored research revenue increased by $0.2 million from $0.2 million to $0.3 million from the six months ended December 31, 2012 to the six months ended December 31, 2013. The increase was primarily the result of increased activity under our sponsored research agreement with The Alpha-1 Project related to the development of our AAT product candidate, which was partly offset by decreased activity under our sponsored research arrangement with FFB related to the development of our XLRS product candidate.

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Research and development expense

	Six Months Ended December 31, 2012	2013	Increase (Decrease)	% Increase (Decrease)
		(dollars in thousands)		
Research and development expense	$ 1,156	$ 3,673	$ 2,517	218%

Research and development expense increased by $2.5 million from $1.2 million for the six months ended December 31, 2012 to $3.7 million for the six months ended December 31, 2013. The increase was the result of increased activity relating to our XLRS and ACHM product candidates, including increased facilities costs relating to new laboratory expansion, increased personnel costs relating to new hires and the acquisition of related laboratory supplies.

General and administrative expense

	Six Months Ended December 31, 2012	2013	Increase (Decrease)	% Increase (Decrease)
		(dollars in thousands)		
General and administrative expense	$ 649	$ 1,971	$ 1,322	204%

General and administrative expense increased by $1.3 million from $0.6 million for the six months ended December 31, 2012 to $2.0 million for the six months ended December 31, 2013. The increase was the result of increased personnel costs relating to new hires, as well as increased legal and accounting costs.

Other income (expense), net

Other income (expense), net decreased from expense of $(0.6) million for the six months ended December 31, 2012 to expense of $(2.9) million for the six months ended December 31, 2013, due primarily to the following factors. Interest expense decreased from $0.2 million to $0, as a result of our repayment of our outstanding bank credit facility. Other expense increased by the $2.9 million fair value adjustments to our Series B purchase rights and our warrant liabilities that are described in the footnotes to our financial statements for the six months ended December 31, 2013 appearing elsewhere in this prospectus.

Liquidity and capital resources

We have incurred cumulative losses and negative cash flows from operations since our inception in 1999, and as of December 31, 2013, we had an accumulated deficit of $56.2 million. It will be several years, if ever, before we have a product candidate ready for commercialization, and we anticipate that we will continue to incur losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may raise through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements.

In August 2012, we amended our existing term loan facility with Square 1 Bank to provide for up to an additional $0.5 million of available funding. We borrowed the full amount in September 2012. The loan bore interest at 9% per annum through December 2012 and 7% per annum thereafter. We were required to make monthly payments of interest only through December 2012. Thereafter the loan was to be repaid through 24 equal monthly installments of principal and accrued interest. In April 2013, we repaid all outstanding principal and accrued interest and terminated the loan facility.

In connection with the funding of the loan, we issued to Square 1 Bank a warrant to purchase 276,968 shares of our Series B-1 preferred stock at an exercise price of $0.1297 per share. The warrants may be exercised at any time until the seventh anniversary of their date of issuance.

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As of December 31, 2013, we had cash and cash equivalents and short-term investments of $28.0 million. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. Currently, our cash and cash equivalents are held in bank accounts and money market mutual funds consisting of U.S. government-backed securities. Our short-term investments consist of certificates of deposits with maturity within 91 and 360 days of the date of purchase.

Cash flows

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

	Fiscal Year Ended June 30,		Six Months Ended December 31,	
	2012	2013	2012	2013
	(in thousands)			
			(unaudited)	
Net cash provided by (used in):				
Operating activities	$ (1,372)	$ (2,777)	$ (1,094)	$ (5,437)
Investing activities	(108)	(14,481)	$ (320)	$ (5,141)
Financing activities	427	25,377	$ 7,956	$ 10,683
Net (decrease) increase in cash and cash equivalents	$ (1,053)	$ 8,119	$ 6,542	$ 105

Operating activities. For the six months ended December 31, 2012 and 2013, net cash used in operating activities was $1.1 million and $5.4 million, respectively. Net cash used in operating activities was $1.4 million for fiscal year 2012 and $2.8 million for fiscal year 2013. The use of net cash in all periods primarily resulted from our net losses and changes in our working capital accounts.

Investing activities. Net cash used in investing activities for the six months ended December 31, 2012 was $0.3 million and consisted primarily of $0.2 million in costs related to the purchase of property and equipment and $0.1 million of costs related to the acquisition and maintenance of intellectual property. Net cash used in investing activities for the six months ended December 31, 2013 was $5.1 million and consisted of $20.5 million related to the purchase of short-term investments, $0.1 million of costs related to the acquisition and maintenance of our intellectual property and $0.1 million related to the purchase of property and equipment, off set by $15.5 million of proceeds received upon the maturity of short-term investments.

Net cash used in investing activities for fiscal year 2012 was $0.1 million and consisted primarily of $0.1 million of costs related to the acquisition and maintenance of intellectual property and $8,000 for equipment purchases. Net cash used in investing activities for fiscal year 2013 was $14.5 million and consisted primarily of the purchase of $14.0 million of short-term investments with a portion of the proceeds from our sale of shares of Series B-1 and Series B-2 preferred stock, $0.4 million for the purchase of equipment to support our continued research and development activities and $0.2 million of costs related to the acquisition and maintenance of our intellectual property.

Net cash provided by financing activities for the six months ended December 31, 2012 was $8.0 million and consisted primarily of $7.5 million in proceeds from the sale of shares of Series B-1 preferred stock and Series B purchase rights and $0.4 million of debt financing, net of repayments. Net cash provided by financing activities for the six months ended December 31, 2013 was $10.7 million and consisted primarily of proceeds from the sale of shares of Series B-3 preferred stock.

Net cash provided by financing activities for fiscal year 2012 was $0.4 million and consisted primarily of the proceeds of debt financing, net of repayments. Net cash provided by financing activities for fiscal year 2013 was $25.4 million and consisted primarily of the proceeds from the issuance of our Series B-1 and Series B-2 preferred stock of $25.7 million offset by a net repayment of debt of $0.4 million.

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Operating capital requirements

To date, we have not generated any revenue from product sales. We do not know when, or if, we will generate any revenue from product sales. We do not expect to generate significant revenue from product sales unless and until we obtain regulatory approval of and commercialize one of our current or future product candidates. We anticipate that we will continue to generate losses for the foreseeable future, and we expect the losses to increase as we continue the development of, and seek regulatory approvals for, our product candidates, and begin to commercialize any approved products. We are subject to all of the risks incident in the development of new gene therapy products, and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. Upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. We anticipate that we will need substantial additional funding in connection with our continuing operations.

We expect that the net proceeds from this offering and our existing cash and cash equivalents will be sufficient to enable us to complete planned preclinical and clinical trials for our lead product candidates through at least the next 24 months. In order to complete the process of obtaining regulatory approval for our lead product candidates and to build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize our lead product candidates, if approved, we will require substantial additional funding.

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

- the timing and costs our planned clinical trials for our XLRS and ACHM product candidates;
- the timing and costs of our planned preclinical studies of our XLRP product candidate;
- the initiation, progress, timing, costs and results of preclinical studies relating to potential applications of our gene therapy platform in other indications in orphan ophthalmology;
- our success in scaling our HAVE manufacturing method;
- the number and characteristics of product candidates that we pursue;
- the outcome, timing and costs of seeking regulatory approvals;
- subject to receipt of marketing approval, revenue received from commercial sales of our product candidates;
- the terms and timing of any future collaborations, licensing, consulting or other arrangements that we may establish;
- the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, defense and enforcement of any patents or other intellectual property rights;

the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims; and

the extent to which we in-license or acquire other products and technologies.

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Contractual obligations and commitments

The following table summarizes our contractual obligations at June 30, 2013.

	Total	Less than 1 Year	1 to 3 Years	3 to 5 Years	More than 5 Years
			(in thousands)		
Operating lease obligations (1)	$ 104	$ 81	$ 23	$	$

(1) We lease office and laboratory space in Alachua, Florida under noncancelable operating leases that expire on December 31, 2014.

Contingent contractual obligations. We also have obligations arising under our license agreements to make future payments to third parties that become due and payable on the achievement of certain development, regulatory and commercial milestones (such as the start of a clinical trial, filing of a Biologics License Application, or BLA, approval by the FDA or product launch). We have not included these obligations on our balance sheet or in the table above because the achievement and timing of these milestones is not fixed and determinable. These obligations include:

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

Under our license agreement with the UAB Research Foundation pursuant to which we license a patent covering the use of HSV helpers to produce AAV vectors, we will be required to make payments based upon development and regulatory milestones for any products covered by the in-licensed intellectual property. We will also be required to pay a royalty on net sale of products covered by the in-licensed intellectual property. The royalty is subject to reduction, subject to a minimum floor, for any third-party payments required to be made. We have the right to sublicense our rights under this agreement, and we will be required to pay a percentage of such license income. We are required to make annual maintenance payments under this license, which payments are creditable against royalty payments on a year-by-year basis.

If any of our product candidates that utilize technology licensed under these agreements reached commercialization, we will be obligated to make royalty payments ranging from 0.5% to 4.0% of our net sales of the applicable product. We are responsible for a portion of the costs related to the preparation, filing, issuance, prosecution and maintenance of the patents covered by the license agreements. In fiscal years 2012 and 2013, we paid annual royalty and license maintenance payments in the aggregate amount of $41,000 and $61,000, respectively.

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Based on the anticipated development timeline for our current product candidates described elsewhere in this prospectus, see Our Business Overview, we estimate that the maximum aggregate amount of milestone payments that we will be required to make pursuant to these license agreements during fiscal years 2014, 2015, 2016, and 2017 and beyond is as follows:

Fiscal Year	Aggregate Milestone Payments
2014	$ 90,000(1)
2015	$ 231,000(2)
2016	$
2017 and beyond	$ 4,927,000

(1) Consists of payments to MedImmune and the UAB Research Foundation in connection with the achievement of regulatory milestones related to our ACHM product candidate. Our license agreement with MedImmune expired on February 4, 2014 and we do not expect that any additional milestone payments will become due under that agreement.

(2) Consists of payments to UFRF in connection with the achievement of regulatory milestones related to our ACHM product candidate and payments to UFRF and Johns Hopkins University in connection with the achievement of regulatory milestones related to our XLRS product candidate.

We enter into contracts in the normal course of business with CROs for preclinical research studies, research supplies and other services and products for operating purposes. These contracts generally provide for termination on notice, and therefore are cancelable contracts and not included in the table of contractual obligations and commitments.

Off-balance sheet arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the Securities and Exchange Commission.

Quantitative and qualitative disclosures about market risks

We are exposed to market risk related to changes in interest rates. As of June 30, 2012 and 2013 and December 31, 2013, we had cash and cash equivalents and short-term investments of $0.8 million, $22.9 million and $28.0 million, respectively, primarily held in bank accounts and certificates of deposit. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term securities. Our available for sale securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 10% increase in interest rates would not have a material effect on the fair market value of our portfolio.

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OUR BUSINESS

Overview

We are a clinical-stage biotechnology company developing gene therapy products designed to transform the lives of patients with severe inherited orphan diseases in ophthalmology. We believe our proprietary gene therapy platform and our expertise in viral vector selection and design, delivery and manufacturing will facilitate the rapid clinical advancement and regulatory approval of our product candidates and enhance their commercial and therapeutic potential.

Our lead product candidates are treatments for X-linked retinoschisis, or XLRS, achromatopsia, or ACHM, and X-linked retinitis pigmentosa, or XLRP. These orphan diseases of the eye are caused by mutations in single genes, significantly affect visual function and currently lack effective medical treatments. XLRS is characterized by abnormal splitting of the layers of the retina, resulting in poor visual acuity in young boys, which can progress to legal blindness in adult men. For our XLRS product candidate, we expect to file an Investigational New Drug Application, or IND, with the United States Food and Drug Administration, or FDA, and initiate Phase 1/2 clinical trials in the United States in late 2014, with initial clinical data expected in mid-2015. ACHM is characterized by the absence of cone photoreceptor function, resulting in extremely poor visual acuity, light sensitivity, day blindness and complete loss of color discrimination. For our ACHM product candidate, we expect to file an IND and initiate Phase 1/2 clinical trials in the United States in early 2015, with clinical data expected in late 2015. We have also begun preclinical studies for our product candidate addressing XLRP, a disease characterized by progressive degeneration of the retina, which can lead to total blindness in adult men.

Our gene therapy platform is based on viral vectors that utilize a modified version of the non-replicating adeno-associated virus, or AAV, to deliver a functional copy of a gene to the patient s own cells through a variety of delivery methods, and we have obtained preliminary indications of safety and efficacy in clinical trials. These vectors deliver the functional genetic material to the nucleus of the cell, providing safe, sustained expression of the therapeutic protein to treat the disease without modifying the existing DNA of the patient.

We have developed extensive internal expertise in viral vector selection and design, delivery and manufacturing that is supported by a broad intellectual property estate. Our proprietary AAV vector manufacturing process is both reproducible and scalable. We have assembled an experienced management team and a world-class group of scientific advisors, and we have strong collaborative relationships with key opinion leaders in the field of gene therapy. Combining these attributes, we have built a gene therapy platform that we believe will provide patients with treatments that may have life-long clinical benefits, potentially based on a one-time therapeutic administration.

We and our scientific collaborators have generated human proof-of-concept data that we believe provide preliminary evidence of the safety and efficacy of our gene therapy approach through preclinical studies and clinical trials in two other eye diseases: Leber congenital amaurosis (type 2) caused by mutations in the RPE65 gene, or LCA2, a form of early onset retinal degeneration, and the wet form of age-related macular degeneration, or wet AMD, an eye disease affecting a large patient population.

Our strategy is to leverage the capabilities of our gene therapy platform to address orphan diseases in ophthalmology where there is significant unmet medical need. The orphan eye diseases we are targeting are well-understood with highly predictive animal models and clearly defined clinical endpoints, which we believe will facilitate clinical development and regulatory approval of our product candidates. The prevalence of these diseases is large by orphan standards, but small enough to permit clinical trials on a manageable scale and to provide markets that we believe can be served using a small, targeted commercial infrastructure. We believe that our focus on orphan diseases in ophthalmology provides an attractive business opportunity and positions us to drive the advancement of gene therapy technology.

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Our AAV vectors can be used to introduce functional genes into many different cell types by a variety of delivery methods and can carry genes of up to 4,000 base pairs in length, a payload capacity sufficient to accommodate more than 90% of the individual genes in the human genome. We have developed a proprietary manufacturing process that we believe will enable our vectors to be manufactured reliably on a commercial scale. Our gene therapy platform therefore has the potential to provide treatments for many other diseases outside of our current focus on orphan ophthalmology, including those with large dosing requirements or in larger markets. We have already conducted preclinical proof-of-concept studies and Phase 1 and Phase 2 clinical trials of a treatment for alpha-1 antitrypsin deficiency, or AAT deficiency, an inherited orphan lung disease. We expect to explore other therapeutic areas selectively, either alone or through partnerships.

The chart below summarizes our current gene therapy programs:

Why focus on orphan ophthalmology?

Many chronically debilitating diseases for which there are currently no effective treatments have patient populations too small to attract the interest of large commercial entities. We believe that such orphan diseases can provide us with an attractive business opportunity. We are concentrating initially on several underserved diseases that are prevalent by orphan disease standards but small enough to allow for clinical trials on a manageable scale and to provide markets that we believe we can serve using a small, targeted commercial infrastructure.

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We have focused on orphan ophthalmology because we believe there is a significant unmet medical need in eye diseases. A number of major pharmaceutical companies are working on eye diseases with large patient populations such as glaucoma and AMD, but we are not aware of any major pharmaceutical or biotechnology companies that are actively developing products to address genetic causes of blindness.

The eye diseases we are targeting are also of interest to us due to a number of factors that, in combination, have enabled us to screen and more accurately predict the potential safety and efficacy of products at an early stage of development:

Well-understood disease mechanisms. Because sight is the most important sense to humans many people fear blindness even more than premature death even very rare diseases that cause vision loss have been studied extensively and are well-understood down to the molecular mechanism of action.

Monogenic diseases. We are initially pursuing eye diseases where the genetic abnormality is known and is caused by mutations in a single gene, known as monogenic diseases. We therefore know exactly what gene sequence to insert into the patient s cells, thus mitigating the uncertainty of disease biology.

Highly predictive animal models. For many eye diseases there are highly predictive animal models in which the disease is caused by the same underlying genetic defect as in humans and has similar clinical outcomes.

Local delivery of therapeutic agent. Direct delivery of a therapeutic agent to the cells affected by the disease, via methods already widely used in ophthalmology, allows us to use lower doses, with reduced risk of unintended effects.

Short time to clinical data. In certain eye diseases such as XLRS and ACHM, we expect to obtain meaningful clinical data within three to six months of a one-time administration of the product candidate to a patient, which we believe will facilitate the rapid clinical development of our product candidates.

Ophthalmology is also attractive to us as a clinical-stage company because treatments for diseases affecting vision have clearly defined, objective clinical endpoints with validated measurement tools that are accepted by the FDA. Other orphan drug companies have spent considerable time and resources working with the FDA to identify acceptable clinical endpoints and develop measurement tools in sometimes ill-defined diseases. In ophthalmology the four accepted endpoints visual acuity, visual fields, contrast sensitivity and color vision are well-understood, and the FDA consistently applies them and provides guidance on how much improvement is required to be clinically relevant. We believe these clearly defined endpoints will help accelerate the process of clinical development and regulatory approval for our ophthalmic products.

Finally, through our internal research work and in collaboration with partners, we have obtained preliminary safety data in clinical trials with the two major delivery routes used in ophthalmology: intravitreal and subretinal injection. In clinical trials conducted by our licensee Genzyme, up to 34 patients with wet AMD were treated by intravitreal injection of an AAV vector (see Business Strategic collaborations Our license to Genzyme), and in trials conducted by us and others more than 50 patients with LCA2 have been treated with subretinal injections of AAV vectors, in both cases without reports of serious adverse events attributed to the vector, and with promising indications of efficacy for LCA2 patients.

Our strategy

Our objective is to become the world leader in developing and commercializing gene therapy treatments for severe inherited orphan diseases in ophthalmology, for which there are no currently available treatments, and to thereby provide a better life for patients with these diseases. Our strategy to accomplish this goal is to:

Develop and commercialize drugs in orphan ophthalmology. Our lead product candidates are treatments for the severe orphan eye diseases XLRS and ACHM, for which we expect to initiate Phase 1/2 trials in late 2014 and early 2015, respectively. We are also pursuing early preclinical research

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in XLRP. Given the severity of these diseases and the current lack of treatment options, an alternative that corrects the underlying genetic defects with a one-time therapeutic administration would provide superior long-term value for patients, their families and the healthcare system more broadly.

Continue our leadership position in orphan ophthalmology. We have developed significant experience in the orphan ophthalmology space through our work on XLRS, ACHM, XLRP and LCA2. We have strong relationships with key opinion leaders in the field and with leading patient advocacy groups. We have been awarded grants aggregating $9.0 million from the Foundation Fighting Blindness, or FFB, the National Institutes of Health, or NIH, the National Eye Institute, or NEI, and the FDA. Our scientific advisory board is comprised of leaders in the fields of ophthalmology and genetics, such as William W. Hauswirth, Ph.D., the Rybaczki-Bullard Professor of Ophthalmology and Molecular Genetics at the University of Florida College of Medicine, who is also one of our scientific founders. We will continue to partner with the world s experts in each eye disease category that we target and build a professional team of employees, advisors and collaborators with industry-leading experience in the discovery, development, manufacturing and commercialization of gene therapy technologies to treat severe genetic diseases in orphan ophthalmology. We believe that by leveraging our team s combined expertise, we will facilitate the rapid clinical advancement and regulatory approval of our product candidates.

Extend our expertise in AAV vector selection and design, delivery and manufacturing. We believe that our understanding of our target indications and our robust internal expertise in viral vector selection and design, physical vector delivery, vector manufacturing, clinical trial design and clinical trial conduct are significant competitive advantages. We intend to continue to devote substantial resources to developing the science underlying successful AAV vector design and delivery, as well as to expanding the capabilities of our reproducible, scalable manufacturing process. We believe these investments will facilitate the rapid advancement of our products through regulatory approval and enhance the commercial and therapeutic potential of our gene therapy platform.

Pursue orphan indications with high unmet medical need and greater probability of clinical, regulatory and commercial success. We focus on diseases for which the underlying genetic defect is well-characterized and can be addressed by correcting or inserting a single gene, for which predictive animal models exist and for which clinical endpoints are objective and have been validated by the FDA. We believe that our focus on these types of indications will enable us to obtain data more rapidly and accelerate the process of clinical trial and regulatory approval of our products. Given the relatively low prevalence of the orphan diseases we are pursuing and the strong key opinion leader communities and patient advocacy groups around them, we also believe we will be able to serve these markets independently with a small, targeted commercial infrastructure.

Develop and partner selectively to expand the scope of our pipeline and the utilization of our gene therapy platform. The adaptability of our platform also presents an opportunity for us to selectively form collaborative alliances to expand our capabilities and product offerings into a range of genetic diseases and potentially to accelerate the development and commercialization of gene therapy products more broadly. One such alliance led to the preclinical development and eventual license to Genzyme Corporation, or Genzyme, of a product candidate for wet AMD. We are also continuing clinical trials of our treatment for the inherited orphan lung disease AAT deficiency. We continue to evaluate similar opportunities to extend the commercial application of our gene therapy platform.

Gene therapy background

Genes enable production of proteins that perform a vast array of functions within all living organisms. Many diseases have a genetic aspect whereby a mutated gene is passed down from generation to generation. Mutated genes can cause production of abnormal proteins, which can cause disease.

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Gene therapy involves the introduction of a functional copy of the gene into a patient s own cells using a delivery system most commonly based on a viral vector to treat the genetic defect. Gene therapy has the potential to change the way these patients are treated, by correcting the underlying genetic defect that is the cause of their disease rather than offering treatments that only address symptoms. We believe that by correcting the underlying genetic defect, gene therapy can provide transformative disease modifying effects potentially with life-long clinical benefits based on a one-time therapeutic administration.

The promise of gene therapy has evolved over the last decade, with a growing body of clinical data that we believe has provided evidence of efficacy and safety in a variety of disease areas, improvements in vector design and manufacturing processes by us and others and the establishment of regulatory guidelines for the development and approval of gene therapy products. These advances have led to increased investment from the biopharmaceutical industry and supported the emergence of gene therapy as an important therapeutic modality for patients with significant unmet medical needs.

Our gene therapy platform

Our approach to gene therapy product development is conceptually straightforward. We design an AAV vector that will carry the functional gene necessary to express the desired protein, produce the vector using our proprietary production methods, and then deliver the product directly to the appropriate cells in a patient by a suitable physical delivery method. Although the concept of gene transfer is simple, the process of developing and manufacturing AAV vectors capable of delivering the genetic material safely into a patient s own cells is highly technical and demands significant expertise, experience and know-how.

Our gene therapy platform is built on our core competencies in three key areas:

- vector selection and design;
- vector manufacturing; and
- vector delivery.

Our vector selection and design process

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

Simplicity AAV is a small, simple non-enveloped virus with only two native genes. This makes the virus straightforward to work with from a vector engineering standpoint.

Stability AAV is extremely stable: it is resistant to degradation by shear, solvents and enzymes, facilitating purification and final formulation. AAV stability could also enable development of a freeze-dried formulation, should this become necessary for larger markets where shipping and distribution of the current frozen formulation would be challenging.

Sustained expression Unlike vectors based on other viruses, our AAV vectors are capable of inserting the functional gene into the patient s cells as an extra-chromosomal episome, which is a stable, circular form of DNA in the nucleus of cells. Inserting the functional gene as an episome supports long-term production of the protein, leading to sustained therapeutic effect, without altering the patient s existing DNA. Sustained expression is a powerful advantage of using AAV as a vector: a one-time therapeutic administration of a functional gene into a cell can potentially support protein production for the life of the cell, which, in the cell types

we are currently focused on treating, may approximate the duration of the patient s lifetime.

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Safety We believe AAV vectors are the safest for use in human gene therapy. In contrast, clinical trials using other vectors, such as lentivirus, adenovirus and herpes virus, have reported serious adverse events. The safety advantages of AAV vectors include the following:

- AAV elicits a low immune response, reducing the risk of adverse inflammatory reactions. In contrast, trials with adenoviral vectors have reported severe inflammatory reactions.
- AAV vectors, while they provide sustained expression, do not alter the patient s existing DNA, and safety is therefore improved over vectors that alter the patient s DNA. Trials using early versions of lentiviral vectors, which insert genes directly into, and thereby alter, the patients DNA, resulted in several well-publicized adverse events, including reported cases of leukemia.
- AAV has never been linked to human disease, unlike most other viruses used as gene delivery vectors such as adenovirus, herpes virus and lentivirus.
- AAV vectors have no viral genes remaining, eliminating the possibility that any viral genes will cause an adverse event.

AAV vectors have been used in more than 100 human clinical trials, by us and others, with no serious adverse events traced to the use of AAV as the gene delivery vector. In our direct experience with human clinical trials for LCA2, AAT deficiency and wet AMD, over 100 patients were treated using AAV vectors, with no serious adverse events attributed to the vector. In a Phase 2 trial of our AAT deficiency product candidate, patients were treated with doses more than 1,000-fold higher than those planned for use in any of our ophthalmic indications, with no serious adverse events reported.

Carrying capacity AAV vectors have the capacity to carry therapeutic gene sequences up to 4,000 base pairs in length into a patient s cell. As more than 90% of human genes have coding sequences less than 3,000 base pairs in length, we expect to be able to pursue a wide variety of indications with our AAV vectors.

Vector design. After the selection of the vector type, there are many other critical factors to be considered when designing a gene therapy product. These include selecting the appropriate:

- therapeutic gene,
- promoter and related gene regulatory elements,
- AAV sequences needed to signal replication and packaging, and
- AAV capsid (the protein shell) in which these elements are packaged.

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The first step in vector design is to identify the therapeutic protein that we want the patient s own cells to produce, and then insert the gene that encodes that protein into an AAV vector. Production of the protein requires a promoter, which is a genetic element to drive expression. Certain promoters function well only in certain cell types, whereas other promoters function well in almost any cell type. We make our selection by comparing different promoters in the specific type of cells that are affected in each disease target, ideally in an animal whose physiology is close to that of humans, to find the promoter that best enables production of therapeutic levels of protein in that cell type.

After the promoter and gene of interest are selected, we insert these elements between AAV viral sequences that are needed for replication and packaging of the vector into the AAV capsid. There are hundreds of variations of AAV capsids with different efficiencies in their ability to bind to and enter varying cell types. We select the capsid for a specific product candidate after comparing different capsids in the type of cells that are affected by the targeted disease.

One of our key capabilities is our depth of understanding of the complex interplay between the clinical disease, the cells in the patient s body that need treatment, the selection of a capsid and a promoter, the design of the gene construct and the physical administration method. We have spent years conducting research on the best combinations of these elements with the aim of developing safe and effective gene therapy treatments.

Vector manufacturing: our HAVE method

We have developed a proprietary, high-yield vector manufacturing process using scalable technologies for herpes-assisted vector expansion, which we refer to as our HAVE manufacturing method. While the HAVE manufacturing method uses the herpes virus as a helper in the first step of a four-step AAV vector manufacturing process, there is no herpes virus in the final product. Our HAVE manufacturing method addresses problems of low productivity and low efficacy that have historically plagued efforts to manufacture AAV vectors and enables us to produce vectors with improved potency, efficiency and safety over processes previously used by us and others. It also enables us to produce a more purified and concentrated end product, as evidenced by an approximately 25- to 30-fold reduction in non-infectious viral contaminants as compared to vectors used in previous clinical trials.

Our manufacturing process has been reviewed by both the FDA and the European Medicines Agency, or EMA, and has been authorized for production of product candidates for use in clinical trials in the United States and Europe. Our manufacturing process is also reproducible and scalable. It has been transferred successfully to Genzyme and to SAFC Pharma, our contract manufacturing organization, where it is used in manufacturing clinical materials pursuant to the FDA s current good manufacturing practices, or GMP, requirements.

We and SAFC Pharma have successfully produced the necessary material for the clinical trials we have conducted to date, and have more than enough manufacturing capacity to meet the requirements of our planned future trials. We are currently investing in the development of mid- to large-scale manufacturing processes with a view towards supporting our product candidates, if approved, at commercial scale.

We hold or have licensed 79 issued and 28 pending patents covering our manufacturing technology. We believe that our core competency and intellectual property estate in vector manufacturing differentiate us competitively and provide a key element of our gene therapy platform.

Vector delivery

Our gene therapy platform allows for vector delivery by a variety of methods, and we select the method that is most beneficial for the disease we are targeting. The method used depends on the type of cells we are targeting for treatment.

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In ophthalmology, the product candidate can best be delivered to cells in the eye by intravitreal or subretinal injection.

Intravitreal injection into the vitreous humor, which is the clear gel that fills the space between the lens and the retina of the eye, is best for delivering the product candidate to the retinal neurons in the inner retina (the portion of the retina closest to the lens), to photoreceptors located in the fovea (the very center of the macula, which is the central part of the retina that is required for fine visual acuity), and other cells in the lateral portions of the eye. This routine procedure can be carried out in an ophthalmologist s office.

Subretinal injection between the photoreceptors in the outer retina and the retinal pigment epithelium just beyond the retina are best for delivering the product candidate to the outer retina, farthest from the lens, where the AAV vector can readily enter photoreceptor cells and retinal pigment epithelium cells. This is a short, outpatient surgical procedure that is frequently performed by retinal surgeons.

We expect to use intravitreal injection as the method of delivery for our XLRS product candidate, and we plan to evaluate both subretinal injection and intravitreal injection as methods of delivery for our ACHM and XLRP product candidates.

For other indications, such as the orphan lung disease AAT deficiency, where secretion of a therapeutic protein into the bloodstream is the goal, we plan to administer the product candidate to muscle cells. There are large numbers of muscle cells in the body, providing the ability to produce a large amount of protein for systemic circulation. This can be accomplished by several methods, including:

intramuscular injection, in which the product candidate is directly injected into muscle cells, and

vascular delivery, in which the product candidate is administered to the muscle cells of an entire leg, using infusion methods similar to those currently employed in cardiac catheterization, oncology and

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anesthesiology. In preclinical animal studies of our product candidate for AAT deficiency, using a vascular delivery method was shown to achieve much higher serum levels and lower immune responses compared to direct intramuscular injection.

These methods of administration of our product candidates are well-established for the safe and effective delivery of other drugs and protein products. AAV vectors can be delivered by these and other methods to a wide array of other cells, such as heart muscle cells in certain cardiac diseases or directly into the brain in certain neurologic diseases.

Our approach can potentially arrest, correct or treat a disease with a one-time therapeutic administration, as many of the cells to which the product candidate is delivered will survive for the life of the patient and treatment of those cells thereby has the potential to deliver life-long effects. For example, cells in the retina, important in XLRS and ACHM, mature shortly after birth and in the absence of disease exist unchanged for the life of the patient. Once treated with our gene therapy products, these cells have the potential to express the therapeutic protein for the remaining life of the cell. This approach potentially provides significant value to patients, families, providers and payors.

Our product programs

Our lead programs address XLRS and ACHM, which are orphan diseases of the eye that are caused by mutations in single genes, significantly affect visual function starting at birth and currently lack effective medical treatments. We are also pursuing early stage preclinical research in treating other orphan eye diseases, such as XLRP.

We initially developed our gene therapy platform and obtained evidence of its safety and efficacy in proof-of-concept programs involving two other eye diseases: LCA2 and wet AMD. In 2010, we licensed our wet AMD technology to Genzyme. Genzyme recently informed us that it no longer intends to use our manufacturing technology to produce the AAV vector being used for the wet AMD product. We currently do not expect to independently commercialize our LCA2 proof-of-concept program.

We are also developing a product candidate for treatment of the inherited orphan lung disease AAT deficiency for which we have conducted preclinical proof-of-concept studies and Phase 1 and Phase 2 clinical trials. We believe our AAT deficiency program provides proof of concept for the use of our gene therapy platform in indications outside our focus area of orphan ophthalmology.

Our proof-of-concept programs in ophthalmology

The programs highlighted below, while not the principal focus of our current efforts, are critical to those efforts in that they establish initial evidence of safety and potential efficacy of our gene therapy approach in preclinical studies and clinical trials. These programs enabled us to develop significant experience working with clinical trial design and conduct, clinical investigators and regulatory agencies and in vector design, delivery and manufacturing. They also demonstrate that our manufacturing platform has been successfully vetted by regulatory agencies and partners and has been able to produce clinical material for multiple trials.

Leber congenital amaurosis

Leber congenital amaurosis, or LCA, is a form of early onset, inherited retinal degeneration caused by mutations in any one of 16 genes involved in retinal function and leads to blindness at birth or in early childhood or adolescence. Studies by Dr. Edward Stone published in the *American Journal of Ophthalmology* (2007) indicate the overall prevalence of LCA is one in 81,000 people, which implies there are about 3,700 cases of LCA in the United States and about 6,200 cases of LCA in Europe.

One form of LCA, referred to as LCA2, is caused by mutations in the RPE65 gene. RPE65 protein is an enzyme that is critical for normal phototransduction, the process whereby a light signal is converted to an electrical signal transmitted to the brain. A review paper by den Hollander, published in *Progress in Retinal and*

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Eye Research (2008), reported that mutations in the RPE65 gene are responsible for about 6% of all cases of LCA, from which we estimate that there are approximately 600 LCA2 patients in the United States and Europe, combined.

In preclinical studies, our LCA2 product candidate was evaluated for efficacy in mouse and dog models of LCA2 caused by mutations in the RPE65 gene. Restoration of visual function in mice and dogs was demonstrated by behavioral testing and electroretinogram, or ERG, testing, which measures electrical signaling in different cells of the retina.

The figure below shows ERG responses to flashes of light of increasing intensity, from dim (-2.6 log units) to very bright (2.8 log units) in a normal animal (left) or in a dog with RPE65 mutations before treatment and at three months and one, two and three years after a one-time therapeutic subretinal injection of our LCA2 product candidate. After treatment, the ERG responses of treated dogs recovered to nearly normal levels within three months and remained there for the three-year duration of the study. Though not illustrated below, follow-up ERG testing has shown that the improvement in ERG responses has been sustained in these animals for 10 years after treatment.

Based on data from Acland et al., *Molecular Therapy* (2005)

Our LCA2 product candidate was also evaluated in single-dose toxicology studies in dogs and monkeys, with no systemic toxicity after subretinal injection. The ocular changes that were observed were consistent with the expected effects of subretinal surgery, were not vector dose-dependent and resolved during the three-month study.

We have made the following progress in clinical development of our LCA2 program:

- our product candidate was granted an orphan drug designation by the FDA for the treatment of LCA2 caused by RPE65 mutations;
- we received a $1.1 million grant from the FDA to conduct a Phase 1/2 clinical trial;
- the NIH Recombinant DNA Advisory Committee, or the NIH RAC, reviewed our draft protocols for the Phase 1/2 clinical trial and its recommendations were incorporated into the final protocol and informed consent documents;
- we had a type B pre-IND meeting with the FDA in 2008, during which the FDA provided guidance on the manufacturing, nonclinical and clinical development of our LCA2 product candidate; and
- we submitted an IND in 2008 and have completed enrollment of a Phase 1/2 clinical trial in 12 patients affected by LCA2. Long-term follow-up is ongoing.

Results of our Phase 1/2 trial and other studies with the same or similar AAV vectors have demonstrated improvement in one or more measurements of visual function in almost all human patients tested and there has been no evidence of safety issues.

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The figure below shows a hill of vision map of the retina for both eyes of a patient one year after receiving a subretinal injection of our LCA2 product candidate in one eye. The map represents the sensitivity of cone photoreceptors to light stimulation, from black (minimal sensitivity) to white (moderate sensitivity). Before treatment, both eyes had a hill of vision restricted to the fovea. One year after treatment, the treated eye had a new hill of vision with dramatically increased cone photoreceptor sensitivity in the area of the retina where the subretinal injection was administered. In fact, light sensitivity is now greater in the treated area than in the fovea of this patient.

Based on data from Cideciyan et al., *New England Journal of Medicine* (2009)

The figure below shows visual fields of a human patient before (left) or two years after (right) one-time therapeutic treatment with our LCA2 product candidate in the left eye. The scotoma, or blind spot, illustrated by the dark spot in the middle of the eye, that was present before treatment disappeared after treatment of the left eye:

Based on unpublished data from AGTC Phase 1/2 clinical trial

We expect to receive additional two-year follow-up data from these studies in late 2014. At the present time we do not plan to conduct additional clinical trials with this product candidate, as we believe the small number of persons affected by the RPE65 form of LCA2, which we estimate at approximately 600 in the United States and Europe combined, are being adequately served by ongoing and planned clinical trials conducted by multiple academic research centers in the United States and several European countries.

Wet age-related macular degeneration

Age-related macular degeneration, or AMD, is a retinal disease that usually affects older adults and results in a loss of vision in the center of the visual field (the macula). It is a major cause of blindness and visual impairment in older adults and occurs in dry and neovascular, or wet, forms. In the wet form, abnormal growth of blood vessels in the retina is stimulated by a protein called vascular endothelial growth factor, or VEGF. The abnormal blood vessel growth, or neovascularization, causes vision loss due to blood and protein

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leakage below the macula. A paper by Friedman et al. published in *Archives of Ophthalmology* (2004) estimated the total number of persons with wet AMD in the United States is about 1.2 million, from which we estimate there are about 3.2 million persons with wet AMD in the United States and Europe combined.

If left untreated, bleeding, leaking and scarring from these blood vessels eventually cause irreversible damage to the photoreceptors and rapid vision loss. Treatment through intravitreal injection with drugs that inhibit VEGF can cause regression of the abnormal blood vessels and improve vision when injected directly into the vitreous humor of the eye. However, the injections must be repeated monthly or bimonthly. The approach to treatment of wet AMD that we licensed to Genzyme used an AAV vector to insert into the patient s own retinal cells a gene, called sFLT01, that encodes an engineered version of the receptor to which VEGF binds, and these cells then provide sustained production of the VEGF-inhibiting sFLT01 protein.

In preclinical studies, the wet AMD product candidate was evaluated in animal models of retinal neovascular diseases, used for testing products that inhibit VEGF, and for safety in rats and nonhuman primates. After intravitreal injection of the wet AMD product candidate, long-term expression of the engineered sFLT01 protein was demonstrated in both mice and monkeys. In the monkey disease model, the wet AMD product candidate resolved the neovascularization, with efficacy results similar to those shown for currently marketed anti-VEGF agents.

The figure below shows retinal photographs in a monkey that received an intravitreal injection of the wet AMD product candidate in one eye and later received nine laser-induced neovascular lesions in each eye followed by injection of a dye used to determine the amount of leakage from retinal blood vessels. The figure shows the marked reduction in leakage, indicated by white patches around a central dark spot, from the lesions in the treated eye (left) compared to the untreated eye (right).

Based on data from Lukason et al., *Molecular Therapy* (2011)

In 2010, we announced the exclusive license of the jointly developed program in wet AMD to Genzyme. The following progress has been made in clinical development of the wet AMD product candidate:

- we had a type B pre-IND meeting with the FDA during which meeting the FDA provided guidance on the manufacturing, nonclinical and clinical development of the wet AMD product candidate;
- the NIH RAC reviewed draft protocols for the Phase 1 clinical trial and its recommendations were incorporated into the final protocol and informed consent; and
- Genzyme submitted an IND and is conducting a Phase 1 clinical trial under this IND. The trial began in 2010, is fully enrolled, and is scheduled to complete the 1-year follow-up evaluations for the last patient in July 2014.

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Genzyme recently informed us that it no longer intends to use our HSV-based manufacturing technology to produce the AAV vector being used for the wet AMD product. Genzyme will be responsible for all future clinical trials and commercialization of its wet AMD product candidate.

Our proof-of-concept programs beyond ophthalmology

In one of our first proof-of-concept programs, we developed a product candidate for the treatment of AAT deficiency, which is an inherited orphan lung disease. We are continuing clinical trials of a vascular method for delivering our AAT deficiency product candidate to muscle cells, and expect to submit an amendment to our existing IND to allow us to conduct a Phase 2b clinical trial in early 2015. For more information about this program, see Proof-of-concept programs beyond ophthalmology; our Alpha-1 antitrypsin deficiency product candidate.

Our lead programs

X-linked retinoschisis

XLRS is an inherited retinal disease caused by mutations in the RS1 gene, which is located on the X chromosome and encodes the retinoschisin, or RS1, protein. Retinoschisin is expressed and secreted primarily from photoreceptor cells and binds strongly and specifically to the surface of photoreceptor and bipolar cells in the retina. Mutated forms of retinoschisin are unable to bind properly, resulting in schisis, or splitting of the nerve fiber layers of the retina, primarily in the macula. The disease begins early in childhood, and affected boys typically have best-corrected visual acuity of 20/60 to 20/120 at initial diagnosis. Complications such as retinal hemorrhage or retinal detachment occur in up to 40% of patients, especially in older patients. According to *Molecular Genetics of Inherited Eye Diseases* (1988), the incidence rate for XLRS is between one in 5,000 and one in 20,000 males. Using an incidence rate of 1 in 11,500 and assuming half the population is male, we estimate that there are about 13,000 persons in the United States and about 22,000 persons in Europe with XLRS, or 35,000 persons in the United States and Europe combined.

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

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The figure below shows an OCT image from a normal individual (top) and from a patient with XLRS (bottom). The black spaces indicated by the arrows in the bottom portion of the figure demonstrate splitting of the layers of the retina leaving spaces that interfere with the movement of electrical signals.

There is currently no approved treatment for XLRS. Management of disease manifestations includes low vision aids such as large-print textbooks, preferential seating in the front of the classroom and use of handouts with high contrast. Surgery may be required to address complications of vitreous hemorrhage or full-thickness retinal detachment. Anecdotal reports suggest that topical carbonic anhydrase inhibitors may provide some reduction in the degree of schisis detected by OCT and improvement in visual acuity in some but not all patients, but the absence of controlled clinical trials makes interpretation of these reports difficult. In addition, treatment with carbonic anhydrase inhibitors does not address the fundamental genetic defect in persons affected by XLRS. Neither carbonic anhydrase inhibitors nor any other medicinal products have been approved by regulatory agencies for treatment of XLRS.

Our XLRS product candidate

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

After the vector containing a functional copy of the RS1 gene enters a retinal cell, the gene is processed by normal biochemical processes into a stable DNA episome in the nucleus of the cell. This stable form of the gene allows production of the normal retinoschisin protein which is then secreted from the retinal cells and binds to the surfaces of photoreceptor and bipolar cells in the retina, pulling them together and eliminating any splitting between the layers of the cells. Upon light stimulation of the photoreceptor cells, the presence of the retinoschisin allows normal transmission of electrical signals from the photoreceptor cells to the bipolar cells and then to other retinal neurons that transmit the signals to the visual cortex in the brain. Production of normal retinoschisin continues as long as the episome persists in the cell, which may be for many years or even life-long, thereby providing long-term potential benefit after a one-time therapeutic administration.

Preclinical proof of concept for our XLRS product candidate

In mouse models of XLRS, our gene therapy approach restores to normal the abnormal ERG characteristic that is present in XLRS. Mouse models of XLRS have been developed by deactivating, or knocking out, the RS1 gene in mice. These knockout mice have clinical features similar to humans with XLRS, including reduced visual acuity, schisis cavities detected by OCT, and a markedly abnormal ERG response.

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The figure below shows staining for retinoschisin (top row) and for nuclei in retinal cells (bottom row) in a normal mouse (left), a RS1 knockout mouse in the absence of treatment (middle) and a RS1 knockout mouse treated with an AAV-RS1 vector (right). The knockout mouse retina has no expression of retinoschisin and has splitting and disorganization of the layers of the retina, indicated by the arrowheads in the middle panel of the nuclear staining. After treatment, RS1 staining is present in a normal fashion and the nuclear staining shows restoration of the organization of the cell layers in the retina (right).

Based on data from Min et al. *Molecular Therapy* (2005)

Treatment by injection of an AAV vector expressing either mouse or human RS1 in these knockout mice improved visual function as measured by increased ERG b-wave responses.

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The figure below shows improved ERG responses in RS1 knockout mice at various times after treatment with an AAV-RS1 vector compared to ERG responses in untreated control RS1 knockout mice. The figure shows a progressive decrease in the ERG response in the untreated mice but a slower decrease and eventual increase in the ERG response in the treated mice.

Based on data from Min et al. *Molecular Therapy* (2005)

We have concluded that intravitreal injection is the preferred route of administration for an AAV-RS1 vector. We therefore evaluated intravitreal injection of an AAV vector expressing a marker protein packaged in several different AAV capsids in monkeys and demonstrated that a vector packaged in an engineered capsid was able to target expression to the macula, which is the primary area in which retinoschisis occurs.

The figure below shows expression of a marker protein (white areas) in the macula, fovea and nerve fibers of a monkey retina after intravitreal injection of a vector contained in the engineered capsid. We believe that intravitreal injection of a vector containing the RS1 gene in the same engineered capsid would show expression of retinoschisin in the same areas.

Based on AGTC animal study data

We are currently conducting additional preclinical studies of our XLRS product candidate that are required for submission of an IND to the FDA. These studies include single-dose toxicology studies in mice and

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nonhuman primates, the design of which is based on specific guidance from the FDA s Office of Cellular, Tissue and Gene Therapy received in early 2013. These studies will evaluate the safety and distribution of the AAV-RS1 vector in animals after the product candidate is delivered by intravitreal injection.

Planned clinical development of our XLRS product candidate

We are currently conducting a natural history study in persons affected by XLRS. This study will document the progression of the disease in the absence of treatment, and its results will provide important information about the best methods for measuring visual function in these patients and will guide us in the design of subsequent clinical trials in which our product candidate will be tested for safety and efficacy. The study is being conducted at three clinical sites that specialize in inherited retinal diseases: the Casey Eye Institute in Portland, Oregon, the Retina Foundation of the Southwest in Dallas, Texas, and the Kellogg Eye Center in Ann Arbor, Michigan.

In late 2014, we plan to submit an IND and to initiate a Phase 1/2 clinical trial of our XLRS product candidate in up to 15 patients affected by XLRS. Results of this trial, which we expect to receive in mid-2015, will guide us in finalizing the design of a pivotal Phase 3 clinical trial. In the planned pivotal Phase 3 trial, up to 40 patients will be enrolled and evaluated for changes in visual function over a 12-month period. If successful, we believe the results of this second trial could support submission of a Biologics License Application, or BLA, to the FDA in the United States and a Marketing Authorization Application, or MAA, to the EMA in Europe for our XLRS product candidate.

Congenital achromatopsia

ACHM is an inherited retinal disease characterized by the lack of cone photoreceptor function. Cone photoreceptors are concentrated in the macula and the fovea. ACHM is present from birth and throughout life. Individuals with this condition have no cone photoreceptor function, markedly reduced visual acuity, photophobia, or light sensitivity, and complete loss of color discrimination. Their only functioning photoreceptors are rod photoreceptors, which respond to low intensity light conditions and mediate night vision but cannot achieve fine visual acuity. Best-corrected visual acuity in persons affected by ACHM, even under subdued light conditions, is usually about 20/200, a level at which people are considered legally blind. They also experience extreme light sensitivity resulting in even worse visual acuity under normal daylight conditions, or day blindness.

ACHM can be caused by mutations in any of at least five genes that are required for normal cone photoreceptor function. The most common causes are mutations in the CNGB3 gene (about half of all cases) or CNGA3 gene (about one-fourth of all cases). These genes encode the CNGB3 and CNGA3 proteins, which combine to form a channel in the photoreceptor membrane that is required for phototransduction, the process whereby a light signal is converted to an electrical signal that is then transmitted to the brain. According to *Retinal Dystrophies and Degenerations* (1988), the incidence rate for ACHM is approximately one in 30,000 people, and we therefore estimate that there are about 10,000 people in the United States and about 17,000 people in Europe with ACHM. Of these, about half, or a total of 13,500 in the United States and Europe combined, have the form of the disease caused by mutations in the CNGB3 gene.

There is currently no specific treatment for ACHM. Symptoms are managed by the use of dark lenses to reduce discomfort from ambient light, and low vision aids such as high-powered magnifiers for reading. Children with ACHM are provided preferential seating in the front of classrooms to benefit maximally from their magnifying devices.

Our ACHM product candidate

Our gene therapy approach to treatment of ACHM involves using an AAV vector to insert a functional copy of the CNGB3 gene into the patient s own photoreceptor cells. Our ACHM product candidate contains the

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CNGB3 gene and a promoter that has been shown in animal models to work well in cone photoreceptors and is packaged into an AAV capsid that is able to efficiently enter cone photoreceptors. We have also recently completed a study in non-human primates that identified a promoter, the PR1.7 promoter, that we believe works well in primate cone receptors. We are designing our product candidate to be delivered either by intravitreal or subretinal injection.

After our ACHM product candidate containing the functional CNGB3 gene enters a photoreceptor cell, the gene is processed by normal biochemical processes into a stable DNA episome in the nucleus of the cell. The stable form of the gene allows production of the normal CNGB3 protein, which combines with the normal CNGA3 protein already being produced in the cell, to form a channel in the photoreceptor membrane that is required for phototransduction. Restoration of phototransduction enables cone photoreceptors to convert light entering the eye into an electrical signal that is transmitted to other retinal neurons and then to the visual cortex in the brain. Production of normal CNGB3 protein continues as long as the episome persists in the cell, which may be for many years or even life-long, thereby providing long-term potential benefit after a one-time therapeutic administration.

Preclinical proof of concept for our ACHM product candidate

In mouse and dog models of ACHM, our product candidate was able to restore photoreceptor function, improve visual acuity and mitigate photophobia and day blindness.

ACHM occurs in two breeds of dogs, Alaskan malamutes and German shorthaired pointers, due to mutations in the CNGB3 gene that either produce an abnormal protein or completely prevent production of the protein. Both breeds have clinical characteristics similar to human ACHM patients, with day blindness and absence of retinal cone function as measured by ERG. Treatment by subretinal injection of an AAV vector expressing human CNGB3 restored cone function in dogs with either mutation. Cone-specific ERG responses were undetectable in these dogs before treatment but were clearly detected after treatment. Day blindness was demonstrated before treatment by testing the ability of the dogs to navigate a maze under progressively brighter conditions. Before treatment, it took the ACHM dogs progressively longer to navigate the maze as the ambient light increased from dim light to normal room lighting and even longer with normal outdoor daytime lighting. After treatment, the day blindness was substantially eliminated, and the treated ACHM dogs were able to navigate the maze under bright light conditions at almost the same speed as normal dogs.

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The figure below shows the average time taken to navigate a maze as the ambient light intensity was increased for three groups of dogs: normal dogs, dogs with ACHM that were untreated and dogs with ACHM that were treated with our ACHM product candidate. The figure shows that under low light conductions (0.2 lux, equivalent to the light conditions on a moonlit night), when vision is normally mediated only by rod photoreceptors, all three groups navigated the maze rapidly. As the light intensity was progressively increased (to 646 lux, equivalent to the light conditions in a business office), and vision became mediated by cone photoreceptors, the untreated ACHM dogs took progressively longer to navigate the maze, as they bumped into walls in the maze and had to advance by trial and error. In contrast, as the light intensity was progressively increased, the time taken to navigate the maze did not change for normal dogs and increased only slightly for the treated ACHM dogs.

Based on Komaromy et al. *Human Molecular Genetics* (2010)

Untreated ACHM dogs also demonstrated photophobia and day blindness when outdoors in daylight, which severely limited their ability to interact with people and objects in their environment. After treatment there was a dramatic improvement in this important clinical manifestation of ACHM. The restored function persisted for more than 2.5 years (the longest duration tested).

In addition, a mouse model of ACHM was developed by knocking out the CNGB3 gene in mice. These knockout mice have markedly impaired cone photoreceptor function, as measured by ERG and visual acuity testing. Treatment by subretinal injection of an AAV vector expressing human CNGB3 in the knockout mice improved cone-specific ERG responses to nearly normal levels and improved visual acuity, as measured by their ability to follow a rotating pattern of vertical stripes of varying thickness.

We are conducting additional preclinical studies required for submission of an IND to the FDA. This will include single-dose toxicology studies in mice and nonhuman primates, the design of which will be based on guidance from the FDA s Office of Cellular, Tissue and Gene Therapy in the form of a pre-pre IND meeting planned for mid-2014. These studies will evaluate the safety and distribution of our ACHM product candidate after delivery by both subretinal and intravitreal injection.

Planned clinical development of our ACHM product candidate

We are currently conducting a natural history study in persons affected by ACHM caused by CNGB3 mutations. Results of this study will provide important information about the best methods for measuring visual function in these patients and will guide us in the design of subsequent clinical trials in which our product

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candidate will be tested for safety and efficacy. This study is being conducted at five clinical sites that specialize in inherited retinal diseases: the Bascom Palmer Eye Institute in Miami, Florida, the Casey Eye Institute in Portland, Oregon, the Chicago Lighthouse in Chicago, Illinois, the Medical College of Wisconsin in Madison, Wisconsin and the University of Florida in Gainesville, Florida.

After completing the ongoing preclinical studies required for submission of an IND to the FDA, we plan in early 2015 to submit an IND and to initiate a Phase 1/2 clinical trial of our ACHM product candidate in up to 30 persons affected by ACHM caused by mutations in the CNGB3 gene. We will first test the safety and efficacy of the less invasive delivery method, intravitreal injection, and then move to subretinal delivery, if required. Results of this trial, which we expect to receive in late 2015, will guide us in finalizing the design of a pivotal Phase 3 clinical trial. In the planned pivotal Phase 3 trial, up to 40 patients will be enrolled and evaluated for changes in visual function over a 12-month period following treatment. If successful, we believe the results of this pivotal Phase 3 trial could support our submission of a BLA to the FDA and of an MAA to the EMA for our ACHM product candidate.

Additional opportunities in ACHM

There are several other genes in which mutations are known to cause ACHM, with signs and symptoms that are the same as in ACHM caused by CNGB3 mutations. AAV vectors expressing these genes would be additional potential product candidates for treatment of ACHM caused by mutations in these genes, and we believe they would have the potential for rapid regulatory approval, if our product candidate for ACHM caused by CNGB3 mutations were already approved.

X-linked retinitis pigmentosa

Retinitis pigmentosa is an inherited retinal dystrophy with progressive loss of vision. It is commonly first observed in young men who notice problems with vision under low light conditions, or night blindness, followed by a restriction of peripheral visual fields, or tunnel vision, leading to poor central vision and eventual total blindness.

The incidence rate for retinitis pigmentosa is about one in 4,000 people, according to *Retinitis Pigmentosa* (1988), and we estimate that there are about 75,000 people in the United States and 125,000 people in Europe with retinitis pigmentosa, or 200,000 people in the United States and Europe combined. According to a paper by Dr. Marianne Haim published in *Acta Ophthalmologica* (1992), about 10% of cases of retinitis pigmentosa are caused by mutations in a gene on the X chromosome and are referred to as X-linked retinitis pigmentosa, or XLRP, from which we therefore estimate that there are about 20,000 persons with XLRP in the United States and Europe combined.

A preclinical study in a dog model of XLRP caused by mutations in the RPGR gene demonstrated a delay in the rate of disease progression in eyes that received a subretinal injection of an AAV vector expressing RPGR. We are currently designing preclinical studies to further evaluate the ability of our XLRP product candidate to delay disease progression in animal models of XLRP. If these studies are successful, we will conduct additional preclinical studies required for submission of an IND to the FDA. These studies will include single-dose toxicology studies in animals that will evaluate the safety and distribution within the animals after our XLRP product candidate is delivered by both subretinal and intravitreal injection.

Other opportunities in ophthalmology

We believe our current gene therapy platform will enable us to develop and test new AAV vectors that carry different gene sequences for other inherited diseases in ophthalmology, reducing the need for early research work. In this way, we anticipate being able to move products rapidly through preclinical studies and into clinical development. We also believe that there are large market ophthalmology diseases where AAV vectors may provide benefit, such as wet AMD.

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Blue cone monochromacy and X-linked color blindness

Humans have three types of cone photoreceptors, termed L, M and S cones, which are responsive to light of long (red), medium (green) or short (blue) wavelength. The coordinated function of the three types of cone photoreceptors provides the ability to perceive the full range of colors in the visual spectrum.

Blue cone monochromacy, or BCM, is an inherited retinal disease characterized by lack of functional L and M cone photoreceptors but generally normal function of S cone photoreceptors. BCM is caused by mutations in the part of the X chromosome, termed the locus control region, which controls expression of the L and M opsin genes. In humans, the fovea contains only L and M cones and no S cones. The clinical manifestations are very similar to persons affected by ACHM, with markedly reduced best corrected visual acuity (about 20/200) and photophobia, but with incomplete loss of color discrimination.

Color vision deficiency, commonly called color blindness, is the inability or decreased ability to perceive the full spectrum of color differences. The condition is usually X-linked, due to mutations in the L or M opsin gene, resulting in either a missing or abnormal L or M opsin protein. These individuals are said to have red-green color blindness and they cannot see pure red colors, which instead appear black, while purple cannot be distinguished from blue and all orange-yellow-green shades appear as yellow. Individuals with color vision deficiency are not blind; their best-corrected visual acuity is usually normal (20/20).

According to a review article by Dr. Matthew Simunovic published in the journal *Eye* (2010), X-linked color blindness affects a large number of individuals, as many as 8% of men and 0.5% of women, but BCM is a rare disease, with an incidence rate of approximately one in 100,000 males, from which we estimate that there are about 1,500 persons in the United States and about 2,500 persons in Europe with BCM.

There are currently no specific treatments for BCM or X-linked color blindness.

We are currently designing preclinical studies to evaluate the ability of our gene therapy approach to correct the visual abnormalities in animal models of BCM. We are also designing studies in which people with X-linked color blindness will be asked to complete a questionnaire to determine the impact of their color vision deficiency on their lives and whether they would be interested in having their color vision deficiency treated if an AAV gene therapy product were available. Results of these studies will help us to determine whether to conduct clinical trials of product candidates for these conditions.

Optogenetics

There are a variety of progressive retinal diseases that ultimately result in advanced retinal degeneration and blindness, including retinitis pigmentosa, AMD and diabetic retinopathy. We and others are developing products to treat these diseases before they progress to blindness, but many patients will have advanced retinal degeneration despite treatment with available therapies.

One approach to treatment of advanced retinal degeneration, in which photoreceptors are no longer functional and able to process new genetic information delivered by gene therapy, is to bypass the photoreceptors and deliver a light-sensitive protein to neurons in the retina. One such light-sensitive protein is channelrhodopsin 2, or ChR2, a protein that controls photosynthesis in green algae. When ChR2 is inserted into a neuron and the neuron is stimulated by light, the neuron is activated and is able to transmit a signal to the visual cortex. This technique is referred to as optogenetics the combination of techniques from optics and genetics to control individual neuron activity in living tissue. We are working with others who hold key gene intellectual property in the optogenetics field to develop an AAV vector for treatment of advanced retinal degeneration.

Proof-of-concept programs beyond ophthalmology; our Alpha-1 antitrypsin deficiency product candidate

We also plan to pursue gene therapy programs that target muscle cells via direct intramuscular injections or vascular delivery, to leverage the unique properties of AAV vectors. For example, in one of our first proof-of-

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concept programs, we have developed a product candidate for the treatment of AAT deficiency, which is characterized by reduced serum levels of AAT protein and increased risk of developing emphysema and liver disease. AAT normally functions to prevent lung tissue damage.

AAT deficiency is implicated in 2.7% of all deaths due to obstructive pulmonary disease among persons in the 35-44 year-old age group, and emphysema is the most common cause of death in AAT-deficient patients, accounting for about 72% of cases. According to the National Institutes of Health Genetics Home Reference, the incidence rate for AAT deficiency is between one in 1,500 and one in 3,500 people of European ancestry, and an article by de Serres and Blanco in *Therapeutic Advances in Respiratory Disease* (2013) estimates that there are approximately 44,000 people in North America and 74,000 people in Northern and Central Europe with the most severe form of AAT deficiency, or about 118,000 people in the United States and Europe combined.

Prevention of lung disease in AAT deficiency is well-understood, since the presence of serum AAT levels of 11 μM or higher is considered to be an indicator of protection from tissue damage. AAT augmentation therapy, consisting of intravenous infusions of AAT protein purified from plasma obtained from healthy human donors, can achieve effective serum levels of AAT. However, the annual cost of augmentation therapy, administered by weekly intravenous infusions over the lifetime of the patient, can be more than $100,000 per year.

Our alternative, gene therapy approach involves using an AAV vector to insert a functional copy of the normal AAT gene into the patient s muscle cells. In preclinical studies, our AAT deficiency product candidate was evaluated in single-dose toxicology studies in mice and rabbits. These studies demonstrated that vector administration was not associated with clinical signs of toxicity and there were no adverse effects on hematology or serum chemistry parameters or gross pathology findings. We plan to perform an additional toxicology study in monkeys to evaluate administration of our AAT deficiency product candidate to muscle cells by a vascular route of delivery that in animals was able to achieve much higher serum levels compared to direct intramuscular delivery.

We have had extensive dialogue with the FDA, the EMA and other regulatory authorities and advisory bodies concerning the clinical advancement of our AAT deficiency product candidate. We have made the following progress in the clinical development of our AAT deficiency product candidate:

- our AAT deficiency product candidate was granted an orphan drug designation by the FDA and by the EMA for the treatment of AAT deficiency;
- we received a $1.1 million grant to conduct the Phase 2 trial from the FDA;
- we had a type B pre-IND meeting with the FDA in 2004, during which the FDA provided guidance on the manufacturing, nonclinical and clinical development of our AAT deficiency product candidate;
- the NIH RAC reviewed our draft protocols for the Phase 1 and Phase 2 clinical trials and its recommendations were incorporated into the final protocol and informed consent documents;
- we submitted our IND in 2005 and have conducted two clinical trials under this IND and no safety issues attributed to the vector have been seen;
- we received Scientific Advice from the EMA s Committee for Medicinal Products for Human Use, or CHMP, in 2010 related to the manufacturing, nonclinical and clinical development of our AAT deficiency product candidate; and
- we have had several type C meetings with the FDA focused on the manufacturing, nonclinical and clinical development of our AAT deficiency product candidate, most recently in June 2013.

Our AAT deficiency product candidate has been evaluated in two clinical trials in 18 patients with AAT deficiency. Both trials were designed to evaluate the product candidate s safety and ability to achieve sustained expression of normal AAT protein in the serum. In these trials, there were no serious adverse events attributed to

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administration of our product candidate. One patient developed bacterial epididymitis and one patient developed diverticulitis, each of which events was considered unrelated to our product candidate. In a Phase 2a trial, concentrations of normal AAT increased linearly in direct proportion to the dose and these AAT levels were sustained for more than two years.

The figure below left shows serum concentrations of normal AAT in subjects who received different doses of the AAT deficiency vector. There was a linear relationship between the increase in serum AAT concentration and the increase in vector dose. The figure below right shows average serum concentration of AAT over time in the group that received the highest vector dose. Serum AAT concentration increased within 30 days and remained significantly above baseline levels for more than two years.

The figure on the left is based on data published by Flotte et al. *Human Gene Therapy* (2011). The figure on the right is based on AGTC human clinical trial data.

Although we observed sustained expression of AAT for more than two years, the serum AAT concentrations were lower than the target of 11 μM that is necessary for adequate protection of the lungs. However, we have established that in animals, delivering AAV vectors to muscle cells using a vascular method can achieve much higher serum levels than when the vector is delivered by direct injection into muscles. We are currently conducting additional nonclinical studies of this new method for delivering our AAT deficiency product candidate to muscle cells, and will submit an amendment to our existing IND to allow us to initiate a Phase 2b trial in early 2015 in which our AAT deficiency product candidate will be administered in up to six patients with AAT deficiency using the vascular delivery method.

As we further develop this program, we will investigate the opportunity to expand to other indications where high levels of circulating proteins are important.

Manufacturing

Until recently, there has been a lack of manufacturing infrastructure to enable the production of gene therapies in a reliable and reproducible manner at a commercially viable scale. The historical challenges for gene therapy manufacturing relate to the difficulty of developing constructs that provide the necessary helper functions, and in having systems that provide adequate yield, scalability and potency. We have made significant investments in developing improved manufacturing processes, which include the following:

- We have developed proprietary AAV vector manufacturing processes and techniques that produce a more purified and concentrated product candidate, as evidenced by the approximately 25- to 30-fold reduction in non-infectious viral contaminants as compared to vectors used in many previous clinical trials.

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We do not need a specially cloned and isolated cell line for each of our disease targets; we instead use specially engineered replication-incompetent herpes simplex helpers, or HSV helpers, which are stable and straightforward to clone.

We have developed approximately 30 assays to accurately characterize our process and the AAV vectors we produce.

We have developed a purification system applicable to multiple AAV capsids.

We are investing in the development of mid- to large-scale manufacturing processes to enable the manufacture of our product candidates at commercial scale.

We believe these improvements and our continued investment in our manufacturing platform will enable us to develop best-in-class, next generation gene therapy products.

Our viral vector production platform for AAV-based gene therapeutics, which we call the herpes-assisted vector expansion, or HAVE method, offers significant benefits in comparison with the methods used by others to manufacture AAV vectors, as summarized in the following table.

AAV production method	Straightforward cloning	High efficiency	High yield	Scalable
Transfection	Yes	No	No	No
Baculovirus	No	No	Yes	Yes
Adenovirus	No	Yes	Yes	Yes
Our HAVE method	**Yes**	**Yes**	**Yes**	**Yes**

The four key steps involved in our proprietary HAVE manufacturing method are as follows:

First, the therapeutic gene and the appropriate AAV capsid genes are inserted into individual HSV helpers, and these helpers are individually grown in a complementing cell line called V27. The complementing cell line is required to provide critical functions that allow the replication-incompetent HSV helpers to grow; the same cell line is used to produce HSV helpers for all disease targets. This step occurs in disposable culture vessels of increasing size, up to and including disposable stirred tank bioreactors. The HSV helpers are harvested, minimally processed and concentrated to prepare them for use in producing our AAV vectors. These HSV helpers can be stored frozen for years before use.

Next, the two HSV helpers are used together to infect a cell line called sBHK, allowing for packaging of the therapeutic gene into the AAV capsid and to produce our AAV vectors. The sBHK cell line does not provide the critical functions that would allow for growth of the HSV helpers, which provides an added layer of safety. The same sBHK cell line is used to produce AAV vectors for all disease targets. This step occurs in disposable culture vessels of increasing size depending on the amount of AAV vector that is required. The AAV vector is recovered by using a detergent solution to break open the sBHK cells and release the AAV vectors. This step also destroys any residual HSV helpers that were used to infect the sBHK cells.

The third step is to purify the harvested AAV vector using two chromatography columns. The exact method used to column-purify our AAV vectors varies depending on the AAV capsid used in the product candidate; we have developed purification methods for multiple AAV capsids. We have shown in formal clearance studies that the combination of detergent treatment and two chromatography columns can remove up to 10^{14} (100 trillion) units of HSV. This step also helps to eliminate any remaining parts, such as proteins or DNA, of the HSV helpers and sBHK production cells.

The final step is to formulate, filter and fill the AAV vector in appropriate containers for use in animal or human studies. This filled AAV vector drug product can be stored frozen for years before use.

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HAVE Production of our AAV Vectors for Gene Therapy

The HAVE method is inherently flexible, allowing the manufacture of a wide range of AAV vectors without the need to modify the manufacturing steps used to produce the HSV helpers or AAV vectors. We have already demonstrated our manufacturing knowledge through multiple successful production batches of both HSV helpers and AAV vectors at SAFC Pharma, our contract manufacturing organization, under current good manufacturing practices, or GMP.

Research is already underway to meet our future manufacturing needs. Projects include scale-up to larger batch production for use in our AAT deficiency program, continued modifications of the purification step to accommodate new AAV capsids, complete removal of animal-derived products from the V27 cell growth step, and formulations that allow for higher AAV vector concentrations.

Strategic collaborations

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

Our license to Genzyme

In 2004, we entered into a collaboration agreement with Genzyme to develop a recombinant AAV product to treat wet AMD. Our agreement originally provided that the parties would share responsibility for planning, budgeting, workload, decision-making, costs and future revenues. The parties had joint ownership of any intellectual property that arose as a direct result of the work done for the partnership. In collaboration with Genzyme, early product development work, production of materials for animal studies, development of several manufacturing and clinical assays, completion of IND-enabling toxicology and biodistribution studies, technology transfer of our HSV-based manufacturing process to Genzyme, production of the AAV vector under GMP for the Phase 1 human clinical trial, and drafting of the IND were conducted.

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In early 2010, as the product candidate was moving into human clinical trials required for wet AMD, we renegotiated our agreement to take the form of a license of our HSV-based manufacturing technology and interest in the wet AMD program to Genzyme. The license provides for modest late-stage milestone payments to us and royalties on sales, as well as forgiveness of our share of development costs from mid-2006 to the date the license was signed. Genzyme is responsible for all further development and commercialization of the wet AMD product candidate. We maintain non-exclusive rights to jointly developed technology. Genzyme also has options, expiring in 2015 and 2017, to license our manufacturing technology, as it existed at the time of the license, for specified genes associated with diseases outside our current area of focus. Genzyme recently informed us that it no longer intends to use our HSV-based manufacturing technology to produce the AAV vector being used for the wet AMD product. Our license agreement with Genzyme was further amended in December 2013 to reflect this fact and, among other things, to terminate our exclusive license to Genzyme for use of our HSV-based manufacturing technology in wet AMD except as to specified pending research activities, and to eliminate restrictions on our activities in the field of treatments for ocular neovascularization disorders, including AMD.

We currently do not expect to derive substantial revenue from our license to Genzyme, but a successful outcome of the clinical trials for which Genzyme is responsible would contribute significantly to the perception and prospects of our gene therapy platform.

Our relationship with the University of Florida

All of our scientific founders spent part of their careers at the University of Florida, or UF, and three are still UF faculty members. Since our inception we have licensed significant technology from and funded research at multiple labs at UF. Pursuant to five agreements, we have licensed three U.S. patents and multiple pending applications covering inventions made at UF. UF has multiple capabilities in genetic cloning, gene therapy manufacturing, animal model development and facilities for both small and large animal testing, and in certain instances we have benefited from the ability to conduct important research at UF without having to expand in-house facilities and personnel. We interact frequently with all members of the Powell Gene Therapy Center at UF and have an excellent working relationship with the UF Office of Technology Licensing.

In May 2013, we and UF were jointly awarded an $8.3 million dollar grant from the NEI to support development of our ACHM product candidate, with Dr. William Hauswirth, one of our scientific founders and Professor and holder of the Rybaczki-Bullard Chair in the Department of Ophthalmology at UF, as principal investigator. As a sub-awardee, as of December 31, 2013, we had received $0.2 million and we expected to receive an additional $3.7 million over four years under this grant.

Our relationships with patient advocacy groups and academic centers

We have long believed that when developing products to treat orphan indications it is important to form strong relationships with patient advocacy groups, and we have done this successfully with both the Foundation Fighting Blindness, or FFB, and the Alpha-1 Foundation. Both organizations are well known for their advocacy of patients interests in obtaining diagnosis, developing treatments and providing for reimbursement. Both actively support research into treatment, and we have been awarded three research grants totaling $1.6 million from the FFB and one grant of $0.3 million from the Alpha-1 Foundation. More importantly, both organizations have been instrumental in assisting us in forming ties with disease experts, recruiting patients into clinical trials and helping us to understand the needs, wants and concerns of patients.

We also have formed strong relationships with key academic centers across the United States that have core competencies in gene therapy, orphan ophthalmology and AAT deficiency. These centers conduct sponsored research, act as advisors and collaborate with us on grant proposals. We have been awarded grant funding aggregating $10.6 million between our inception and December 31, 2013, either independently or with our collaborators. This funding provides peer-reviewed scientific validation of our programs and has facilitated critical early stage research for our leading product candidates.

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Intellectual property

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

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

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

As of February 28, 2014, our patent portfolio included approximately 53 patents and patent applications that we own and approximately 54 patents and patent applications that we have licensed. More specifically, we own five U.S. patents, five pending U.S. applications, 27 foreign patents and 16 foreign patent applications. We have licensed 21 U.S. patents, four pending U.S. applications, 26 foreign patents and three pending foreign patent applications.

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

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

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the date of filing the non-provisional application. In the United States, a patent s term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the United States Patent and Trademark Office in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent.

The term of a patent that covers an FDA-approved drug may also be eligible for patent term extension, which permits patent term restoration of a U.S. patent as compensation for the patent term lost during the FDA regulatory review process. The Hatch-Waxman Act permits a patent term extension of up to five years beyond

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the expiration of the patent. The length of the patent term extension is related to the length of time the drug is under regulatory review. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent per approved drug may be extended. Moreover, a patent can only be extended once, and thus, if a single patent is applicable to multiple products, it can only be extended based on one product. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug. When possible, depending upon the length of clinical trials and other factors involved in the filing of a new drug application, or NDA, we expect to apply for patent term extensions for patents covering our product candidates and their methods of use.

We may rely, in some circumstances, on trade secrets to protect our technology. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Our patents and patent applications.

Manufacturing. We own or in-license 34 patents and patent applications that cover methods to manufacture AAV vectors. More specifically, we have 7 U.S. patents and patent applications and 27 foreign patents and patent applications covering manufacturing methods. There are still patents pending in this group. The longest lived and most significant manufacturing patent is expected to expire in 2025.

Small Cone Promoters. We own or in-license 13 patent applications that cover small cone promoters and uses thereof. Of these 13 patent applications, four are U.S. patent applications and nine are foreign patent applications. There are still patents pending in this group. As these patent applications have been filed recently, no issued patents exist covering small cone promoters. A patent issuing from this group could have an expiration date of 2033.

The following table summarizes our material owned and in-licensed patents and patent applications that are practiced in the manufacture and use of our product candidates:

Description of Patent or Patent Application	Product Candidate
High Titer Recombinant AAV Production (1)	All of our product candidates
Recombinant Herpes Viruses for Preparing Recombinant Adeno-Associated Viruses (2)	All of our product candidates
Production Of AAV Using Cells In Suspension (3)	All of our product candidates
Use of HSV to Produce rAAV (4)	All of our product candidates
Use of HSV Variants to Produce AAV (5)	All of our product candidates
Tyrosine Modifications of AAV Capsids (6)	All of our product candidates
CNGB3 Gene (7)	Our ACHM product candidate
Expression Cassettes for Achromatopsia (8)	Our ACHM product candidate
Small Cone Promoter (9)	Our ACHM product candidate
Composition and Methods to Treat Alpha 1 (10)	Our AAT deficiency product candidate
Delivery of AAV to Muscle and Blood (11)	Our AAT deficiency product candidate
Pseudotypes and Other AAV Compositions (12)	Our AAT deficiency product candidate

(1) Includes one issued patent in the United States which is expected to expire in 2022 and issued foreign patents which are expected to expire in 2023 in Australia, New Zealand, Austria, Switzerland, Germany,

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Spain, France, the United Kingdom, Ireland, Italy, Luxembourg, Monaco, and The Netherlands, and pending patent applications in the United States and Canada.

(2) Includes issued patents, each of which is expected to expire in 2018, in Canada, France, Germany, Israel, Italy, the United Kingdom, and the United States, and one pending patent application in Japan.
(3) Includes pending patent applications in the United States, Australia, Canada and the European Patent Office.
(4) Includes issued patents, each of which is expected to expire in 2019, in Australia, New Zealand and the United States.
(5) Includes one issued United States patent, which is expected to expire in 2025.
(6) Includes one issued United States patent, which is expected to expire in 2029, and three pending United States patent applications.
(7) Includes two issued United States patents, one expected to expire in 2021 and the other expected to expire in 2022, and one issued patent in each of the following jurisdictions, all of which are expected to expire in 2021: Australia, Canada, Denmark, France and the United Kingdom. Also includes two pending patent applications in Japan.
(8) Includes three pending patent applications in the United States and one pending patent application in each of Australia, Canada, China, India, Japan, New Zealand, and the European Patent Office.
(9) Includes one pending multijurisdictional patent application filed pursuant to the Patent Cooperation Treaty.
(10) Includes issued patents, each of which is expected to expire in 2019, in Belgium, Ireland, Monaco, Greece, Cyprus, Switzerland, Germany, Denmark, Spain, Finland, Italy, Luxembourg, New Zealand, Portugal, Sweden, Netherland, Hong Kong, the United States, Canada, Great Britain, Austria, France and the European Patent Office.
(11) Includes 11 issued United States patents and 1 issued Canadian patent, each of which is expected to expire in 2016, and one pending patent application in each of the United States and Canada.
(12) Includes four issued United States patents, three of which are expected to expire in 2019 and one of which is expected to expire in 2021.

License agreements

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

Information about our principal licenses is set forth below. The aggregate amount of all cash up-front payments that we have made pursuant to the license agreements described below is $0.2 million, all of which is included in our historical results of operations.

University of Florida. We currently have five license agreements with the University of Florida Research Foundation, or UFRF, an affiliate of UF:

A license from UFRF signed in September 2001 relates to the AAV construct containing the AAT gene and the method to treat AAT deficiency using this construct. We have an exclusive license in all fields of use.

Under the terms of this license, we made cash and stock-based up-front payments to UFRF and are required to make payments ranging from the mid-five figures to the low-six figures based upon development, regulatory and commercial milestones for any products covered by the in-licensed intellectual property. Assuming that we meet each of the specified development, regulatory and commercial milestones not more than once for each product, which we expect will be the case, the maximum aggregate milestone payments payable under this license with respect to any individual product that we commercialize will be $0.3 million. We will also be required to pay a royalty on net sale of products covered by the in-licensed intellectual property in the mid-single digits. The royalty is subject to reduction for any third-party payments required to be made, with a minimum floor in the low single digits. We have the right to sublicense our rights under this agreement, and we will be required to

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pay a percentage of such license income in the low-double digits. We are required to make annual maintenance payments in the low four figures under this license, which payments are creditable against royalty payments on a year-by-year basis.

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

The longest-lived patent covered by this license is expected to expire in 2019.

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

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

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

The longest-lived patent covered by this license is expected to expire in 2018.

A license from UFRF signed in September 2012 relates to the use of a small cone cell specific promoter. We have an exclusive license in the field of ophthalmology. Currently this patent would be most useful for ACHM but could be important to treating any ophthalmic disease that involves cone cells.

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

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

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There are patent applications pending under this license.

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

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

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

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

University of Alabama at Birmingham. A license agreement from the UAB Research Foundation affiliated with The University of Alabama at Birmingham signed in 2006, relates to one U.S. patent with claims covering the use of HSV helpers to produce AAV vectors. The patent is expected to expire in 2025. We have a non-exclusive license in the field of human gene therapy using AAV vectors.

Under the terms of this license, we made a cash up-front payment to the UAB Research Foundation, and we will be required to make payments ranging from the mid-five figures to the mid-seven figures based upon development and regulatory milestones for any products covered by the in-licensed intellectual property. Assuming that we meet each of these development and regulatory milestones not more than once for each product, which we expect will be the case, the maximum aggregate milestone payments payable under this license with respect to any individual product that we commercialize will be $4.7 million. We will also be required to pay a royalty on net sale of products covered by the in-licensed intellectual property in the low-single digits. The royalty is subject to reduction for any third-party payments required to be made, with a minimum floor in the low single digits. We have the right to sublicense our rights under this agreement, and we will be required to pay a percentage of such license income in the low-double digits. We are required to make annual maintenance payments in the mid-four figures to mid-five figures under this license, which payments are creditable against royalty payments on a year-by-year basis.

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

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Competition

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

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

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of treatments and the commercialization of those treatments. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

Government regulation

Biological products, including gene therapy products, are subject to regulation under the Federal Food, Drug, and Cosmetic Act, or FD&C Act, and the Public Health Service Act, or PHS Act, and other federal, state, local and foreign statutes and regulations. Both the FD&C Act and the PHS Act and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, reporting, advertising and other promotional practices involving biological products. Before clinical testing of biological products may begin, we must submit an IND which must go into effect, and each clinical trial protocol for a gene therapy product candidate is reviewed by the FDA and, in some instances, the NIH, through its Recombinant DNA Advisory Committee, or RAC. FDA approval of a BLA also must be obtained before marketing of biological products. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals.

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

Ethical, social and legal concerns about gene therapy, genetic testing and genetic research have led to the enactment of legislation such as the Genetic Information Nondiscrimination Act of 2008 and could result in additional regulations restricting or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed interest in further regulating biotechnology.

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More restrictive regulations or claims that our products are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

Recent developments in regulation of gene therapy

Although the FDA has not yet approved any human gene therapy product for sale, it has provided guidance for the development of gene therapy products. For example, the FDA has established the Office of Cellular, Tissue and Gene Therapies, or OCTGT, within CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee, or CTGTAC, to advise CBER on its reviews. In addition, the FDA has issued a growing body of clinical guidelines, chemical, manufacturing and control, or CMC, guidelines and other guidelines, all of which are intended to facilitate industry s development of gene therapy products.

In 2012, the EMA approved a gene therapy product called Glybera, which is the first gene therapy product approved by regulatory authorities anywhere in the Western world.

United States biological products development process

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

- completion of nonclinical laboratory tests and animal studies according to good laboratory practices, or GLP, requirements and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an IND, which must become effective before human clinical trials may begin;
- performance of adequate and well-controlled human clinical trials according to the FDA s regulations commonly referred to as good clinical practices, or GCP, requirements and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biological product candidate for its intended use;
- submission to the FDA of a Biologics License Application, or BLA, for marketing approval that includes substantive evidence of safety, purity, and potency from results of nonclinical testing and clinical trials;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biological product candidate is produced to assess compliance with GMP requirements, to assure that the facilities, methods and controls are adequate to preserve the biological product candidate s identity, strength, quality and purity;
- potential FDA audit of the nonclinical and clinical trial sites that generated the data in support of the BLA; and
- FDA review and approval, or licensure, of the BLA prior to any commercial marketing or sale of the product candidate in the United States.

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

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Where a gene therapy trial is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, prior to the submission of an IND to the FDA, a protocol and related documentation is submitted to and the trial is registered with the NIH Office of Biotechnology Activities, or OBA, pursuant to the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules, or NIH Guidelines. Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for research involving recombinant DNA, however many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. The NIH is responsible for convening the RAC, a federal advisory committee, which discusses protocols that raise novel or particularly important scientific, safety or ethical considerations at one of its quarterly public meetings. The OBA will notify the FDA of the RAC s decision regarding the necessity for full public review of a gene therapy protocol. RAC proceedings and reports are posted to the OBA web site and may be accessed by the public.

The clinical trial sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical trial on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. With gene therapy protocols, if the FDA allows the IND to proceed, and the RAC decides that full public review of the protocol is warranted but did not take place before the IND review is complete, the FDA will request at the completion of its IND review that sponsors delay initiation of the protocol until after completion of the RAC review process. The FDA may also impose clinical holds on a biological product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that suspend or terminate such trials.

Clinical trials involve the administration of the biological product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor s control. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical trial will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. Clinical trials must be conducted and monitored in accordance with the FDA s GCP requirements, including the requirement that all research subjects provide informed consent. Further, each clinical trial must be reviewed and approved by an independent institutional review board, or IRB, at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. Clinical trials also must be reviewed by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees basic and clinical research conducted at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

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

Phase 1. The biological product candidate is initially introduced into healthy human subjects and tested for safety. In the case of some product candidates for severe or life-threatening diseases, especially when the product candidate may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.

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Phase 2. The biological product candidate is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product candidate for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.

Phase 3. Clinical trials are undertaken to further evaluate dosage, clinical efficacy, potency, and safety in an expanded patient population at geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the product candidate and provide an adequate basis for product labeling.

Post-approval clinical trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up. The FDA recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by 10 years of annual queries, either in person or by questionnaire, of trial subjects.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical trial investigators. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA, the NIH and the investigators for serious and unexpected adverse events, any findings from other trials, tests in laboratory animals or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor s initial receipt of the information. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA or the sponsor or its data safety monitoring board may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB s requirements or if the biological product candidate has been associated with unexpected serious harm to patients.

Human gene therapy products are a new category of therapeutics. Because this is a relatively new and expanding area of novel therapeutic interventions, there can be no assurance as to the length of the trial period, the number of patients the FDA will require to be enrolled in the trials in order to establish the safety, efficacy, purity and potency of human gene therapy products, or that the data generated in these trials will be acceptable to the FDA to support marketing approval. The NIH and the FDA have a publicly accessible database, the Genetic Modification Clinical Research Information System, which includes information on gene transfer trials and serves as an electronic tool to facilitate the reporting and analysis of adverse events on these trials. Over the last several years the FDA has issued helpful guidance on development of gene therapy products and shown a willingness to work closely with developers, especially with those working in orphan disease areas.

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

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United States review and approval processes

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

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a user fee. The FDA adjusts the PDUFA user fees on an annual basis. According to the FDA s fee schedule for fiscal year 2014, effective October 1, 2013, the user fee for an application requiring clinical data, such as a BLA, is $2,169,100. PDUFA also imposes an annual product fee for biologics ($104,060) and an annual establishment fee ($526,500) on facilities used to manufacture prescription biologics. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on BLAs for product candidates designated as orphan drugs, unless the product candidate also includes a non-orphan indication.

Within 60 days following submission of the application, the FDA reviews a BLA submitted to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product candidate is safe and potent, or effective, for its intended use, and has an acceptable purity profile, and whether the product candidate is being manufactured in accordance with GMP regulations to assure and preserve the product candidate s identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the biological product approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy, or REMS, is necessary to assure the safe use of the biological product candidate. A REMS may be imposed to ensure safe use of the drug, and could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

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

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

If a product candidate receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product candidate. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a REMS, or otherwise limit the scope of any approval. In addition, the FDA may require post marketing clinical trials, sometimes referred to as Phase 4 clinical trials, designed to further assess a biological product s safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

One of the performance goals agreed to by the FDA under the PDUFA is to review 90% of standard BLAs in 10 months and 90% of priority BLAs in six months, whereupon a review decision is to be made. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs and its review goals are subject to change from time to time. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the BLA sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

Orphan drug designation

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

If a product candidate that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan product exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same biological product as defined by the FDA or if our product candidate is determined to be contained within the competitor s product for the same indication or disease. If a drug or biological product designated as an orphan product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan product exclusivity. Orphan drug status in the European Union has similar, but not identical, benefits.

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Expedited development and review programs

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

Any product candidate submitted to the FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. Any product candidate is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a disease compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biological product candidate designated for priority review in an effort to facilitate the review, and aims to review such applications within six months as opposed to ten months for standard review. Additionally, a product candidate may be eligible for accelerated approval. Drug or biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval, which means that they may be approved on the basis of adequate and well-controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug or biological product candidate receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

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

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

Post-approval requirements

Maintaining compliance with applicable federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Rigorous and extensive FDA regulation of biological products continues after approval, particularly with respect to GMP requirements. We will rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of any products that we

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may commercialize. Manufacturers of our products are required to comply with applicable requirements in the GMP regulations, including quality control and quality assurance and maintenance of records and documentation. Other post-approval requirements applicable to biological products include reporting of GMP deviations that may affect the identity, potency, purity and overall safety of a distributed product, record-keeping requirements, reporting of adverse effects, reporting updated safety and efficacy information, and complying with electronic record and signature requirements. After a BLA is approved, the product may also be subject to official lot release. In this case, as part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer s tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products, such as viral vaccines, before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products.

We also must comply with the FDA s advertising and promotion requirements, such as those related to direct-to-consumer advertising, the prohibition on promoting products for uses or in patient populations that are not described in the product s approved labeling (known as off-label use), industry-sponsored scientific and educational activities, and promotional activities involving the internet. Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant or manufacturer to administrative or judicial civil or criminal sanctions and adverse publicity. FDA sanctions could include refusal to approve pending applications, withdrawal of an approval, clinical hold, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, mandated corrective advertising or communications with doctors, debarment, restitution, disgorgement of profits, or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

Biological product manufacturers and other entities involved in the manufacture and distribution of approved biological products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with GMPs and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain GMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved BLA, including withdrawal of the product from the market. In addition, changes to the manufacturing process or facility generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

United States patent term restoration and marketing exclusivity

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

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United States Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for one or more of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant BLA.

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

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

A reference biologic is granted twelve years of exclusivity from the time of first licensure of the reference product. On April 10, 2013, President Obama released his proposed budget for fiscal year 2014 and proposed to cut this twelve-year period of exclusivity down to seven years. He also proposed to prohibit additional periods of exclusivity for brand biologics due to minor changes in product formulations, a practice often referred to as evergreening. The first biologic product submitted under the abbreviated approval pathway that is determined to be interchangeable with the reference product has exclusivity against other biologics submitting under the abbreviated approval pathway for the lesser of (i) one year after the first commercial marketing, (ii) 18 months after approval if there is no legal challenge, (iii) 18 months after the resolution in the applicant s favor of a lawsuit challenging the biologics patents if an application has been submitted, or (iv) 42 months after the application has been approved if a lawsuit is ongoing within the 42-month period.

Pharmaceutical Coverage, Pricing and Reimbursement

Sales of our products will depend, in part, on the extent to which our products will be covered by third-party payors, such as federal, state, and foreign government health care programs, commercial insurance and managed healthcare organizations. These third-party payors are increasingly reducing reimbursements for medical products, drugs and services. In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. Adoption of price controls and cost containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our net revenue and results. Decreases in third-party reimbursement for our product candidates or a decision by a third-party payor not to cover our product candidates could reduce physician usage of our products once approved and have a material adverse effect on our sales, results of operations and financial condition.

Other Healthcare Laws

Although we currently do not have any products on the market, if our product candidates are approved and we begin commercialization, we may be subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which we conduct our business.

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Such laws include, without limitation, state and federal anti-kickback, fraud and abuse, false claims, privacy and security and physician sunshine laws and regulations. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply to us, we may be subject to penalties, including, without limitation, civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability to operate our business and our financial results.

Facilities

Our corporate headquarters are located in Alachua, Florida. Our current leased facility encompasses approximately 5,374 square feet of office and laboratory space. The lease for the primary laboratory facility expires on December 31, 2014. The lease for the secondary laboratory facility expires on June 30, 2014. The lease for the office facility expires on December 31, 2014, subject to our option to renew for up to one additional three-year term. We are currently reviewing options to re-locate the office space within the same corporate campus as the existing laboratory space or to potentially relocate both spaces into a concurrent building starting in January 2015, and believe that suitable space will be available on commercially reasonable terms.

Employees

As of March 10, 2014, we had 17 full-time employees, 13 of whom have Ph.D., M.D. or other post-graduate degrees. Of these full-time employees, ten are engaged in research and development activities and five are engaged in finance, legal, human resources, facilities and general management.

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

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

Legal proceedings

We are not currently a party to any material legal proceedings. From time to time, we may be subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, any such future litigation could have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

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MANAGEMENT

Executive Officers, Directors and Key Employees

Our executive officers, directors and key employees, their current positions and their current ages as of March 10, 2014 are set forth below:

Name	Age	Position(s)
Susan B. Washer	52	President, chief executive officer and director
Jeffrey D. Chulay, M.D.	67	Vice president and chief medical officer
Daniel Menichella	54	Vice president and chief business officer
Lawrence E. Bullock	58	Chief financial officer
David R. Knop, Ph.D.	40	Director, process development
Scott Koenig, M.D., Ph.D.	61	Chairman of the board of directors
Jill Carroll (1) (2)	38	Director
Ed Hurwitz (1) (2)	50	Director
Arnold L. Oronsky, Ph.D. (2)	74	Director
James Rosen (1)	44	Director
Sam Wu, M.D., Ph.D. (2)	47	Director

(1) Member of the audit committee.
(2) Member of the compensation committee.

Susan B. Washer has served as our president and chief executive officer since March 2002 and as a member of our board of directors since November 2003. Prior to becoming our president and chief executive officer, Ms. Washer served as our chief operating officer from October 2001 to March 2002. From August 1996 to October 2001, Ms. Washer was president and chief executive officer of Scenic Productions Inc., a specialty construction firm providing sculpting, painting and construction services to the entertainment industry. From June 1994 to August 1996, Ms. Washer served as the Founding Executive Director and then Business Advisor for the North Florida Technology Innovation Center, a public-private organization financing and providing services to entrepreneurial companies licensing technology from Florida universities. From October 1983 to June 1994, Ms. Washer served in various research and pharmaceutical management positions with Abbott Laboratories and Eli Lilly and Company. Ms. Washer received a B.S. in biochemistry from Michigan State University and an M.B.A. from the University of Florida. We believe that Ms. Washer s education and professional background in science and business management, her years of experience in the pharmaceutical and biotechnology industries, her service as a senior executive of entrepreneurial companies and her extensive knowledge of our company and its business qualify her to serve as a member of our board of directors.

Jeffrey D. Chulay, M.D. has served as our vice president and chief medical officer since July 2007. Dr. Chulay came to the company from AlphaVax, Inc., a privately-held biopharmaceutical company, where he served as senior vice president of medical and regulatory affairs and chief medical officer from 2004 to 2007 and medical director from 2001 to 2004. Prior to AlphaVax, Inc., Dr. Chulay served as principal clinical program head of HIV and opportunistic infections clinical development for GlaxoWellcome Inc. from 1994 to 2001, and in various positions at the United States Army Medical Research Institute of Infectious Diseases, including chief of the virology division from 1992 to 1994, chief of the department of pathogenesis and immunology in 1992, chief of the department of intracellular pathogens from 1991 to 1992 and research investigator in the virology division from 1989 to 1991. Dr. Chulay earned a medical degree from Northwestern University Medical School and a diploma in tropical medicine and hygiene from the London School of Hygiene and Tropical Medicine. Dr. Chulay served his residency at Cleveland Metropolitan General Hospital and was a fellow in Infectious Disease at the Walter Reed Army Institute of Research. He is the author of more than 100 peer-reviewed publications.

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Daniel Menichella has served as our vice president and chief business officer since September 2013. From November 2011 to May 2013, he served as the chief business officer for Zyngenia, Inc., a biotherapeutics company. From October 2007 to September 2011, Mr. Menichella served as the senior vice president for corporate business development and strategy for Talecris Biotherapeutics, Inc., a producer of plasma-derived protein therapies. Prior to joining Talecris, Mr. Menichella served in various corporate business development and alliance management roles for the chemical and pharmaceutical company Merck KGaA, as the president and senior vice president of global corporate development for MorphoSys AG, a biotechnology company focused on human antibodies, and as the vice president, business development and national accounts for Novartis Animal Health, US Inc. Mr. Menichella has served on the board of directors of Paloma Pharmaceuticals, Inc. since August 2013. Mr. Menichella received a B.A. from Harvard University and an M.B.A. from the University of North Carolina at Chapel Hill.

Lawrence E. Bullock has served as our chief financial officer since February 2014. From January 2004 through May 2013, Mr. Bullock served as the chief financial officer of BioMimetic Therapeutics, Inc., a biotechnology company specializing in the development and commercialization of products to address musculoskeletal conditions. Prior to his tenure at BioMimetic Therapeutics, Mr. Bullock served as the chief financial officer of Ribozyme Pharmaceuticals Inc. from 1996 to 2003 and La Jolla Pharmaceutical Company from 1990 to 1996. Mr. Bullock received a B.A. in Business Administration from Indiana University and an M.B.A. from the University of Utah.

David R. Knop, Ph.D. joined us in March 2002, immediately after completing his doctoral research in chemical engineering at Michigan State University where he also earned his B.S. in chemical engineering. Since that time, Dr. Knop has served in a number of positions with us relating to our HAVE manufacturing method, including as our associate director, process development from February 2006 to June 2009 and our director, process development since June 2009.

Scott Koenig, M.D., Ph.D. has served as the chairman of our board of directors since April 2004. Dr. Koenig is the president, chief executive officer and a director of MacroGenics, Inc., a biopharmaceutical company, a role which he has held since September 2001. Prior to joining MacroGenics, Dr. Koenig served as senior vice president of research at MedImmune, Inc., a biopharmaceuticals company. From 1984 to 1990, he worked in the Laboratory of Immunoregulation at the National Institute of Allergy and Infectious Diseases (NIAID) at the National Institutes of Health (NIH), where he investigated the immune response to retroviruses and studied the pathogenesis of AIDS. Dr. Koenig currently serves as a member of the boards of the Biotechnology Industry Organization (BIO), the Children s National Medical Center and the Children s Research Institute of Children s National Medical Center, for which he also serves as chairman of the board. Dr. Koenig received his A.B. and Ph.D. from Cornell University and his M.D. from the University of Texas Health Science Center in Houston. We believe that Dr. Koenig s education and professional background in science and medicine, his experience as chief executive officer of MacroGenics and as a scientist and senior executive at other life science companies and research organizations and his service as a director of other biopharmaceutical companies, medical institutions and industry groups qualify him to serve as a member of our board of directors.

Jill Carroll has served as a member of our board of directors since April 2013. Ms. Carroll has served as a senior associate for S.R. One, Limited, the corporate venture capital arm of GlaxoSmithKline, since September 2011. Prior to her tenure at S.R. One, Limited, she was Senior Director, Corporate Development at Dynavax Technologies Corporation, a biopharmaceutical company, from May 2004 to August 2010 and the Vice President of Corporate Development at Limerick Biopharma Inc., a pharmaceuticals company, from August 2010 to September 2011. Ms. Carroll also served as a consultant for the consulting firms Clearview Projects, Inc. from October 2001 to May 2004 and Mercer Management Consulting from March 1999 to July 2001. She received her B.S. in Chemistry from Duke University and her M.S. in Biochemistry, Cellular and Molecular Biology from Johns Hopkins University. We believe that Ms. Carroll s educational background in science, her work as a management consultant and as an executive and venture capitalist focused on the biotechnology and pharmaceutical industries and her experience with biotechnology and pharmaceutical partnering deals qualify her to serve as a member of our board of directors.

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Ed Hurwitz has served as a member of our board of directors since November 2012. Mr. Hurwitz is a managing director of Precision Bioventures, LLC, a consulting and advisory firm, and a director of the general partner of Alta BioPharma III, L.P., a fund affiliated with Alta Partners, a venture capital firm. He was a director at Alta Partners from 2002 through December 2013 and continues to serve as a consultant to that firm and as a board representative on its portfolio companies. He also serves on the boards of directors of Cara Therapeutics Inc., a biotechnology company and of MacroGenics, Inc. Prior to joining Alta, Mr. Hurwitz served as senior vice president and chief financial officer of Affymetrix, Inc., a manufacturer of DNA microarrays, from 1997 to 2002. From 1994 to 1997, Mr. Hurwitz was a biotechnology research analyst for the investment bank Robertson Stephens & Company, and from 1992 to 1994, was a biotechnology research analyst for the investment bank Smith Barney Shearson. From 1990 to 1992, he practiced commercial law at Cooley LLP. Mr. Hurwitz earned a J.D. and an M.B.A. from the U.C. Berkeley School of Law and Haas School of Business, respectively. He also holds a B.A. in Molecular Biology from Cornell University. We believe that Mr. Hurwitz s education and professional background in science, business management and law, his work as a lawyer, research analyst and senior executive in the biotechnology industry and his experience as a director of other public and private biotechnology companies qualify him to serve as a member of our board of directors.

Arnold L. Oronsky, Ph.D. has served as a member of our board of directors since November 2003. Dr. Oronsky has been a general partner at InterWest Partners, LLC, a venture capital firm, since 1994. Prior to joining InterWest, Dr. Oronsky was vice president for discovery research at Lederle Laboratories, a division of American Cyanamid Company focused on the production of vaccines. Dr. Oronsky holds a Ph.D. in Immunology from Columbia University and has published over 125 scientific articles. He also serves as a Senior Lecturer in the Department of Medicine at Johns Hopkins Medical School. Dr. Oronsky serves as the chairman of the board of directors of Dynavax Technologies Corporation, a biopharmaceutical company, as well as on the boards of directors of Macrogenics, Inc., and TESARO, Inc., an oncology-focused biopharmaceutical company. Dr. Oronsky also served on the boards of directors of the biopharmaceutical companies, Metabasis Therapeutics, Inc., from 2000 to 2010, and Anesiva, Inc., from 2005 to 2010. Anesiva filed a voluntary petition for relief under Chapter 7 of the U.S. Bankruptcy Code in the U.S. Bankruptcy Court for the Northern District of California in January 2010. We believe that Dr. Oronsky s education and professional experience in science and medicine, his experience building and operating research and development operations and his experience in the venture capital industry, particularly with biotech and pharmaceutical companies, qualify him to serve as a member of our board of directors.

James Rosen has served as a member of our board of directors since March 2010. Mr. Rosen is a partner at Intersouth Partners, a venture capital firm, where he serves on the life sciences investment team. Mr. Rosen began is work with Intersouth in 2005 and held various roles before becoming a partner in 2009. Prior to joining Intersouth, he spent 15 years in clinical, research and financial positions in the health care and biotechnology sectors, including serving as an equity research analyst at Brean Murray & Co., from 2000 to 2003, covering biopharmaceuticals, genomics, generics, drug delivery and medical device companies. Mr. Rosen holds a B.A. from Duke University, an M.B.A. from the University of North Carolina-Chapel Hill s Kenan-Flagler School of Business and an M.S.P.H. from the University of North Carolina School of Public Health. We believe that Mr. Rosen s education and professional background in science, business management and finance and his operational experience as a scientist and executive in the healthcare and biotechnology industries and as a venture capitalist concentrating on those industries, qualify him to serve as a member of our board of directors.

Sam Wu, M.D., Ph.D. has served as a member of our board of directors since December 2010. Dr. Wu has been a managing director of MedImmune Ventures, Inc., a venture capital fund within the AstraZeneca Group, since September 2010. Before joining MedImmune, Dr. Wu held various roles, including principal, at SV Life Sciences Advisers, LLC, from 2002 through 2010. Prior to his tenure at SV Life Sciences, Dr. Wu was an engagement manager with McKinsey and Company's Pharmaceuticals and Medical Products practice. Dr. Wu holds an A.B. in Biochemistry from Harvard College, and an M.D. and a Ph.D. in Biochemistry from Stanford University, where he was a Howard Hughes Predoctoral Fellow. We believe that Dr. Wu s education and professional background in biochemistry and internal medicine and his experience in management consulting and as a venture capitalist concentrating on the biotechnology and pharmaceutical industries qualify him to serve as a member of our board of directors.

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There are no family relationships among any of our directors or executive officers.

Board Composition

Our board of directors currently consists of seven members, all of whom were elected as directors pursuant to a stockholders agreement that we have entered into with the holders of our preferred stock. The stockholders agreement will terminate upon the closing of this offering and there will be no further contractual obligations regarding the election of our directors. Our directors hold office until their successors have been elected and qualified or until the earlier of their resignation or removal. Ms. Carroll has notified us that she intends to resign from our board of directors following this offering. In addition, Dr. Oronsky has informed us that he intends to resign from our board of directors no later than the first anniversary of the closing of this offering.

Our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering provide that the authorized number of directors may be changed only by resolution of the board of directors. Our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering also provide that our directors may be removed only for cause by the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in an annual election of directors, and that any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

In accordance with the terms of our certificate of incorporation and bylaws that will become effective upon the closing of this offering, our board of directors will be divided into three classes, class I, class II and class III, with members of each class serving staggered three-year terms. Upon the closing of this offering, the members of the classes will be divided as follows:

- the class I directors will be Dr. Oronsky and Dr. Wu, and their initial term will expire at the annual meeting of stockholders to be held in 2014;
- the class II directors will be Ms. Carroll and Dr. Koenig, and their initial term will expire at the annual meeting of stockholders to be held in 2015; and
- the class III directors will be Ms. Washer, Mr. Hurwitz and Mr. Rosen, and their initial term will expire at the annual meeting of stockholders to be held in 2016.

Upon the expiration of the term of a class of directors, directors in that class will be eligible to be elected for a new three-year term at the annual meeting of stockholders in the year in which their term expires.

We have no formal policy regarding board diversity. Our priority in selection of board members is identification of members who will further the interests of our stockholders through an established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, knowledge of our business and understanding of the competitive landscape.

Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that each of our directors, with the exception of Ms. Washer, is an independent director as defined under Rule 5605(a)(2) of the NASDAQ Listing Rules.

One of the key functions of our board of directors is informed oversight of our risk management process. The board of directors does not have a standing risk management committee, but rather administers this oversight function directly through the board of directors as a whole, as well as through various standing committees of our board of directors that address risks inherent in their respective areas of oversight. In particular, our board of directors is responsible for monitoring and assessing strategic risk exposure and our audit committee has the responsibility to consider and discuss our major financial risk exposures and the steps our management has taken

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to monitor and control these exposures, including guidelines and policies to govern the process by which risk assessment and management is undertaken. The audit committee also monitors compliance with legal and regulatory requirements. Our nominating and corporate governance committee monitors the effectiveness of our corporate governance practices, including whether they are successful in preventing illegal or improper liability-creating conduct. Our compensation committee assesses and monitors whether any of our compensation policies and programs has the potential to encourage excessive risk-taking.

Board Committees

Our board of directors has established an audit committee and a compensation committee. We have also established a nominating and corporate governance committee, effective upon the closing of this offering. Each of these committees, which are the only standing committees of our board of directors, will operate under a charter that has been approved by our board of directors.

Audit committee. The current members of our audit committee are Ms. Carroll, Mr. Hurwitz and Mr. Rosen. Effective as of the closing of this offering, our audit committee will consist of Mr. Hurwitz, Dr. Oronsky and Mr. Rosen. Our board of directors has determined that Mr. Hurwitz, Dr. Oronsky and Mr. Rosen satisfy the NASDAQ Stock Market independence standards and the independence standards of Rule 10A-3(b)(1) of the Securities Exchange Act. Each of the members of our audit committee meets the requirements for financial literacy under applicable rules and regulations of the SEC and the NASDAQ Stock Market. The board of directors has also determined that Mr. Hurwitz qualifies as an audit committee financial expert, as defined by applicable rules of the NASDAQ Stock Market and the SEC. The audit committee assists our board of directors in its oversight of:

- the integrity of our financial statements;
- our compliance with legal and regulatory requirements;
- the qualifications and independence of our independent registered public accounting firm; and
- the performance of our independent registered public accounting firm.

The audit committee has direct responsibility for the appointment, compensation, retention and oversight of the work of our independent registered public accounting firm. The audit committee establishes and implements policies and procedures for the pre-approval of all audit services and all permissible non-audit services provided by our independent registered public accounting firm and reviews and approves any related party transactions entered into by us.

Compensation committee. The current members of our compensation committee are Ms. Carroll, Mr. Hurwitz, Dr. Oronsky and Dr. Wu. Effective as of the closing of this offering, our compensation committee will consist of Mr. Hurwitz, Dr. Koenig and Mr. Rosen, each of whom is an independent director. The compensation committee:

- approves the compensation and benefits of our executive officers;
- reviews and makes recommendations to the board of directors regarding benefit plans and programs for employee compensation; and
- administers our equity compensation plans.

Nominating and corporate governance committee. Effective upon the closing of this offering, the members of our nominating and corporate governance committee will be Mr. Hurwitz and Dr. Wu, each of whom is an independent director. The nominating and corporate governance committee will:

identify individuals qualified to become board members;

recommend to the board of directors nominations of persons to be elected to the board; and

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advise the board regarding appropriate corporate governance policies and assists the board in achieving them.

Compensation Committee Interlocks and Insider Participation

None of our executive officers serves, or in the past has served, as a member of the board of directors or compensation committee, or other committee serving an equivalent function, of any entity that has one or more executive officers who serve as members of our board of directors or our compensation committee. None of the members of our compensation committee is an officer or employee of our company, nor has any of them ever been an officer or employee of our company.

Code of Business Conduct and Ethics

We have adopted a written code of business conduct and ethics that applies to our directors, executive officers and employees. Following this offering, a copy of the code will be posted on the Corporate Governance section of our website, which is located at www.agtc.com. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website.

Director Compensation

Prior to this offering, we did not have a formal policy regarding compensation of our non-employee directors, other than our chairman. We paid Dr. Koenig, the chairman of our board of directors, an annual cash retainer of $20,000. Dr. Koenig also received $1,500 for each meeting of our board of directors, or any board committee, that he attended in person. None of our other non-employee directors has historically received any compensation. We do not pay any compensation to our president and chief executive officer in connection with her service on our board of directors.

Following the closing of this offering, our non-employee directors will receive equity-based compensation and cash fees as follows:

each non-employee director will receive an annual cash fee in the amount of $35,000;

our chairman will receive an additional cash fee in the amount of $27,500;

the chairperson of each of our board committees will receive an additional annual cash fee as follows: audit committee chair, $15,000; compensation committee chair, $10,000; and nominating and corporate governance committee chair, $7,000; and

each other member of a board committee will receive an additional annual cash fee as follows: audit committee, $7,500; compensation committee, $5,000; and nominating and corporate governance committee, $3,500.

The cash fees described above will be paid quarterly in arrears. Non-employee directors are also reimbursed upon request for travel and other out-of-pocket expenses incurred in connection with their attendance at meetings of the board and of committees on which they serve.

Each non-employee director in office on the date of this prospectus, or who is first elected to the board after the date of this prospectus, will receive a non-qualified stock option, vesting in equal installments on each of the first three anniversaries of the date of grant, to purchase 9,375 shares of our common stock. In addition, each non-employee director remaining in office will receive annually a non-qualified stock option, vesting on the first anniversary of the date of grant, to purchase 4,688 shares of our common stock. Each such initial or annual stock option will be at an exercise price equal to the fair value of our common stock on the date of grant.

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In February 2012, we granted Dr. Koenig an option to purchase 285 shares of our common stock at an exercise price of $3.50 per share, and in January 2013, we granted Dr. Koenig an option to purchase 32,018 shares of our common stock at an exercise price of $0.35 per share. The options granted to Dr. Koenig in February 2012 were fully vested upon their issuance. The options granted to Dr. Koenig in January 2013 provide for vesting in equal monthly installments over a period of four years. We have not granted stock options to any of our other non-employee directors.

The following table sets forth information regarding compensation awarded to, earned by or paid to Dr. Koenig during fiscal year 2013. See Executive Compensation for a discussion of the compensation of Ms. Washer.

Name	Fees earned or paid in cash (1)	Option awards ($)(2)	Total ($)
Scott Koenig, M.D., Ph.D.	$ 20,000	$ 6,276	$ 26,276

(1) Represents amount paid during fiscal year 2013.

(2) Represents the grant date fair value of option awards granted in fiscal year 2013 in accordance with ASC Subtopic 505-50. The assumptions we use in calculating these amounts are discussed in note 5 to notes to financial statements appearing elsewhere in this prospectus.

The table below shows the aggregate numbers of option awards held as of June 30, 2013 by each non-employee director who was serving as of June 30, 2013.

Name	Options Outstanding at Fiscal Year End (#)
Scott Koenig, M.D., Ph.D.	45,673

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EXECUTIVE COMPENSATION

Summary Compensation Table

The following table sets forth information regarding compensation earned by our President and Chief Executive Officer and our other two executive officers who served during fiscal year 2013. We refer to these individuals as our named executive officers.

Name	Year	Salary ($)	Option awards ($)(1)	Non-equity incentive plan compensation ($)(2)	Other ($)(3)	Total ($)
Susan B. Washer	2013	285,000	23,655	112,175	9,010	429,840
President and chief executive officer						
Jeffrey D. Chulay, M.D.	2013	326,398	6,960	106,334	10,126	449,818
Vice president and chief medical officer						
David R. Knop, Ph.D.	2013	122,431	2,607	10,896	5,318	141,252
Director, process development						

(1) Represents the grant date fair value of option awards granted in fiscal year 2013 in accordance with ASC 718. The assumptions we use in calculating these amounts are discussed in note 5 to notes to financial statements appearing elsewhere in this prospectus.
(2) Amounts represent cash bonuses earned in fiscal year 2013, and paid during fiscal year 2014, based on achievement of individual performance goals and other factors deemed relevant by our compensation committee and board of directors.
(3) Consists of 401(k) matching contributions.

The annual base salary of our president and chief executive officer will increase to $375,000, effective upon the closing of this offering.

Narrative Disclosure to Summary Compensation Table

We review compensation annually for all of our employees, including our executives. In setting executive base salaries and bonuses and granting equity incentive awards, we consider compensation for comparable positions in the market, the historical compensation levels of our executives, individual performance as compared to our expectations and objectives, our desire to motivate our employees to achieve short- and long-term results that are in the best interests of our stockholders, and a long-term commitment to our company. We do not target a specific competitive position or a specific mix of compensation among base salary, bonus or long-term incentives.

Our board of directors has historically determined our executives compensation. Our compensation committee typically has reviewed and discussed management s proposed compensation with the chief executive officer for all executives other than our chief executive officer. Based on those discussions and its discretion, the compensation committee then has recommended the compensation for each executive officer. Our board of directors, without members of management present, has discussed the compensation committee s recommendations and ultimately approved the compensation of our executive officers. Effective upon the closing of this offering, our compensation committee will approve the compensation and benefits of our executive officers.

In preparing to become a public company, we have begun a thorough review of all elements of our executive compensation program, including the function and design of our equity incentive programs, and in fiscal year 2014, our compensation committee engaged Aon Consulting s Radford Surveys + Consulting to assist us with the identification of an appropriate peer group of companies for purposes of benchmarking the competitiveness of our executive compensation. Our compensation committee will evaluate the need for revisions to our executive compensation program to ensure that our program is competitive with the companies with which we compete for executive talent and that it is appropriate for a public company.

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Outstanding Equity Awards at Year End

The following table sets forth information regarding outstanding stock options held by our named executive officers as of June 30, 2013.

	Option Awards				
Name	Number of Securities Underlying Unexercised Options (#) exercisable	Number of Securities Underlying Unexercised Options (#) unexercisable	Option Exercise Price ($)	Option Expiration Date	Option Grant Date
Susan B. Washer	15,000		$ 3.50	2/10/2014	2/10/2004
	5,328		$ 3.50	11/8/2016	11/8/2006
	18,320(1)	1,221	$ 3.50	9/18/2019	9/18/2009
	3,877		$ 3.50	11/2/2021	11/2/2011
	12,571(2)	108,155	$ 0.35	1/6/2023	1/6/2013
Jeffrey D. Chulay, M.D.	8,028		$ 3.50	5/31/2017	5/31/2007
	2,277		$ 3.50	5/31/2017	5/31/2007
	2,008(1)	134	$ 3.50	9/18/2019	9/18/2009
	3,699(2)	31,812	$ 0.35	1/6/2023	1/6/2013
David R. Knop, Ph.D.	400		$ 3.50	2/10/2014	2/10/2004
	285		$ 3.50	12/10/2014	12/10/2004
	285		$ 3.50	6/29/2016	6/29/2006
	3,696(1)	246	$ 3.50	9/18/2019	9/18/2009
	57		$ 3.50	11/2/2021	11/2/2011
	1,385(2)	11,914	$ 0.35	1/6/2023	1/6/2013

(1) This option becomes exercisable for 25% of the underlying shares on the first anniversary of the grant date, and thereafter becomes exercisable for the remaining underlying shares in equal monthly installments over three years, resulting in the option being exercisable for 100% of the underlying shares on the fourth anniversary of the grant date.

(2) This option becomes exercisable in equal monthly installments over four years from the date of grant.

Employment Agreements, Severance and Change in Control Arrangements

We do not have formal employment agreements with any of our named executive officers and none of our named executive officers is entitled to any severance payments in connection with the termination of his or her employment. Each of our named executive officers is an employee-at-will of the Company.

Stock Option and Other Compensation Plans

We believe that equity-based awards are important vehicles by which to align the interest of our employees with the financial interests of our stockholders, and we historically have awarded stock options broadly to our employees, including our named executive officers. The material terms and conditions of our stock option and other equity compensation plans are described below.

We have the following equity incentive plans: (i) 2001 Stock Option Plan; (ii) 2011 Stock Incentive Plan; (iii) 2013 Equity and Incentive Plan; and (iv) 2013 Employee Stock Purchase Plan. Following the closing of this offering, our 2013 Equity and Incentive Plan and 2013 Employee Stock Purchase Plan will be the only effective equity compensation plans pursuant to which we will make new awards.

2001 Stock Option Plan

The 2001 Stock Option Plan, as amended, provides for the grant of incentive and nonqualified stock options. Stock options may no longer be granted under the terms of the 2001 Stock Option Plan.

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The material features of our 2001 Stock Option Plan are summarized below. The complete text of our 2001 Stock Option Plan and amendments are filed as exhibits to the registration statement of which this prospectus forms a part.

General. The total number of shares of common stock reserved for issuance under the 2001 Stock Option Plan is 124,050. Any shares that may be issued under our 2001 Stock Option Plan to any person pursuant to an award are counted against this limit as one share for every one share granted.

Purpose. The purpose of our 2001 Stock Option Plan is to promote the company s financial success by creating an additional incentive for key employees, directors and consultants or advisors of the company and certain successors or affiliates.

Administration. Our 2001 Stock Option Plan is administered by our board of directors, and such responsibility may be delegated to a duly appointed committee of our board of directors.

Source of shares. The shares of common stock issued or to be issued under our 2001 Stock Option Plan consist of authorized but unissued shares. Shares of common stock underlying any awards issued under the 2001 Stock Option Plan that were terminated, unexercised, or repurchased without having been fully exercised could be granted under the 2001 Stock Option Plan.

Eligibility. Options under the 2001 Stock Option Plan could be granted to employees (including officers) and directors of the company, any successor corporations thereto, and any present or future parent and/or subsidiary corporations of such corporation, or collectively, the Company Group. Options could also be granted to individuals rendering services as consultants, advisors or other independent contractors to the Company Group.

Options. Our 2001 Stock Option Plan permitted the grant of options to purchase shares of common stock intended to qualify as incentive stock options under the Internal Revenue Code of 1986, as amended, or the Code, and options that do not qualify as incentive stock options, which are referred to as nonqualified stock options. The 2001 Stock Option Plan permitted the grant of incentive stock options only to our employees.

Pursuant to the 2001 Stock Option Plan, the exercise price of each incentive stock option could not be less than 100% of the fair market value of shares of our common stock on the date of grant. Grants of incentive stock options to any 10% stockholder required that the exercise price be not less than 110% of the fair market value of shares of our common stock on the date of grant. The exercise price of any non-qualified stock option granted under the plan was determined by our board of directors but in no event could be less than the fair market value of shares of our common stock.

The term of options granted under the plan was subject to the discretion of the board of directors, but no incentive stock options granted under the plan are exercisable after the expiration of ten years from the date of grant (five years in the event the optionee owned 10% of the voting power of all classes of stocks as of the date of grant).

The 2001 Stock Option Plan permits for payment of the option price by cash or cash equivalent, check or any other form as permitted by our board of directors in its discretion.

No option granted pursuant to the 2001 Stock Option plan may be assigned, except by will or by the laws of decent and distribution.

Effect of a transfer of control. Upon the occurrence of a transfer of control (as defined in the 2001 Stock Option Plan), except as may be otherwise provided in any individual stock option award agreement, any unvested portion of an outstanding option that would otherwise become vested within twelve months following the effective time of a transfer of control shall become immediately vested as of a date prior to the transfer of control, which date shall be determined by our board of directors. Upon the occurrence of a transfer of control, the surviving, continuing, successor or purchasing corporation, or parent corporation thereof, may either assume

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the company s rights and obligations or substitute for outstanding options substantially equivalent options for the acquiring corporation s stock. Any options not assumed prior to the transfer of control shall be deemed canceled effective as of the closing of a transfer of control.

2011 Stock Incentive Plan

We have adopted our 2011 Stock Incentive Plan, which provides for the issuance of equity-based awards, denominated in shares of our common stock and including incentive stock options, nonstatutory stock options, restricted stock awards, restricted stock units, stock appreciation rights and other share-based awards. No restricted stock awards, restricted stock units, stock appreciation rights or other share-based awards have been granted under the 2011 Stock Incentive Plan. We will not make any new awards under the 2011 Stock Incentive Plan following the closing of this offering.

The material features of our 2011 Stock Incentive Plan are summarized below. The complete text of our 2011 Stock Incentive Plan is filed as an exhibit to the registration statement of which this prospectus forms a part.

General. The total number of shares of common stock reserved for issuance under the 2011 Stock Incentive Plan is 909,101. Any shares that may be issued under our 2011 Stock Incentive Plan to any person pursuant to an award are counted against this limit as one share for every one share granted.

Purpose. The purpose of our 2011 Stock Incentive Plan is to advance the interests of our stockholders by enhancing our ability to attract, retain and motivate persons who are expected to make important contributions to the company and by providing such persons with equity ownership opportunities and performance-based incentives that are intended to align their interests with those of our stockholders.

Administration. Our 2011 Stock Incentive Plan is administered by our board of directors. The board of directors may, to the extent permitted by law, delegate any or all of its powers under the 2011 Stock Incentive Plan to one or more committees or subcommittees of the board of directors. Subject to the terms of our 2011 Stock Incentive Plan, such committee or subcommittee may determine the types of awards and the terms and conditions of such awards, interpret provisions of our 2011 Stock Incentive Plan and select participants to receive awards.

Source of shares. The shares of common stock issued or to be issued under our 2011 Stock Incentive Plan consist of authorized but unissued shares and shares that we have reacquired. Shares of common stock underlying any awards issued under the 2011 Stock Incentive Plan that are terminated, surrendered, or cancelled without having been fully exercised or is forfeited in whole or in part (including as the result of shares of common stock subject to such award being repurchased by us at the original issue price pursuant to a contractual repurchase right) or results in common stock not being issued shall be added back to the shares of common stock with respect to which awards may be granted under the 2011 Stock Incentive Plan.

Eligibility. Awards may be granted under the 2011 Stock Incentive Plan to our employees, officers, directors, and individual consultants and advisors.

Amendment or termination of our stock incentive plan. Our board of directors may terminate, suspend or amend the 2011 Stock Incentive Plan at any time. No amendment or termination may adversely impair the rights of participants with respect to outstanding awards without the affected participant s consent to such amendment. In addition, an amendment will be contingent on approval of our stockholders to the extent required by law. Unless terminated earlier, our 2011 Stock Incentive Plan will terminate in 2021, but will continue to govern unexpired awards.

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Options. Our 2011 stock incentive plan permits the granting of options to purchase shares of common stock intended to qualify as incentive stock options under the Code, and options that do not qualify as incentive stock options, which are referred to as nonstatutory stock options. We may grant nonstatutory stock options to our employees, directors, officers, consultants or advisors in the discretion of our board of directors. Incentive stock options will only be granted to our employees and employees of other entities which are eligible to receive incentive stock options under the Code.

The exercise price of each incentive stock option may not be less than 100% of the fair market value of shares of our common stock on the date of grant. If we grant incentive stock options to any person holding 10% or more of the outstanding voting stock of the company, the exercise price may not be less than 110% of the fair market value of shares of our common stock on the date of grant. The exercise price of any non-qualified stock option will be determined by our board of directors and generally may not be less than the fair market value of shares of our common stock on the date of grant.

The term of each option may be established at the discretion of the board of directors. The board of directors may determine at what time or times each option may be exercised and the period of time, if any, after retirement, death, disability or termination of employment during which options may be exercised. Options may be made exercisable in installments. The vesting and exercisability of options may be accelerated by the board of directors. The exercise price of an option may be amended to provide an exercise price per share that is lower than the then-current exercise price of such option provided that such amended exercise price is at least equal to the then-current fair market value.

In general, an optionee may pay the exercise price of an option by cash or check payable to the company, delivery of an irrevocable or unconditional undertaking by a broker to deliver funds, by tendering shares of our common stock, by a cashless exercise through a broker supported by an irrevocable and unconditional undertaking by such broker to deliver sufficient funds to pay the applicable exercise price, by delivery of shares of common stock having a fair market value equal to the aggregate exercise price of the options being exercised, delivery of a promissory note or such other lawful consideration as approved by the board of directors, or by any combination of these forms of payment.

Except as the board of directors may otherwise expressly determine or provide in an option grant, options granted under our 2011 stock incentive plan may not be sold, assigned, transferred, pledged or otherwise encumbered except by will or the laws of decent and distribution or, other than in the case of an incentive stock option, pursuant to a qualified domestic relations order.

Restricted stock. Awards of restricted stock consist of the right to acquire shares of common stock, subject to vesting restrictions and a right of repurchase in favor the company. Our board of directors determines the terms and conditions of restricted stock awards.

Restricted stock awards may have restrictions that lapse based upon length of service of the recipient or based upon the attainment of performance goals. Unless otherwise specified in the agreement governing the restricted stock award, all shares subject to the restricted stock award shall be entitled to vote and shall receive dividends during the periods of restriction.

Restricted stock units. Restricted stock units entitle the recipient to acquire shares of common stock pursuant to certain terms and conditions. The board of directors may determine the terms and conditions, including vesting, if any, related to award of restricted stock units, including the number of shares of common stock that the recipient shall be entitled to receive or purchase, the price to be paid, if any, and all other limitations and conditions applicable to the restricted stock units.

Stock appreciation rights. Stock appreciation rights entitle the recipient to receive, upon exercise of the stock appreciation right, a number of shares of common stock or, alternatively, a cash payment or combination of shares and cash, having an aggregate fair market value equal to the product of (a) the excess of the fair market

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value (as of the exercise date) over the exercise price per share of common stock specified in the stock appreciation right by (b) the number of shares of common stock subject to the stock appreciation rights. Stock appreciation rights may be subject to vesting or other restrictions determined by our board of directors.

Adjustments for share dividends and similar events. We will make appropriate adjustments in outstanding awards and the number of shares available for issuance under our stock incentive plan, including the individual limitations on awards, to reflect any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of common stock other than an ordinary cash dividend.

Effect of a change in control. Upon the occurrence of a change in control (as defined in the 2011 Stock Incentive Plan), the board of directors may take one or more of the following actions:

- provide that the participant s awards will be assumed, or substantially equivalent awards will be substituted, by the acquiring or succeeding corporation (or an affiliate thereof) in compliance with the applicable provisions of the Code;
- upon written notice to the participant, provide that the participant s unexercised options or other unexercised awards will terminate immediately prior to the consummation of such change of control unless exercised within a specified period following the date of such notice;
- provide that outstanding awards shall become exercisable, realizable or deliverable, or restrictions applicable to any award shall lapse, in whole or in part prior to or upon such change of control;
- provide for a cash payment to be made to each holder of an outstanding stock option equal to the difference between (a) the cash consideration the holder of a share of common stock would receive upon consummation of the change of control and (b) the aggregate exercise price of all outstanding options, in exchange for the termination of such options;
- provide that, in connection with a liquidation or dissolution of the company, awards shall convert into the right to receive liquidation proceeds; and
- any combination of the foregoing.

Upon a change in control, the board of directors is not obligated to treat all awards, or all awards of the same type, identically.

Upon the occurrence of a change in control other than a liquidation or dissolution of the company, the repurchase rights of the company under each outstanding restricted stock award (as defined in the 2011 Stock Incentive Plan) shall inure to the benefit of the company s successor.

Upon a change in control involving a liquidation or dissolution of the company, except to the extent specifically provided to the contrary in the instrument evidencing a restricted stock award, all restrictions and conditions on such awards then outstanding shall automatically be deemed terminated and satisfied.

2013 Equity and Incentive Plan

Our board of directors has adopted, and our stockholders have approved, our 2013 Equity and Incentive Plan. A total of 1,151,428 shares of our common stock will initially be reserved for issuance under our 2013 Equity and Incentive Plan, subject to automatic annual increases as set forth in the plan. The 2013 Equity and Incentive Plan provides for the issuance of (i) cash awards and (ii) equity-based awards, denominated in shares of our common stock, including incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock units, unrestricted stock awards, performance share awards and dividend equivalent rights.

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Purpose. The purpose of our 2013 Equity and Incentive Plan is to (i) provide long-term incentives and rewards to those employees, officers, directors and other key persons (including consultants) of the company and its subsidiaries who are in a position to contribute to the long-term success and growth of the company and its subsidiaries, (ii) to assist the company and its subsidiaries in attracting and retaining persons with the requisite experience and ability, and (iii) to more closely align the interests of such employees, officers, directors and other key persons with the interests of the company s stockholders.

Administration. Our 2013 Equity and Incentive Plan will be administered by the compensation committee of our board of directors. The compensation committee is generally granted broad authority to administer the plan, including the power to determine and modify the terms and conditions, not otherwise inconsistent with the terms of the plan, of any award. All decisions and interpretations of the compensation committee shall be binding on all persons subject to the plan including the company and plan grantees.

Sources of shares. The shares of common stock to be issued under the 2013 Equity and Incentive Plan consist of authorized but unissued shares and shares that we have reacquired. Shares of common stock underlying any award issued under the 2013 Equity and Incentive Plan that are forfeited, canceled, satisfied without issuance of stock, otherwise terminated or, for shares of stock issued pursuant to any unvested full value award, reacquired by the company shall be added back to the shares of common stock with respect to which awards may be granted under the plan.

Eligibility. Incentive stock options may only be granted to our employees. All other awards may be granted to our employees, officers, directors and key persons (including consultants and prospective employees).

Amendment or termination of our 2013 Equity and Incentive Plan. Subject to requirements of law or any stock exchange or similar rules which would require a vote of our stockholders, our board of directors may, at any time, amend or discontinue the plan and the compensation committee may, at any time, amend or cancel any outstanding award for the purpose of satisfying changes in law or for any other lawful purpose, but no such action shall adversely affect rights under any outstanding award without the holder s consent.

Options. Our 2013 Equity and Incentive Plan permits the granting of options to purchase common stock that are intended to qualify as incentive stock options under the Code, and options that do not qualify as incentive stock options, which are referred to as nonstatutory stock options. We may grant non-qualified stock options to our employees, directors, officers, consultants or advisors in the discretion of our board of directors. Incentive stock options will only be granted to our employees.

The exercise price of each incentive stock option may not be less than 100% of the fair market value of shares of our common stock on the date of grant. If we grant incentive stock options to any person holding 10% or more of the outstanding voting stock of the company, the exercise price may not be less than 110% of the fair value of shares of our common stock on the date of grant. The exercise price of any non-qualified stock option will be determined by our board of directors and may not be less than the fair value of shares of our common stock.

The term of each option may not exceed 10 years from the date of grant, and no option shall be transferable by the optionee other than by will or by the laws of descent and distribution. Notwithstanding the foregoing, the compensation committee, in its sole discretion, may provide in the award agreement regarding a given option, or may agree in writing with respect to an outstanding option, that the optionee may transfer their nonstatutory stock options to members of their immediate family, to trusts for the benefit of such family members, or to partnerships in which such family members are the only partners, provided that the transferee agrees in writing with the company to be bound by all of the terms and conditions of this plan and the applicable option.

In general, an optionee may pay the exercise price of an option by cash or, if so provided in the applicable option agreement, by tendering shares of our common stock, by a cashless exercise through a broker supported by an irrevocable instruction to such broker to deliver sufficient funds to pay the applicable exercise price, by

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reducing the number of shares otherwise issuable to the optionee upon exercise of the option by a number of shares having a fair market value equal to the aggregate exercise price of the options being exercised or by any other method permitted by the compensation committee.

Stock appreciation rights. Pursuant to the 2013 Equity and Incentive Plan, we may grant stock appreciation rights, or an award entitling the recipient to receive cash or shares of our common stock having a value on the date of exercise calculated as follows: (i) the exercise price of a share of common stock on the grant date is less the fair market value of the common stock on the date of exercise and (ii) multiplied by the number of shares of stock with respect to which the stock appreciation right shall have been exercised.

The exercise price of a stock appreciation right shall not be less than 100% of the fair market value of our common stock on the date of grant, and the terms and conditions of the stock appreciation rights shall be determined from time to time by the compensation committee.

Restricted stock awards. Pursuant to the 2013 Equity and Incentive Plan, we may grant restricted stock awards entitling the recipient to acquire, at such a price as determined by the compensation committee, shares of common stock subject to such restrictions and conditions as the compensation committee may determine at the time of grant. Conditions may be based on continuing employment or achievement of pre-established performance goals and objectives. A holder of a restricted stock award may exercise voting rights upon (i) execution of a written instrument setting forth the award and (ii) payment of any applicable purchase.

Restricted stock units. Pursuant to the 2013 Equity and Incentive Plan, we may grant restricted stock units which entitle the holder, upon vesting of the right, to a number of shares of common stock as determined in the award agreement. The compensation committee shall determine the restrictions and conditions applicable to each restricted stock unit at the time of grant, and a holder of a restricted stock unit shall only have exercisable rights as a stockholder upon settlement of restricted stock units. Unless otherwise provided in the award agreement, a holder s rights in all restricted stock units that have not vested shall automatically terminate immediately following the holder s termination of employment with the company for any reason.

Unrestricted stock awards. Pursuant to the 2013 Equity and Incentive Plan, we may grant unrestricted awards of shares of common stock free of any restrictions under the plan. The right to receive shares of unrestricted stock awards on a deferred basis may not be sold, assigned, transferred, pledged or otherwise encumbered, other than by will or the laws of descent and distribution.

Performance share awards. Pursuant to the 2013 Equity and Incentive Plan, we may grant performance share awards entitling the recipient to acquire shares of common stock upon the attainment of specified performance goals; provided, however, that the compensation committee, in its discretion, may provide either at the time of grant or at the time of settlement that a performance share award will be settled in cash. The period during which performance is to be measured for performance share awards shall not be less than one year, and such performance share awards, and all rights with respect to such awards, may not be sold, assigned, transferred, pledged or otherwise encumbered.

Dividend equivalent rights. Pursuant to the 2013 Equity and Incentive Plan, we may grant dividend equivalent rights entitling the recipient to receive credits based on cash dividends that would be paid on the shares of stock specified in the dividend equivalent right (or other award to which it relates). Dividend equivalent rights may be settled in cash or shares of stock or a combination thereof, in a single installment or installments. A dividend equivalent right granted as a component of another award may provide that such dividend equivalent right shall be settled upon exercise, settlement, or payment of, or lapse of restrictions on, such other award, and that such dividend equivalent right shall expire or be forfeited or annulled under the same conditions as such other award.

Cash awards. The compensation committee, in its discretion, may provide for cash payments to be made under the 2013 Equity and Incentive Plan. Such cash awards may be made subject to such terms, conditions and restrictions as the compensation committee considers necessary or advisable.

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Effect of a change in control. If we experience a change in control, as defined in the 2013 Equity and Incentive Plan, the compensation committee may in its discretion, at the time an award is made or at any time thereafter, take one or more of the following actions: (i) provide for the acceleration of any time period relating to the exercise or payment of the award; (ii) provide for termination of any awards not exercised prior to the occurrence of a change in control; provided that the holder of any such award is given written notice of such prospective action by the administrator at least ten calendar days prior to the effective date of the change in control; (iii) provide for payment to the holder of the award of cash or other property with a fair market value equal to the amount that would have been received upon the exercise or payment of the award had the award been exercised or paid upon the change in control in exchange for cancellation of the award; (iv) adjust the terms of the award in a manner determined by the compensation committee to reflect the change in control; (v) cause the award to be assumed, or new rights substituted therefor, by another entity; or (vi) make such other provision as the compensation committee may consider equitable to the holders of awards and in our best interests.

2013 Employee Stock Purchase Plan

Concurrently with this offering, we are establishing our 2013 Employee Stock Purchase Plan, or the ESPP. Our board of directors has adopted the ESPP, and our stockholders have approved it, effective upon the closing of this offering. Our executive officers and all of our other employees will be allowed to participate in our ESPP. A total of 128,571 shares of our common stock will be reserved for issuance under our ESPP, subject to the eligibility requirements described below. Our compensation committee has full and exclusive authority to interpret the terms of the ESPP and determine eligibility.

Our employees are eligible to participate at the beginning of the first offering period that begins following their commencement of employment with us. However, an employee may not be granted rights to purchase stock under our ESPP if such employee:

- is not customarily employed at least 20 hours per week and more than five months in a calendar year;
- immediately after the grant would own stock possessing 5% or more of the total combined voting power or value of all classes of our capital stock; or
- holds rights to purchase stock under all of our employee stock purchase plans that would accrue at a rate that exceeds $25,000 worth of our stock for each calendar year.

Our ESPP is intended to qualify under Code Section 423, and provides for consecutive 6-month offering periods. The offering periods generally start on the first trading day on or after January 1 and July 1 of each year. Each offering period will begin after one exercise date and will end with the next exercise date approximately six months later. The administrator may, in its discretion, modify the terms of future offering periods.

Our ESPP permits participants to purchase common stock through payroll deductions of up to 15% of their compensation. On the last trading day of each offering period, each participant will be automatically granted an option to purchase shares of our common stock. The option will be immediately exercisable for a number of shares equal to the lowest of (a) a number equal to the quotient of the aggregate payroll deductions that have been withheld for the account of the participant during the offering period divided by the purchase price for the shares (as described below); (b) 28 shares; or (c) such other lesser maximum number of shares as the administrator may determine prior to the commencement of the offering period.

The purchase price for the shares will be 85% of the fair market value of our common stock on the first or last trading day of the offering period, whichever is less. Participants may end their participation at any time during an offering period, and will be paid their accrued payroll deductions that have not yet been used to purchase shares of common stock. Participation ends automatically upon termination of employment with us.

A participant may not transfer rights granted under the ESPP other than by will, the laws of descent and distribution or as otherwise provided under the ESPP.

In the event of our merger or change in control, as defined under the ESPP, a successor corporation may assume or substitute each outstanding purchase right. If the successor corporation refuses to assume or substitute for the outstanding purchase rights, the offering period then in progress will be shortened, and a new exercise

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date will be set which will occur prior to the proposed merger or change in control. The administrator will notify each participant in writing that the exercise date has been changed and that the participant s option will be exercised automatically on the new exercise date unless the participant has already withdrawn from the offering period.

Our ESPP will automatically terminate in 2024, unless we terminate it sooner. In addition, our board of directors or our compensation committee has the authority to amend, suspend or terminate our ESPP, except that, subject to certain exceptions described in the ESPP, no such action may adversely affect any outstanding rights to purchase stock under our ESPP.

401(k) Retirement Plan

We maintain a 401(k) retirement plan through our professional employer organization that is intended to be a tax-qualified defined contribution plan under Section 401(k) of the Code. In general, all of our employees are eligible to participate, beginning on the first day of the month following commencement of their employment. The 401(k) plan includes a salary deferral arrangement pursuant to which participants may elect to reduce their current compensation by up to the statutorily prescribed limit and have the amount of the reduction contributed to the 401(k) plan. We match participant contributions to the 401(k) plan up to 4% of a participant s annual compensation, subject to statutory limits.

Limitation of Liability and Indemnification

Our amended and restated certificate of incorporation includes provisions that will limit or eliminate the personal liability of our directors to the maximum extent permitted by Delaware law. Delaware law provides that directors of a corporation will not be personally liable for monetary damages for breaches of their 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;
- any unlawful payments related to dividends or unlawful stock repurchases, redemptions or other distributions; or
- any transaction from which the director derived an improper personal benefit.

These limitations do not apply to liabilities arising under federal securities laws and do not affect the availability of equitable remedies, including injunctive relief or rescission. If Delaware law is amended to authorize the further elimination or limiting of a director, then the liability of our directors will be eliminated or limited to the fullest extent permitted by Delaware law as so amended.

As permitted by Delaware law, our certificate of incorporation that will be effective as of the closing date of this offering will also provide that:

- we will indemnify our directors and officers to the fullest extent permitted by law;
- we may indemnify our other employees and other agents to the same extent that we indemnify our officers and directors, unless otherwise determined by our board of directors; and
- we will advance expenses to our directors and officers in connection with legal proceedings in connection with a legal proceeding to the fullest extent permitted by law.

The indemnification provisions contained in our certificate of incorporation that will be effective as of the closing date of this offering are not exclusive.

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We believe that these provisions are necessary to attract and retain qualified persons as directors and officers. Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers or persons controlling our company pursuant to the foregoing provisions, we understand that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

In addition, we have entered into indemnification agreements with each of our directors and maintain standard policies of insurance under which coverage is provided to our directors and officers against losses arising from claims made by reason of breach of duty or other wrongful act, and to us with respect to payments which may be made by us to such directors and officers pursuant to the above indemnification provisions or otherwise as a matter of law.

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RELATED PERSON TRANSACTIONS

The following is a description of transactions since July 1, 2011 to which we have been a party, and in which any of our directors, executive officers or beneficial owners of more than 5% of our voting securities, or any of their respective affiliates or immediate family members, had or will have a direct or indirect material interest. We believe the terms obtained or consideration that we paid or received, as applicable, in connection with the transactions described below were comparable to terms available or the amounts that would be paid or received, as applicable, from unrelated third parties.

2012 Bridge Loan and Series B Preferred Stock Financing

In May 2012, we issued and sold convertible promissory notes, which we refer to as our May 2012 notes, in an aggregate principal amount of $0.7 million to five holders of more than 5% of our voting securities. In connection with the issuance of the May 2012 notes, we issued to each purchaser warrants to purchase either (i) shares of the series of preferred stock issued in our next equity financing, at an exercise price equal to the amount per share paid by investors in such next equity financing, or (ii) at any time prior to such next equity financing, shares of our Series A-1 preferred stock, at an exercise price to be determined based on our fully-diluted capitalization at the time of exercise, with the number shares of preferred stock subject to each warrant equal to the quotient of (a) 20% of the principal amount of the note purchased by the applicable purchaser, divided by (b) the applicable exercise price of the warrant.

In connection with the November 2012 closing of our Series B preferred stock financing, these warrants became exercisable for an aggregate of 1,421,918 shares of our Series B-1 preferred stock at an exercise price of $0.1297 per share (or $4.54 on an as-converted to common stock basis).

The following table sets forth the aggregate principal amount of promissory notes and the number of shares of Series B-1 preferred stock underlying the warrants that were issued to our directors, executive officers and holders of more than 5% of our voting securities, and their affiliates or immediate family members.

Purchaser	Principal amount of convertible promissory notes	Number of shares Series B-1 Preferred Stock Underlying Warrants (#)
Intersouth Partners VI, L.P.	$ 202,480	312,228
Entities affiliated with InterWest Partners	$ 270,010	416,361
MedImmune Ventures, Inc.	$ 270,010	416,361

In November 2012, pursuant to our Series B Purchase Agreement, we issued and sold 66,147,709 shares of our Series B-1 preferred stock for aggregate consideration of $7.8 million in cash plus the conversion of all outstanding principal and accrued interest on the May 2012 notes. In April 2013, as part of the same financing pursuant to the Series B Purchase Agreement, we issued and sold 122,749,634 shares of our Series B-2 preferred stock for aggregate cash consideration of $18.2 million.

Pursuant to the Series B Purchase Agreement, the holders of our Series B preferred stockholders were entitled to purchase an aggregate of 58,816,897 shares of our Series B-3 preferred stock for an aggregate of $10.7 million. The Series B holders exercised this right and we completed the sale of these Series B-3 shares on November 5, 2013.

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The following table sets forth the number of shares of Series B-1 preferred stock, Series B-2 preferred stock and Series B-3 preferred stock that were issued to our directors, executive officers and holders of more than 5% of our voting securities, and their affiliates or immediate family members.

Purchaser	Shares of Series B-1 preferred stock	Series B-1 purchase price	Shares of Series B-2 preferred stock	Series B-2 purchase price	Shares of Series B-3 preferred stock	Series B-3 purchase price
Alta Partners VIII, L.P.	26,473,934	$ 3,429,000	49,060,606	$ 7,285,500	23,507,953	$ 4,285,500
S.R. One, Limited	17,625,289	$ 2,286,000	32,707,070	$ 4,857,000	15,671,969	$ 2,857,000
Entities affiliated with InterWest Partners	6,409,436	$ 831,304(1)	11,893,926	$ 1,766,248	5,699,111	$ 1,038,948
Intersouth Partners VI, L.P.	4,806,416	$ 623,392(2)	8,919,218	$ 1,324,504	4,273,746	$ 779,104
MedImmune Ventures, Inc.	6,409,436	$ 831,304(1)	11,893,926	$ 1,766,248	5,699,111	$ 1,038,948
Osage University Partners I, L.P.	4,406,322	$ 571,500	8,176,767	$ 1,214,250	3,917,992	$ 714,250

(1) Includes conversion of an aggregate of $281,591 in principal and accrued interest on May 2012 notes.
(2) Includes conversion of an aggregate of $211,164 in principal and accrued interest on May 2012 notes.

Agreements with Our Stockholders

In November 2012, in connection with our Series B preferred stock financing, we entered into an amended and restated investor rights agreement with the purchasers of our preferred stock and certain holders of our common stock. Under the amended and restated investor rights agreement, those holders have the right to demand that we file a registration statement, subject to certain limitations, and to request that their shares be covered by a registration statement that we may otherwise file. See Description of Capital Stock Registration Rights for additional information. Under the amended and restated investor rights agreement, holders of our preferred stock also have a participation right to purchase their pro rata share of new securities that we may propose to sell and issue, from time to time. The participation right does not apply to this offering, and will terminate upon the closing of this offering.

In connection with our Series B preferred stock financing, we also entered into an amended and restated right of first refusal and co-sale agreement and an amended and restated voting agreement with certain purchasers of our common stock and preferred stock. The amended and restated right of first refusal and co-sale agreement provides for rights of first refusal and co-sale rights in respect of sales of securities by certain holders of our capital stock. The amended and restated voting agreement contains provisions with respect to the election of our board of directors and its composition. The amended and restated right of first refusal and co-sale agreement and the amended and restated voting agreement will each terminate upon the closing of this offering.

On October 22, 2013, the holders of a majority of the shares of our preferred stock, on behalf of all of the parties to the amended and restated investor rights agreement, agreed to waive their rights under the amended and restated investor rights agreement to require inclusion of our securities held by them in the registration statement for this offering.

Some of our directors are associated with our principal stockholders as indicated in the table below:

Director	Principal Stockholder
Ed Hurwitz	Alta Partners VIII, L.P.(1)
Jill Carroll	S.R. One, Limited
Arnold L. Oronsky, Ph.D.	Entities affiliated with InterWest Partners
Sam Wu, M.D., Ph.D.	MedImmune Ventures, Inc.
James Rosen	Intersouth Partners VI, L.P.

(1) From 2006 through December 2013, Mr. Hurwitz was a director of the general partner of Alta Partners VIII, L.P. Mr. Hurwitz is a director of the general partner of Alta Biopharma III, L.P., a fund affiliated with Alta Partners and continues to serve as a consultant to that firm

and as a board representative on its portfolio companies.

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Indemnification of Officers and Directors

Our certificate of incorporation that will be effective as of the closing date of this offering provides that we will indemnify our directors and officers to the fullest extent permitted by Delaware law. In addition, we have entered into indemnification agreements with each of our directors that are broader in scope than the specific indemnification provisions contained in the Delaware General Corporation Law. See the Executive Compensation Limitation of Liability and Indemnification section of this prospectus for a further discussion of these arrangements.

Policies and Procedures for Related Person Transactions

While we have not historically had a written policy with respect to the review and approval of transactions with our directors, officers and principal stockholders, it has been the practice of our board of directors to review all interested party transactions and not to authorize any such transaction unless the board of directors, excluding any interested directors, determines that the terms of the proposed transaction are as favorable or more favorable to our company than would be available from an unrelated party in an arms length negotiation. Pursuant to the amended and restated charter of our audit committee that we expect to become effective upon the closing of this offering, our audit committee will be responsible for reviewing and approving in advance any related person transactions. For the purposes of this policy, a related person transaction is any transaction between us or any of our subsidiaries and any (a) of our directors or executive officers, (b) nominee for election as a director, (c) person known to us to own more than five percent of any class of our voting securities, or (d) member of the immediate family of any such person, if the nature of such transaction is such that it would be required to be disclosed under Item 404 of Regulation S-K (or any similar successor provision).

In determining whether to approve a related person transaction, the audit committee will take into account, among other factors it deems appropriate, whether the related person transaction is on terms no less favorable than terms generally available to an unaffiliated third-person under the same or similar circumstances and the extent of the related person s interest in the transaction.

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PRINCIPAL STOCKHOLDERS

The following table sets forth certain information with respect to beneficial ownership of our common stock, as of December 31, 2013, by:

- each person or entity, or group of affiliated persons or entities, known by us to beneficially own more than 5% of our common stock;
- each of our directors;
- each of our named executive officers; and
- all of our executive officers and directors as a group.

Beneficial ownership is determined in accordance with the rules of the SEC. In computing the number of shares beneficially owned by a person and the percentage ownership of that person, shares of common stock subject to options or warrants held by that person that are currently exercisable or exercisable within 60 days of December 31, 2013 are deemed outstanding, but are not deemed outstanding for computing the percentage ownership of any other person. To our knowledge, except as set forth in the footnotes to this table and subject to applicable community property laws, each person named in the table has sole voting and investment power with respect to the shares set forth opposite such person s name. Except as otherwise indicated, the address of each of the persons in this table is c/o Applied Genetic Technologies Corporation, 11801 Research Drive, Suite D, Alachua, Florida 32615.

Each stockholder s percentage ownership before the offering is determined in accordance with Rule 13d-3 under the Exchange Act and is based on 109,039 shares of our common stock outstanding as of December 31, 2013, plus 9,120,081 shares of common stock into which our outstanding preferred stock will convert upon the closing of this offering. Each stockholder s percentage ownership after the offering assumes the issuance of the 3,571,000 shares of our common stock offered hereby and assumes no exercise of the underwriters over-allotment option. Except as otherwise set forth under the heading Right to Acquire, the table below assumes no exercise of stock options and warrants outstanding at December 31, 2013 to purchase an aggregate of 842,212 shares of our common stock. Amounts under the heading Right to Acquire represent shares that may be acquired upon exercise of outstanding stock options or warrants exercisable within 60 days of December 31, 2013.

Certain of our existing stockholders have indicated an interest in purchasing up to an aggregate of approximately $10.0 million in shares of our common stock in this offering at the initial public offering price. The information set forth in the table below does not reflect the potential purchase of any shares in this offering by these stockholders.

Name of Beneficial Owner	Shares Outstanding	Right to Acquire	Total	Before the Offering	After the Offering
Alta Partners VIII, L.P. (1)	2,828,755		2,828,755	30.7%	22.1%
S.R. One, Limited (2)	1,885,836		1,885,836	20.4%	14.7%
Entities affiliated with InterWest Partners (3)	1,376,480	15,008	1,391,488	15.1%	10.9%
MedImmune Ventures, Inc. (4)	1,376,461	15,010	1,391,471	15.1%	10.9%
Intersouth Partners VI, L.P. (5)	1,142,666	12,034	1,154,700	12.5%	9.0%
Osage University Partners I, L.P. (6)	471,457		471,457	5.1%	3.7%
Susan B. Washer (7)		78,946	78,946	*	*
Jeffrey D. Chulay, M.D. (8)		22,804	22,804	*	*
David R. Knop, Ph.D. (9)		8,848	8,848	*	*
Scott Koenig, M.D., Ph.D. (10)		22,994	22,994	*	*
Jill Carroll (2)				*	*
Edward Hurwitz (1)				*	*
Arnold L. Oronsky, Ph.D. (3)				*	*
James Rosen (5)				*	*
Samuel Wu, M.D., Ph.D. (4)				*	*

All current executive officers and directors (11 persons)	145,936	145,936	1.4%	1.3%

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* Less than 1.0%

(1) Consists of (i) 755,369 shares of common stock issuable upon conversion of Series B-1 preferred stock, (ii) 1,401,731 shares of common stock issuable upon conversion of Series B-2 preferred stock and (iii) 671,655 shares of common stock issuable upon conversion of Series B-3 preferred stock. The address of Alta Partners VIII, L.P. is One Embarcadero Center, 37th Floor, San Francisco, California 94111. Alta Partners Management VIII, LLC is the general partner of Alta Partners VIII, L.P. and shares voting and dispositive power over the shares of our common stock held by Alta Partners VIII, L.P. Farah Champsi, Daniel Janney, and Guy Nohra are the managing directors of Alta Partners Management VIII, LLC and share dispositive and voting control over the shares of our common stock held by Alta Partner VIII, L.P. From 2006 through December 2013, Ed Hurwitz, a member of our board of directors, was a director of Alta Partners Management VIII, LLC.

(2) Consists of (i) 503,579 shares of common stock issuable upon conversion of Series B-1 preferred stock, (ii) 934,487 shares of common stock issuable upon conversion of Series B-2 preferred stock and (iii) 447,770 shares of common stock issuable upon conversion of Series B-3 preferred stock. The address of S.R. One, Limited is 161 Washington Street, Suite 500, Conshohocken, Pennsylvania 19428. Jill Carroll, a member of our board of directors, is a senior associate of S.R. One, Limited.

(3) Consists of (i) 690,699 shares of common stock issuable upon conversion of Series A-1 preferred stock, (ii) 183,126 shares of common stock issuable upon conversion of Series B-1 preferred stock, (iii) 339,825 shares of common stock issuable upon conversion of Series B-2 preferred stock, (iv) 162,830 shares of common stock issuable upon conversion of Series B-3 preferred stock, (v) 3,113 shares of common stock issuable upon conversion of Series A-1 preferred stock underlying warrants to purchase shares of our Series A-1 preferred stock exercisable within 60 days of the date of this table, and (vi) 11,895 shares of common stock issuable upon conversion of Series B-1 preferred stock underlying warrants to purchase shares of our Series B-1 preferred stock exercisable within 60 days of the date of this table. InterWest Partners VIII, L.P., InterWest Investors VIII, L.P., and InterWest Investors Q VIII, L.P. are collectively referred to as the entities affiliated with InterWest Partners. InterWest Management Partners VIII, LLC is the general partner of the entities affiliated with InterWest Partners and has sole voting and investment control over the shares held by the entities affiliated with InterWest Partners. Harvey B. Cash, Philip T. Gianos, W. Scott Hedrick, W. Stephen Holmes, Gilbert H. Kliman and Arnold L. Oronsky, a member of our board of directors, are the managing directors of InterWest Management Partners VIII, LLC. Each of the managing directors share voting and investment control with respect to the shares held by the entities affiliated with InterWest Partners. The address for these entities is c/o InterWest Partners, 2710 Sand Hill Road, Second Floor, Menlo Park, California 94025.

(4) Consists of (i) 690,678 shares of common stock issuable upon conversion of Series A-1A preferred stock, (ii) 183,126 shares of common stock issuable upon conversion of Series B-1 preferred stock, (iii) 339,826 shares of common stock issuable upon conversion of Series B-2 preferred stock, (iv) 162,831 shares of common stock issuable upon conversion of Series B-3 preferred stock, (v) 3,114 shares of common stock issuable upon conversion of Series A-1 preferred stock underlying warrants to purchase shares of our Series A-1 preferred stock exercisable within 60 days of the date of this table, and (vi) 11,896 shares of common stock issuable upon conversion of Series B-1 preferred stock underlying warrants to purchase shares of our Series B-1 Preferred Stock exercisable within 60 days of the date of this table. The address of MedImmune Ventures, Inc. is One MedImmune Way, Gaithersburg, Maryland 20878. Sam Wu, a member of our board of directors, is a managing director of MedImmune Ventures, Inc.

(5) Consists of (i) 628,399 shares of common stock issuable upon conversion of Series A-1 preferred stock, (ii) 137,326 shares of common stock issuable upon conversion of Series B-1 preferred stock, (iii) 254,834 shares of common stock issuable upon conversion of Series B-2 preferred stock, (iv) 122,107 shares of common stock issuable upon conversion of Series B-3 preferred stock, (v) 3,114 shares of common stock issuable upon conversion of Series A-1 preferred stock underlying warrants to purchase shares of our Series A-1 preferred stock exercisable within 60 days of the date of this table, and (vi) 8,920 shares of common stock issuable upon conversion of Series B-1 preferred stock underlying warrants to purchase shares of our Series B-1 preferred stock exercisable within 60 days of the date of this table. The address of Intersouth Partners VI, L.P. is 102 City Hall Plaza, Suite 200, Durham, North Carolina 27701. Mitchell Mumma and

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Dennis Dougherty are the managing members of Intersouth Associates VI, LLC, the sole general partner of Intersouth Partners VI, L.P., and share the power to vote or direct the voting of and to dispose or direct the disposition of the shares of our common stock held by Intersouth Partners VI, L.P. James Rosen, a member of our board of directors, is a Partner at Intersouth Associates VI, LLC.

(6) Consists of (i) 125,894 shares of common stock issuable upon conversion of Series B-1 preferred stock, (ii) 233,621 shares of common stock issuable upon conversion of Series B-2 preferred stock and (iii) 111,952 shares of common stock issuable upon conversion of Series B-3 preferred stock. The address of Osage University Partners I, L.P. is 50 Monument Road, Suite 201, Bala Cynwyd, Pennsylvania 19004. Osage University GP, LP is the general partner of Osage University Partners I, L.P. Osage Partners, LLC is the general partner of Osage University GP, LP. William Harrington, Marc Singer and Robert Adelson share the power to vote or direct the voting of and to dispose or direct the disposition of the shares of our common stock held by Osage University Partners I, L.P.

(7) Excludes 207,986 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(8) Excludes 60,688 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(9) Excludes 18,433 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(10) Excludes 35,179 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

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DESCRIPTION OF CAPITAL STOCK

The following section contains a description of our common stock and other securities that we have issued from time to time. Our authorized capital stock immediately prior to this offering consists of 410,000,000 shares of common stock, $0.001 par value per share, and 332,091,376 shares of preferred stock, $0.001 par value per share. As of December 31, 2013, we had 109,039 shares of common stock issued and outstanding, 281,660,161 shares of preferred stock issued and outstanding, 782,947 shares of common stock potentially issuable pursuant to outstanding stock options, and 59,247 shares of common stock potentially issuable pursuant to outstanding stock warrants to purchase preferred stock. The numbers of outstanding shares of common stock set forth above and in the discussion below give effect to a 1-for-35 reverse split of our common stock effected on March 4, 2014. As of December 31, 2013, there were 28 holders of record of our common stock.

Common Stock

Voting rights. Holders of our common stock are entitled to one vote per share held of record on all matters to be voted upon by our stockholders. The election of directors by our stockholders is determined by a plurality of the votes cast by the stockholders entitled to vote on the election. Other matters subject to a vote by our stockholders are decided by the affirmative vote of our stockholders having a majority in voting power of the votes cast by the stockholders present or represented and voting on such matter. Our common stock does not have cumulative voting rights.

Dividends. Subject to preferences that may be applicable to the holders of any outstanding shares of our preferred stock, the holders of our common stock are entitled to receive such lawful dividends as may be declared by our board of directors.

Liquidation and dissolution. In the event of our liquidation, dissolution or winding up, and subject to the rights of the holders of any outstanding shares of our preferred stock, the holders of shares of our common stock will be entitled to receive pro rata all of our remaining assets available for distribution to our stockholders.

Other rights and restrictions. Our certificate of incorporation does not permit us to redeem shares of our common stock at our election, provide for a sinking fund with respect to our common stock or provide for the granting of preemptive rights to any stockholder. All outstanding shares are fully paid and nonassessable.

Preferred Stock

Immediately prior to this offering, our certificate of incorporation provided for five series of preferred stock. In connection with this offering, all outstanding shares of preferred stock will be converted into shares of common stock in accordance with the provisions of our certificate of incorporation.

Upon the closing of this offering, our board of directors will be authorized, without stockholder approval, from time to time to issue up to 5,000,000 shares of preferred stock in one or more series, each of the series to have such rights and preferences, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, as the board of directors may determine. The rights of the holders of common stock will be subject to, and may be adversely affected by, the rights of holders of any preferred stock that we may issue in the future. The issuance of preferred stock, while providing desirable flexibility in connection with possible acquisitions and other corporate purposes, could have the effect of making it more difficult for others to acquire, or of discouraging others from attempting to acquire, a majority of our outstanding voting stock. We have no current plans to issue any shares of preferred stock.

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Options

As of December 31, 2013, options to purchase 782,947 shares of our common stock were outstanding under our 2001 Stock Option Plan and our 2011 Stock Incentive Plan, at a weighted average exercise price of $3.23 per share.

Warrants

As of December 31, 2013, we had outstanding warrants to purchase 59,247 shares of our common stock at a weighted average exercise price of $8.16 per share.

Registration Rights

Under the terms of an investor rights agreement between us and certain of our investors, the holders of approximately 7.5 million shares of common stock and warrants to purchase up to approximately 0.1 million shares of common stock, or their transferees, have the right to require us to register their shares with the SEC so that those shares may be publicly resold, or to include their shares in certain registration statements we file.

Demand registration rights. At any time on or after the date that is 180 days after the effective date of the registration statement for this offering, the holders who in the aggregate hold more than 50% of the shares having registration rights have the right to demand that we file up to two resale registration statements. These registration rights are subject to specified conditions and limitations, including the right of the underwriters to limit the number of shares included in any such registration under certain circumstances.

Form S-3 registration rights. If we are eligible to file a registration statement on Form S-3, each holder of shares having registration rights has the right to demand that we file up to two resale registration statements per year for such holder on Form S-3 so long as the aggregate offering price, net of any underwriters discounts or commissions, of securities to be sold under the registration statement on Form S-3 is at least $3,000,000, subject to specified exceptions, conditions and limitations.

Piggyback registration rights. If we register any securities for public sale, stockholders with registration rights will have the right to include their shares in the registration statement. The underwriters of any underwritten offering will have the right to limit the number of shares included in such offering for the account of stockholders with registration rights.

Expenses of registration. We will pay all expenses, other than underwriting discounts and commissions, relating to all demand registrations, Form S-3 registrations and piggyback registrations.

Expiration of registration rights. The registration rights described above will terminate upon the earlier of the fifth anniversary of the closing of this offering and, as to a given holder of registrable securities, when such holder of registrable securities, together with its affiliates, holds less than 1% of the outstanding shares of our common stock and all of such holder s and such holder s affiliates registrable securities may be sold during a 90 pursuant to Rule 144 promulgated under the Securities Act.

If our stockholders with registration rights cause a large number of securities to be registered and sold in the public market, those sales could cause the market price of our common stock to fall. If we were to initiate a registration and include registrable securities because of the exercise of registration rights, the inclusion of registrable securities could adversely affect our ability to raise capital.

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Anti-Takeover Effects of Provisions of Delaware Law and Our Charter and By-laws

Provisions of Delaware law and our certificate of incorporation and by-laws could make it more difficult to acquire us by means of a tender offer, a proxy contest, open market purchases, removal of incumbent directors and otherwise. These provisions, summarized below, are expected to discourage types of coercive takeover practices and inadequate takeover bids and to encourage persons seeking to acquire control of us to first negotiate with us. We believe that the benefits of increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure us outweigh the disadvantages of discouraging takeover or acquisition proposals because negotiation of these proposals could result in an improvement of their terms.

We must comply with Section 203 of the Delaware General Corporation Law, an anti-takeover law. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a business combination with an interested stockholder for a period of three years following the date the person became an interested stockholder, unless the business combination or the transaction in which the person became an interested stockholder is approved in a prescribed manner. Generally, a business combination includes a merger, asset or stock sale, or other transaction resulting in a financial benefit to an interested stockholder. An interested stockholder includes a person who, together with affiliates and associates, owns, or did own within three years before the determination of interested stockholder status, 15% or more of the corporation s voting stock. The existence of this provision generally will have an anti-takeover effect for transactions not approved in advance by the board of directors, including discouraging attempts that might result in a premium over the market price for the shares of common stock held by stockholders.

Upon the closing of this offering, our certificate of incorporation and by-laws will require that any action required or permitted to be taken by our stockholders must be effected at a duly called annual or special meeting of the stockholders and may not be effected by a consent in writing. In addition, upon the closing of this offering, special meetings of our stockholders may be called only by the board of directors and some of our officers. Our by-laws establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of candidates for election to our board of directors. Our certificate of incorporation and by-laws also provide that, effective upon the closing of this offering, our board of directors will be divided into three classes, with each class serving staggered three-year terms. These provisions may have the effect of deterring hostile takeovers or delaying changes in our control or management.

Listing on the NASDAQ Global Market

We have applied to have our common stock listed on the NASDAQ Global Market under the symbol AGTC.

Authorized but Unissued Shares

The authorized but unissued shares of common stock and preferred stock are available for future issuance without stockholder approval, subject to any limitations imposed by the NASDAQ Listing Rules. These additional shares may be used for a variety of corporate finance transactions, acquisitions and employee benefit plans. The existence of authorized but unissued and unreserved common stock and preferred stock could make it more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tender offer, merger or otherwise.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is Computershare Trust Company, N.A.

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SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our common stock, and a liquid public trading market for our common stock may not develop or be sustained after this offering. Future sales of significant amounts of our common stock, including shares issued upon exercise of outstanding options or warrants or in the public market after this offering, or the anticipation of those sales, could adversely affect the public market prices prevailing from time to time and could impair our ability to raise capital through sales of our equity securities. We intend to apply to have our common stock listed on the NASDAQ Global Market under the symbol AGTC.

Upon the closing of this offering, and after giving effect to the issuance of the 3,571,000 shares of our common stock offered in this offering and the conversion of our outstanding shares of preferred stock into 9,120,081 shares of common stock upon the closing of this offering, we will have outstanding an aggregate of 12,800,120 shares of common stock, assuming no exercise of outstanding options or warrants after December 31, 2013. Of these shares, the 3,571,000 shares sold by us (assuming that the underwriters do not exercise their over-allotment option), in this offering will be freely tradable without restriction or further registration under the Securities Act, except for any shares purchased by our affiliates, as that term is defined in Rule 144 under the Securities Act, whose sales would be subject to the Rule 144 resale restrictions described below, other than the holding period requirement.

The remaining 9,229,120 shares of common stock will be restricted securities, as that term is defined in Rule 144 under the Securities Act and will further be subject to either restrictions on transfer under the lock-up agreements described below or restrictions on transfer for a period of 180 days from the effectiveness of the registration statement of which this prospectus forms a part under stock option agreements entered into between us and the holders of those shares. Following the expiration of these restrictions, these shares will become eligible for public sale if they are registered under the Securities Act or if they qualify for an exemption from registration under Rules 144 or 701 under the Securities Act, which are summarized below.

In addition, of the 782,947 shares of common stock that were issuable pursuant to stock options outstanding under our 2001 Stock Option Plan and our 2011 Stock Incentive Plan as of December 31, 2013, options to purchase 201,686 shares of common stock had vested and were exercisable as of December 31, 2013. Upon exercise, these shares will be eligible for sale, subject to the lock-up agreements and securities laws described below. All of the 59,247 shares of common stock that were issuable pursuant to warrants outstanding as of December 31, 2013, were exercisable as of December 31, 2013 and upon issuance these shares will be eligible for sale, subject to the lock-up agreements and securities laws described below.

Rule 144

Affiliate Resales of Restricted Securities

In general, beginning 90 days after the effective date of the registration statement of which this prospectus forms a part, a person who is an affiliate of ours, or who was an affiliate at any time during the 90 days before a sale, who has beneficially owned shares of our common stock for at least six months would be entitled to sell in broker s transactions or certain riskless principal transactions or to market makers, a number of shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately 128,291 shares immediately after this offering; or
- the average weekly trading volume in our common stock on the NASDAQ Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to such sale.

Affiliate resales under Rule 144 are also subject to the availability of current public information about us. In addition, if the number of shares being sold under Rule 144 by an affiliate during any three-month period exceeds

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5,000 shares or has an aggregate sale price in excess of $50,000, the seller must file a notice on Form 144 with the SEC and the NASDAQ Stock Market concurrently with either the placing of a sale order with the broker or the execution directly with a market maker.

Non-Affiliate Resales of Restricted Securities

In general, beginning 90 days after the effective date of the registration statement of which this prospectus forms a part, a person who is not an affiliate of ours at the time of sale, and has not been an affiliate at any time during the three months preceding a sale, and who has beneficially owned shares of our common stock for at least six months but less than a year, is entitled to sell such shares subject only to the availability of current public information about us. If such person has held our shares for at least one year, such person can resell under Rule 144(b)(1) without regard to any Rule 144 restrictions, including the 90-day public company requirement and the current public information requirement.

Non-affiliate resales are not subject to the manner of sale, volume limitation or notice filing provisions of Rule 144.

Rule 701

In general, under Rule 701, any of our employees, directors, officers, consultants or advisors who purchased shares from us in connection with a compensatory stock or option plan or other written agreement entered into before the effective date of our initial public offering is entitled to sell such shares without further restriction under the Securities Act.

Lock-up Agreements

Our executive officers and directors and the holders of substantially all of our outstanding stock have agreed with the underwriters, subject to certain exceptions, not to dispose of or hedge any of their common stock or securities convertible into or exchangeable for shares of common stock for a period through the date 180 days after the date of this prospectus, except with the prior written consent of the representatives of the underwriters.

The representatives of the underwriters currently do not anticipate shortening or waiving any of the lock-up agreements and do not have any pre-established conditions for such modifications or waivers. The representatives of the underwriters may, however, with the approval of our board of directors, release for sale in the public market all or any portion of the shares subject to the lock-up agreements.

Registration Rights

Subject to the lock-up agreements described above, upon the closing of this offering, the holders of approximately 7.5 million shares of common stock and warrants to purchase up to approximately 0.1 million shares of common stock will have the right to require us to register these shares under the Securities Act under specified circumstances. After registration pursuant to these rights, these shares will become freely tradable without restriction under the Securities Act. See Description of Capital Stock Registration Rights for additional information regarding these registration rights.

Stock Options and Warrants

As of December 31, 2013, we had outstanding options to purchase 782,947 shares of common stock, of which options to purchase 201,686 shares of common stock were vested and exercisable. Following this offering, we intend to file registration statements on Form S-8 under the Securities Act to register all of the shares of common stock subject to outstanding options and options and other awards issuable pursuant to the 2013 Equity and Incentive Plan and the 2013 Employee Stock Purchase Plan.

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As of December 31, 2013, we also had outstanding and exercisable warrants to purchase 59,247 shares of common stock (calculated on an as-converted basis). Any shares purchased by our non-affiliates pursuant to the cashless exercise features of our warrants will be freely tradable under Rule 144(b)(1), subject in certain cases to the 180-day lock-up period described above. Any shares purchased through the exercise of these warrants for cash will be eligible for sale subject to the lock-up agreements and securities laws described above.

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MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS

The following is a general discussion of the material U.S. federal income tax considerations applicable to non-U.S. holders (as defined below) with respect to their purchase, ownership and disposition of shares of our common stock. This discussion is for general information only and is not tax advice. Accordingly, all prospective non-U.S. holders of our common stock should consult their tax advisors with respect to the U.S. federal, state, local and non-U.S. tax consequences of the purchase, ownership and disposition of our common stock.

This discussion is based on current provisions of the U.S. Internal Revenue Code of 1986, as amended, which we refer to as the Code, existing and proposed U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, all as in effect as of the date of this prospectus, all of which are subject to change or to differing interpretation, possibly with retroactive effect. Any change could alter the tax consequences to non-U.S. holders described in this prospectus. We assume in this discussion that a non-U.S. holder holds shares of our common stock as a capital asset (generally, property held for investment).

This discussion does not address all aspects of U.S. federal income taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder s individual circumstances nor does it address any aspects of U.S. federal estate or gift taxes, and state, local or non-U.S. taxes. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- insurance companies;
- tax-exempt organizations;
- financial institutions;
- brokers or dealers in securities or currencies;
- regulated investment companies;
- pension plans;
- controlled foreign corporations;
- passive foreign investment companies;
- persons subject to the alternative minimum tax;
- owners that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment; and
- certain U.S. expatriates.

In addition, this discussion does not address the tax treatment of partnerships or other pass-through entities, or persons who hold our common stock through partnerships or other pass-through entities, for U.S. federal income tax purposes. A partner in a partnership or other pass-through entity that will hold our common stock should consult his, her or its tax advisor regarding the tax consequences of acquiring, holding and disposing of our common stock through a partnership or other pass-through entity, as applicable.

We have not sought and will not seek any ruling from the Internal Revenue Service, which we refer to as the IRS, with respect to the statements made and the conclusions reached in the following discussion. There can be no assurance that the IRS will not challenge one or more of the tax consequences described herein, or that any such challenge would not be sustained by a court.

NON-U.S. HOLDERS ARE URGED TO CONSULT THEIR TAX ADVISORS WITH RESPECT TO THE APPLICATION OF THE U.S. FEDERAL TAX LAWS TO THEIR PURCHASE, OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK IN LIGHT OF THEIR PARTICULAR SITUATIONS, AS WELL AS ANY TAX CONSEQUENCES ARISING UNDER THE LAWS OF ANY STATE, LOCAL, FOREIGN OR OTHER TAXING JURISDICTION OR UNDER ANY APPLICABLE TAX TREATY.

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Non-U.S. Holder Defined

For purposes of this discussion, a non-U.S. holder means a beneficial owner of our common stock that, for U.S. federal income tax purposes, is an individual, corporation, estate or trust that is not a U.S. person. For purposes of this discussion, a U.S. person is:

- an individual who is a citizen or resident of the United States;
- a corporation, or any other organization taxable as a corporation for U.S. federal income tax purposes, created or organized in the United States or under the laws of the United States, any political subdivision thereof, any state thereof or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income tax regardless of its source; or
- a trust if (1) a U.S. court is able to exercise primary supervision over the trust s administration and one or more U.S. persons have the authority to control all of the trust s substantial decisions or (2) the trust has a valid election in effect to be treated as a U.S. person.

Distributions on Our Common Stock

As described in the section entitled Dividend Policy, we have not made distributions on our common stock and do not plan to make any distributions for the foreseeable future. However, if we do make distributions of cash or property on our common stock, those payments generally 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. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of the non-U.S. holder s investment, up to such holder s tax basis in the common stock. Any remaining excess will be treated as capital gain, subject to the tax treatment described below in Gain on Sale, Exchange or Other Disposition of Our Common Stock.

Subject to the discussion below on backup withholding and FATCA, dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty. A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty generally will be required to provide a properly executed IRS Form W-8BEN (or other appropriate version of IRS Form W-8 or successor form) and satisfy applicable certification and other requirements. Non-U.S. holders are urged to consult their tax advisors regarding their entitlement to benefits under any applicable income tax treaty.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States and, if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States, are generally exempt from the 30% withholding tax if the non-U.S. holder satisfies applicable certification and disclosure requirements by providing a properly executed IRS Form W-8ECI (or successor form). However, such U.S. effectively connected income, net of specified deductions and credits, is taxed at the same graduated U.S. federal income tax rates applicable to U.S. persons. In addition, any U.S. effectively connected income received by a non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional branch profits tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty.

A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing a U.S. tax return with the IRS.

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Gain on Sale, Exchange or Other Disposition of Our Common Stock

Subject to the discussion below on backup withholding and FATCA, a non-U.S. holder generally will not be subject to any U.S. federal income tax on any gain realized upon such holder s sale, exchange or other disposition of shares of our common stock unless:

- the gain is effectively connected with the non-U.S. holder s conduct of a U.S. trade or business and, if an applicable income tax treaty so provides, is attributable to a permanent establishment or a fixed base maintained by such non-U.S. holder in the United States, in which case the non-U.S. holder generally will be taxed at the graduated U.S. federal income tax rates applicable to U.S. persons and, if the non-U.S. holder is a foreign corporation, it also may be subject to a branch profits tax at a rate of 30% (or such lower rate as may be specified by an applicable income tax treaty) on such effectively connected gain.

- the non-U.S. holder is a nonresident alien individual who is present in the United States for 183 days or more in the taxable year of the disposition and certain other conditions are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty) on the net gain derived from the disposition, which may be offset by certain U.S. source capital losses of the non-U.S. holder, if any; or

- we are, or have been, at any time during the five-year period preceding such disposition (or the non-U.S. holder s holding period, if shorter) a U.S. real property holding corporation. Generally, a corporation is a U.S. real property holding corporation only if the fair market value of its U.S. real property interests equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. Although there can be no assurance, we do not believe that we are, or have been, a U.S. real property holding corporation, or that we are likely to become one in the future. Even if we are or were to become a U.S. real property holding corporation, gains realized by a non-U.S. holder on a disposition of our common stock will not be subject to U.S. federal income tax if our common stock is regularly traded on an established securities market and the non-U.S. holder holds no more than 5% of our outstanding common stock, directly or indirectly, during the shorter of the 5-year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. No assurance can be provided that our common stock will continue to be regularly traded on an established securities market for purposes of the rules described above.

Backup Withholding and Information Reporting

We must report annually to the IRS and to each non-U.S. holder payments of dividends on our common stock to such holder and the tax withheld, if any, with respect to such dividends, along with certain other information. Non-U.S. holders may have to comply with specific certification procedures to establish that the holder is not a U.S. person in order to avoid backup withholding with respect to dividends on our common stock. Dividends paid to non-U.S. holders subject to the U.S. withholding tax, as described above in Distributions on Our Common Stock, generally will be exempt from U.S. backup withholding.

Information reporting and backup withholding generally will apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their tax advisors regarding the application of the information reporting and backup withholding rules to them.

Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or other agreement.

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Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder can be refunded or credited against the non-U.S. holder s U.S. federal income tax liability, if any, provided that an appropriate claim is timely filed with the IRS.

FATCA Withholding and Information Reporting

The Foreign Account Tax Compliance Act of 2010, commonly referred to as FATCA, generally will impose a U.S. federal withholding tax at a rate of 30% on dividends on, or gross proceeds from the sale or other disposition of, our common stock paid to certain foreign entities (including foreign financial institutions and foreign intermediaries), unless such foreign entity satisfies various U.S. information reporting and due diligence requirements (generally relating to ownership by U.S. persons of interests in or accounts with the entity). The withholding tax rules generally will be applicable to dividends on our common stock that are paid after June 30, 2014, and to gross proceeds from a sale or other disposition of our common stock that occurs after December 31, 2016. Although U.S. Treasury Regulations implementing FATCA were recently finalized, these rules remain unclear in many respects and are subject to material changes. Prospective investors should consult their tax advisors regarding the possible implications of FATCA on their investment in our common stock.

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UNDERWRITING

BMO Capital Markets Corp. and Wedbush Securities, Inc. are acting as representatives of the underwriters and book-running managers of this offering. Under the terms of an underwriting agreement, which will be filed as an exhibit to the registration statement, each of the underwriters named below has severally agreed to purchase from us the respective number of shares of common stock shown opposite its name below:

Underwriters	Number of Shares
BMO Capital Markets Corp.	
Wedbush Securities Inc.	
Cantor Fitzgerald & Co.	
Roth Capital Partners, LLC	
Total	

The underwriting agreement provides that the underwriters obligation to purchase shares of common stock depends on the satisfaction of the conditions contained in the underwriting agreement including:

- the representations and warranties made by us to the underwriters are true;
- there is no material change in our business or the financial markets; and
- we deliver customary closing documents to the underwriters.

Commissions and Expenses

The following table summarizes the underwriting discounts and commissions we will pay to the underwriters. These amounts are shown assuming both no exercise and full exercise of the underwriters option to purchase additional shares. The underwriting fee is the difference between the initial price to the public and the amount the underwriters pay to us for the shares.

	No Exercise	Full Exercise
Per Share	$	$
Total	$	$

The representatives have advised us that the underwriters propose to offer the shares of common stock directly to the public at the public offering price on the cover page of this prospectus and to selected dealers, which may include the underwriters, at such offering price less a selling concession not in excess of $ per share. After the offering, the representatives may change the offering price and other selling terms.

The expenses of the offering that are payable by us are estimated to be approximately $1,650,000 (excluding underwriting discounts and commissions). We have also agreed to reimburse the underwriters for certain of their expenses, in an amount of up to $55,000, as set forth in the underwriting agreement.

Option to Purchase Additional Shares

We have granted the underwriters an option, exercisable for 30 days after the date of this prospectus, to purchase, from time to time, in whole or in part, up to an aggregate of 535,650 shares from us at the public offering price less underwriting discounts and commissions. This option may be exercised to the extent the underwriters sell more than 3,571,000 shares in connection with this offering. To the extent that this option is exercised, each underwriter will be obligated, subject to certain conditions, to purchase its pro rata portion of these additional shares based on

the underwriter s percentage underwriting commitment in the offering as indicated in the table at the beginning of this Underwriting Section.

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Lock-Up Agreements

We, all of our directors and executive officers, and holders of substantially all of our outstanding stock have agreed that, for a period of 180 days, or the lock-up period, after the date of this prospectus subject to certain limited exceptions described below, we and they will not directly or indirectly, without the prior written consent of BMO Capital Markets Corp., (1) offer for sale, sell, pledge, or otherwise dispose of (or enter into any transaction or device that is designed to, or could be expected to, result in the disposition by any person at any time in the future of) any shares of common stock (including, without limitation, shares of common stock that may be deemed to be beneficially owned by us or them in accordance with the rules and regulations of the SEC and shares of common stock that may be issued upon exercise of any options or warrants) or securities convertible into or exercisable or exchangeable for common stock, (2) enter into any swap or other derivatives transaction that transfers to another, in whole or in part, any of the economic benefits or risks of ownership of shares of common stock, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of common stock or other securities, in cash or otherwise, (3) make any demand for or exercise any right or cause to be filed a registration statement, including any amendments thereto, with respect to the registration of any shares of common stock or securities convertible into or exercisable or exchangeable for common stock or any of our other securities, or (4) publicly disclose the intention to do any of the foregoing.

These lock-up restrictions will not apply to: (1) transactions relating to shares of common stock or other securities acquired in the open market after the date of this prospectus; (2) bona fide gifts, sales or other dispositions made exclusively by the holder to the holder s family, partners, members, stockholders or affiliates (as applicable), and transfers or other dispositions by will, other testamentary documents or intestate succession, *provided*, that such transferee agrees to be bound by the terms of the lock-up agreement, the parties agree to not make any filing or public announcement regarding such transfer or disposition prior to the expiration of the lock-up period and the holder notifies BMO Capital Markets Corp. at least two business days prior to the proposed transfer or disposition; (3) the exercise of warrants or stock options granted pursuant to the Company s stock option/incentive plans or otherwise, or the conversion of securities, in each case outstanding on the date of this prospectus, *provided* that the restrictions shall apply to the shares of common stock issued upon such exercise or conversion; (4) the establishment of any trading plan established pursuant to Rule 10b5-1 under the Exchange Act, *provided* that no sales or securities convertible into common stock shall be made pursuant to such plan prior to the expiration of the lock-up period, and the Company does not, and is not required to, report the establishment of such plan in any public report or filing with the SEC under the Exchange Act prior to the expiration of the lock-up period; (5) any forfeiture, sale or other transfer to the company in connection with the termination of the holder s employment with or services to the company; and (6) the transfer of shares to the company to satisfy withholding taxes for any equity award granted prior to the date of this prospectus.

BMO Capital Markets Corp. may release the common stock and other securities subject to the lock-up agreements described above in whole or in part at any time. When determining whether or not to release common stock and other securities from lock-up agreements, BMO Capital Markets Corp. will consider, among other factors, the holder s reasons for requesting the release, the number of shares of common stock and other securities for which the release is being requested and market conditions at the time. In addition, BMO Capital Markets Corp. has agreed that it will not grant any such release without the prior written consent of Wedbush Securities, Inc. BMO Capital Markets Corp. and Wedbush Securities, Inc. may make any such determination in their sole discretion.

At least three business days before the effectiveness of any release or waiver of any of the restrictions described above with respect to an officer or director of the Company, BMO Capital Markets Corp. and Wedbush Securities, Inc. will notify us of the impending release or waiver and we have agreed to announce the impending release or waiver by press release through a major news service at least two business days before the effective date of the release or waiver.

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Offering Price Determination

Prior to this offering, there has been no public market for our common stock. The initial public offering price was negotiated between the representatives and us. In determining the initial public offering price of our common stock, the representatives considered:

- the history and prospects for the industry in which we compete;
- our financial information;
- the ability of our management and our business potential and earning prospects;
- the prevailing securities markets at the time of this offering; and
- the recent market prices of, and the demand for, publicly traded shares of generally comparable companies.

Indemnification

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act, and to contribute to payments that the underwriters may be required to make for these liabilities.

Stabilization, Short Positions and Penalty Bids

The representatives may engage in stabilizing transactions, short sales and purchases to cover positions created by short sales, and penalty bids or purchases for the purpose of pegging, fixing or maintaining the price of the common stock, in accordance with Regulation M under the Exchange Act:

- Stabilizing transactions permit bids to purchase the underlying security so long as the stabilizing bids do not exceed a specified maximum.
- A short position involves a sale by the underwriters of shares in excess of the number of shares the underwriters are obligated to purchase in the offering, which creates the syndicate short position. This short position may be either a covered short position or a naked short position. In a covered short position, the number of shares involved in the sales made by the underwriters in excess of the number of shares they are obligated to purchase is not greater than the number of shares that they may purchase by exercising their option to purchase additional shares. In a naked short position, the number of shares involved is greater than the number of shares in their option to purchase additional shares. The underwriters may close out any short position by either exercising their option to purchase additional shares and/or purchasing shares in the open market. In determining the source of shares to close out the 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 their option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there could be downward pressure on the price of the shares in the open market after pricing that could adversely affect investors who purchase in the offering.
- Syndicate covering transactions involve purchases of the common stock in the open market after the distribution has been completed in order to cover syndicate short positions.

Penalty bids permit the representatives to reclaim a selling concession from a syndicate member when the common stock originally sold by the syndicate member is purchased in a stabilizing or syndicate covering transaction to cover syndicate short positions.

These stabilizing transactions, syndicate covering transactions and penalty bids 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 the common stock. As a result, the price of the common stock may be higher than the price that might otherwise exist in the open market. These transactions may be effected on the NASDAQ Global Market or otherwise and, if commenced, may be discontinued at any time.

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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 the common stock. In addition, neither we nor any of the underwriters make any representation that the representatives will engage in these stabilizing transactions or that any transaction, once commenced, will not be discontinued without notice.

Listing on The NASDAQ Global Market

We have applied to have our common stock approved for listing on the NASDAQ Global Market under the symbol AGTC.

Stamp Taxes

If you purchase shares of common stock offered in this prospectus, you may be required to pay stamp taxes and other charges under the laws and practices of the country of purchase, in addition to the offering price listed on the cover page of this prospectus.

Other Relationships

The underwriters and certain of their affiliates are full service financial institutions engaged in various activities, which may include securities trading, commercial and investment banking, financial advisory, investment management, investment research, principal investment, hedging, financing and brokerage activities. The underwriters and certain of their affiliates have, from time to time, performed, and may in the future perform, various commercial and investment banking and financial advisory services for the issuer and its affiliates, for which they received or may in the future receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and certain of 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, and such investment and securities activities may involve securities and/or instruments of the issuer or its affiliates. If the underwriters or their affiliates have a lending relationship with us, certain of those underwriters or their affiliates routinely hedge, and certain other of those underwriters or their affiliates may hedge, their credit exposure to us consistent with their customary risk management policies. Typically, the underwriters and their affiliates would hedge such exposure by entering into transactions which consist of either the purchase of credit default swaps or the creation of short positions in our securities or the securities of our affiliates, including potentially the shares of common stock offered hereby. Any such credit default swaps or short positions could adversely affect future trading prices of the shares of common stock offered hereby. The underwriters and certain of their affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such securities or instruments and may at any time hold, or recommend to clients that they acquire, long and/or short positions in such securities and instruments.

Selling Restrictions

This prospectus does not constitute an offer to sell to, or a solicitation of an offer to buy from, anyone in any country or jurisdiction (i) in which such an offer or solicitation is not authorized, (ii) in which any person making such offer or solicitation is not qualified to do so or (iii) in which any such offer or solicitation would otherwise be unlawful. No action has been taken that would, or is intended to, permit a public offer of the shares of common stock or possession or distribution of this prospectus or any other offering or publicity material relating to the shares of common stock in any country or jurisdiction (other than the United States) where any such action for that purpose is required. Accordingly, each underwriter has undertaken that it will not, directly or indirectly, offer or sell any shares of common stock or have in its possession, distribute or publish any prospectus, form of application, advertisement or other document or information in any country or jurisdiction except under circumstances that will, to the best of its knowledge and belief, result in compliance with any applicable laws and regulations and all offers and sales of shares of common stock by it will be made on the same terms.

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European Economic Area

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive, each, a Relevant Member State, an offer to the public of any common stock which are the subject of the offering contemplated herein may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of any common stock may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

- to legal entities which are qualified investors as defined under the Prospectus Directive;
- by the underwriters to fewer than 100, or, if the Relevant Member State has implemented the relevant provisions of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives of the underwriters for any such offer; or
- in any other circumstances falling within Article 3(2) of the Prospectus Directive,

provided that no such offer of common stock shall result in a requirement for us or any underwriter 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 in a Relevant Member State who receives any communication in respect of, or who acquires any common stock under, the offers contemplated here in this prospectus will be deemed to have represented, warranted and agreed to and with each underwriter and us that:

- it is a qualified investor as defined under the Prospectus Directive; and
- in the case of any common stock acquired by it as a financial intermediary, as that term is used in Article 3(2) of the Prospectus Directive, (i) the common stock acquired by it in the offering have not been acquired on behalf of, nor have they been acquired with a view to their offer or resale to, persons in any Relevant Member State other than qualified investors, as that term is defined in the Prospectus Directive, or in the circumstances in which the prior consent of the representatives of the underwriters has been given to the offer or resale or (ii) where common stock have been acquired by it on behalf of persons in any Relevant Member State other than qualified investors, the offer of such common stock to it is not treated under the Prospectus Directive as having been made to such persons.

For the purposes of this representation and the provision above, the expression an offer of common stock to the public in relation to any common stock in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any common stock to be offered so as to enable an investor to decide to purchase or subscribe for the common stock, as the same may be varied in that Relevant Member State by any measure implementing the Prospectus Directive in that Relevant Member State, the expression Prospectus Directive means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in each Relevant Member State and the expression 2010 PD Amending Directive means Directive 2010/73/EU.

United Kingdom

This prospectus has only been communicated or caused to have been communicated and will only be communicated or caused to be communicated as an invitation or inducement to engage in investment activity (within the meaning of Section 21 of the Financial Services and Markets Act of 2000, or the FSMA) as received in connection with the issue or sale of the common stock in circumstances in which Section 21(1) of the FSMA does not apply to us. All applicable provisions of the FSMA will be complied with in respect to anything done in relation to the common stock in, from or otherwise involving the United Kingdom.

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Switzerland

This document, as well as any other material relating to the shares which are the subject of the offering contemplated by this prospectus, do not constitute an issue prospectus pursuant to Article 652a and/or 1156 of the Swiss Code of Obligations. The shares will not be listed on the SIX Swiss Exchange and, therefore, the documents relating to the shares, including, but not limited to, this document, do not claim to comply with the disclosure standards of the listing rules of SIX Swiss Exchange and corresponding prospectus schemes annexed to the listing rules of the SIX Swiss Exchange. The shares are being offered in Switzerland by way of a private placement, i.e., to a small number of selected investors only, without any public offer and only to investors who do not purchase the shares with the intention to distribute them to the public. The investors will be individually approached by the issuer from time to time. This document, as well as any other material relating to the shares, is personal and confidential and does not constitute an offer to any other person. This document may only be used by those investors to whom it has been handed out in connection with the offering described herein and may neither directly nor indirectly be distributed or made available to other persons without express consent of the issuer. It may not be used in connection with any other offer and shall in particular not be copied and/or distributed to the public in (or from) Switzerland.

Hong Kong

The shares may not be offered or sold by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies Ordinance (Cap.32, Laws of Hong Kong), or (ii) to professional investors within the meaning of the Securities and Futures Ordinance (Cap.571, Laws of Hong Kong) and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a prospectus within the meaning of the Companies Ordinance (Cap.32, Laws of Hong Kong), and no advertisement, invitation or document relating to the shares may be issued or may be in the possession of any person for the purpose of issue (in each case 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 in Hong Kong (except if permitted to do so under the 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 within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Hong Kong) and any rules made thereunder.

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 the SFA, (ii) to a relevant person, 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 by a relevant person which is: (i) a corporation (which is not an accredited investor) 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 is an accredited investor, shares, debentures and units of shares and debentures of that corporation or the beneficiaries rights and interest in that trust shall not be transferable for 6 months after that corporation or that trust has acquired the shares under Section 275 except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person, or any person pursuant to Section 275(1A), and in accordance with the conditions, specified in Section 275 of the SFA; (2) where no consideration is given for the transfer; or (3) by operation of law.

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Japan

The securities have not been and will not be registered under the Financial Instruments and Exchange Law of Japan (the Financial Instruments and Exchange Law) and each underwriter has agreed that it will not offer or sell any securities, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan (which term as used herein means any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the Financial Instruments and Exchange Law and any other applicable laws, regulations and ministerial guidelines of Japan.

Electronic Distribution

A prospectus in electronic format may be made available on the Internet sites or through other online services maintained by one or more of the underwriters and/or selling group members participating in this offering, or by their affiliates. In those cases, prospective investors may view offering terms online and, depending upon the particular underwriter or selling group member, prospective investors may be allowed to place orders online. The underwriters may agree with us to allocate a specific number of shares for sale to online brokerage account holders. Any such allocation for online distributions will be made by the representatives on the same basis as other allocations.

Other than the prospectus in electronic format, the information on any underwriter s or selling group member s web site and any information contained in any other web site maintained by an underwriter or selling group member is not part of the prospectus or the registration statement of which this prospectus forms a part, has not been approved and/or endorsed by us or any underwriter or selling group member in its capacity as underwriter or selling group member and should not be relied upon by investors.

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LEGAL MATTERS

The validity of the common stock being offered will be passed upon for us by Foley Hoag LLP, Boston, Massachusetts. Certain legal matters in connection with this offering will be passed upon for the underwriters by Latham & Watkins LLP, Boston, Massachusetts.

EXPERTS

The balance sheets as of June 30, 2012 and 2013, and the related statements of operations, convertible preferred stock and stockholders (deficit) equity and cash flows for the years then ended, appearing in this prospectus have been audited by McGladrey LLP, an independent registered public accounting firm, as stated in their report appearing elsewhere herein, and are included in reliance upon such report and upon the authority of such firm as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act of 1933 with respect to the shares of common stock to be sold in this offering. This prospectus, which constitutes part of the registration statement, does not include all of the information contained in the registration statement and the exhibits, schedules and amendments to the registration statement. Some items are omitted in accordance with the rules and regulations of the SEC. For further information with respect to us and our common stock, we refer you to the registration statement and to the exhibits and schedules to the registration statement filed as part of the registration statement. Statements contained in this prospectus about the contents of any contract or any other document filed as an exhibit are not necessarily complete, and, and in each instance, we refer you to the copy of the contract or other documents filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You may read and copy the registration statement of which this prospectus is a part at the SEC s public reference room, which is located at 100 F Street, N.E., Room 1580, Washington, D.C. 20549. You can request copies of the registration statement by writing to the SEC and paying a fee for the copying cost. Please call the SEC at 1-800-SEC-0330 for more information about the operation of the SEC s public reference room. In addition, the SEC maintains an Internet website, which is located at www.sec.gov, that contains reports, proxy and information statements and other information regarding issuers that file electronically with the SEC. You may access the registration statement of which this prospectus is a part at the SEC s Internet website.

Upon the closing of this offering, we will become subject to the full informational and periodic reporting requirements of the Exchange Act. We will fulfill our obligations with respect to such requirements by filing periodic reports and other information with the SEC. We intend to furnish our stockholders with annual reports containing financial statements certified by an independent registered public accounting firm. We also maintain a website at www.agtc.com, at which you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. Our website is not a part of this prospectus.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

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Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders of Applied Genetics Technologies Corporation:

We have audited the accompanying balance sheets of Applied Genetics Technologies Corporation (the Company) as of June 30, 2012 and 2013, and the related statements of operations, convertible preferred stock and stockholders (deficit) equity and cash flows for the years then ended. These financial statements are the responsibility of the Company s management. Our responsibility is to express an opinion on these financial statements based on our audits.

We 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. We were not engaged to perform an audit of the Company s internal control over financial reporting. Our audit included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company s internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Applied Genetics Technologies Corporation as of June 30, 2012 and 2013, and the results of its operations and its cash flows for the years then ended, in conformity with U.S. generally accepted accounting principles.

/s/ McGladrey LLP

Raleigh, North Carolina

November 4, 2013, except for Note 14(b), as to which the date is March 4, 2014.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

BALANCE SHEETS

JUNE 30, 2012 AND 2013

(in thousands, except per share data)

	2012	June 30, 2013	Pro forma 2013 (Note 2) (unaudited)
ASSETS			
Current assets:			
Cash and cash equivalents	$ 774	$ 8,893	$ 19,615
Restricted cash	50		
Short-term investments		14,000	14,000
Grants receivable	184	143	143
Other current assets	87	475	475
Total current assets	1,095	23,511	34,233
Property and equipment, net	53	341	341
Intangible assets, net	1,672	1,630	1,630
Other assets	4	8	8
Total assets	$ 2,824	$ 25,490	$ 36,212
LIABILITIES, CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS (DEFICIT) EQUITY			
Current liabilities:			
Accounts payable	$ 118	$ 792	$ 792
Accrued expenses	369	359	359
Deferred revenue		212	212
Current portion of debt and capital lease	1,007	1	1
Series B purchase rights		2,096	
Total current liabilities	1,494	3,460	1,364
Long-term liabilities:			
Debt and capital lease, net of current portion	16		
Warrant liabilities	80	110	
Total liabilities	1,590	3,570	1,364
Commitments and contingencies (Note 8)			
Series A-1 convertible preferred stock, par value $0.001 per share, 29,737 shares authorized, 22,466 shares issued and outstanding at June 30, 2012 and 2013, and no shares issued and outstanding pro forma (unaudited) (aggregate liquidation preference of $21,698)	21,526	21,526	
Series A-1A convertible preferred stock, par value $0.001 per share, 11,572 shares authorized, 11,479 shares issued and outstanding at June 30, 2012 and 2013, and no shares issued and outstanding pro forma (unaudited) (aggregate liquidation preference of $11,086)	10,998	10,998	
Series B-1 convertible preferred stock, par value $0.001 per share, 67,570 shares authorized, no shares and 66,147 shares issued and outstanding at June 30, 2012 and 2013, respectively, and no shares issued and outstanding pro forma (unaudited) (aggregate liquidation preference of $8,579)		6,539	
Series B-2 convertible preferred stock, par value $0.001 per share, 140,542 shares authorized, no shares and 122,750 shares issued: outstanding at June 30, 2012 and 2013, respectively, and no shares issued and outstanding pro forma (unaudited) (aggregate liquidation preference of $18,228)		19,040	

Series B-3 convertible preferred stock, par value $0.001 per share, 82,670 shares authorized, no shares issued and outstanding at June 30, 2012 and 2013 and pro forma (unaudited)			
Stockholders (deficit) equity:			
Common stock, par value $0.001 per share, 45,102 shares and 410,000 shares authorized at June 30, 2012 and 2013, respectively, 109 shares issued and outstanding at June 30, 2012 and 2013, and 9,229 shares issued and outstanding pro forma (unaudited)			9
Additional paid-in capital	12,146	12,243	83,265
Accumulated deficit	(43,436)	(48,426)	(48,426)
Total stockholders (deficit) equity	(31,290)	(36,183)	34,848
Total liabilities, convertible preferred stock and stockholders (deficit) equity	$ 2,824	$ 25,490	$ 36,212

The accompanying notes to financial statements

are an integral part of these statements.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

STATEMENTS OF OPERATIONS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(in thousands, except per share data)

	Fiscal Years Ended June 30,	
	2012	**2013**
Revenue:		
Grant revenue	$ 718	$ 439
Sponsored research revenue	364	503
Total revenue	1,082	942
Operating expenses:		
Research and development	2,354	3,133
General and administrative	787	1,403
Total operating expenses	3,141	4,536
Loss from operations	(2,059)	(3,594)
Other income (expense):		
Interest income		10
Interest expense	(69)	(191)
Fair value adjustments to warrant liabilities	204	(8)
Fair value adjustments to Series B purchase rights		(1,207)
Total other income (expense), net	135	(1,396)
Net loss	$ (1,924)	$ (4,990)
Net loss per share, basic and diluted	$ (17.65)	$ (45.78)
Weighted-average shares outstanding, basic and diluted	109	109
Pro forma net loss per share, basic and diluted (unaudited) (Note 2)		$ (1.20)
Weighted-average pro forma shares outstanding, basic and diluted (unaudited) (Note 2)		4,146

The accompanying notes to financial statements

are an integral part of these statements.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS (DEFICIT) EQUITY

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(in thousands)

	Convertible Preferred Stock										Common Stock		Additional Paid-In	Accumulated	Tota Stockho
	Series A-1		Series A-1A		Series B-1		Series B-2		Series B-3						
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Capital	Deficit	Defic
nce, June 2011	22,466	$ 21,526	11,479	$ 10,998		$		$		$	109	$	$ 12,122	$ (41,512)	$ (29,3
e-based pensation													24		
oss														(1,924)	(1,9
nce, June 2012	22,466	$ 21,526	11,479	$ 10,998		$		$		$	109	$	$ 12,146	$ (43,436)	$ (31,2
ficial version of s payable eferred k													72		
version of s payable					5,970	741									
nce of rred stock Series B hase rights, f issuance					60,177	5,798	122,750	19,040							
e-based pensation													25		
oss														(4,990)	(4,9
nce, June 2013	22,466	$ 21,526	11,479	$ 10,998	66,147	$ 6,539	122,750	$ 19,040		$	109	$	$ 12,243	$ (48,426)	$ (36,1
nce of es B-3 vertible rred stock udited)									58,817	10,722					
version of vertible rred stock ommon k udited)	(22,466)	(21,526)	(11,479)	(10,998)	(66,147)	(6,539)	(122,750)	(19,040)	(58,817)	(10,722)	9,120	9	68,816		68,8
assification eries B hase rights udited)													2,096		2,0
assification arrants to hase stock dditional -in capital udited)													110		1

Forma, **e 30, 2013** **udited)**	$	$	$	$	9,229	$ 9	$ 83,265	$ (48,426)	$ 34,8

The accompanying notes to financial statements

are an integral part of this statement.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

STATEMENTS OF CASH FLOWS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(in thousands)

	Fiscal Year Ended June 30,	
	2012	**2013**
Cash flows from operating activities		
Net loss	$ (1,924)	$ (4,990)
Adjustments to reconcile net loss to net cash used in operating activities:		
Share-based compensation	24	25
Depreciation and amortization	262	285
Non-cash interest expense	23	163
Fair value adjustments to warrant liabilities	(204)	8
Fair value adjustments to Series B purchase rights		1,207
Change in operating assets and liabilities		
Decrease in grant receivable	294	41
Increase in other current assets	(22)	(392)
(Decrease) increase in accounts payable	(24)	674
Increase in deferred revenues		212
Increase (decrease) in accrued expenses	199	(10)
Net cash used in operating activities	(1,372)	(2,777)
Cash flows from investing activities		
Purchase of property and equipment	(8)	(352)
Purchase of and costs related to intangible assets	(100)	(179)
Release of restricted cash		50
Purchase of short-term investments		(14,000)
Net cash used in investing activities	(108)	(14,481)
Cash flows from financing activities		
Proceeds from issuance of preferred stock and Series B purchase rights, net of issuance costs of $306		25,727
Proceeds from issuance of convertible notes with detachable warrants	750	
Proceeds from issuance of bank term note and warrants		507
Payment of bank term notes and capital lease	(323)	(857)
Net cash provided by financing activities	427	25,377
Net (decrease) increase in cash and cash equivalents	(1,053)	8,119
Cash and cash equivalents, beginning of year	1,827	774
Cash and cash equivalents, end of year	$ 774	$ 8,893
Supplemental disclosure of cash flow information		
Cash paid for interest	$ 38	$ 39
Supplemental disclosure of non-cash financing activities		

Capital lease of property and equipment	$	7	$	
Conversion of Series B purchase rights to Series B-2 convertible preferred stock	$		$	834
Conversion of notes payable and accrued interest to Series B-1 convertible preferred stock	$		$	741

The accompanying notes to financial statements

are an integral part of these statements.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO FINANCIAL STATEMENTS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(shares and dollars in thousands, except per share data)

(1) Organization and Operations:

Applied Genetic Technologies Corporation (the Company or AGTC) was incorporated as a Florida corporation on January 19, 1999 and reincorporated as a Delaware corporation on October 24, 2003. The Company is a clinical-stage biotechnology company developing gene therapy products designed to transform the lives of patients with severe inherited orphan diseases in ophthalmology.

The Company has devoted substantially all of its efforts to research and development, including clinical trials. The Company has not completed the development of any products. The Company has generated revenue from collaboration agreements, sponsored research payments and grants, but has not generated product revenue to date and is subject to a number of risks similar to those of other early stage companies, including dependence on key individuals, the difficulties inherent in the development of commercially viable products, the need to obtain additional capital necessary to fund the development of its products, and competition from other companies. As of June 30, 2013, the Company had an accumulated deficit of $48,426. The Company has financed its operations to date primarily through private placements of its convertible preferred stock, collaborations, bank debt, convertible debt financings, grant funding and payments for sponsored research. The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, development by the Company or its competitors of technological innovations, risks of failure of clinical studies, dependence on key personnel, protection of proprietary technology, compliance with government regulations and ability to transition to large-scale production of products. The Company expects to continue to incur losses for the foreseeable future. At June 30, 2013, the Company had capital resources consisting of cash, cash equivalents and short-term investments of $22,893 and believes that these resources will be sufficient to allow the Company to fund its current operating plan for at least the next 12 months.

(2) Summary of Significant Accounting Policies:

(a) **Basis of Presentation** The accompanying financial statements have been prepared in conformity with accounting principles generally accepted in the United States (GAAP).

(b) **Segment Reporting** The Company operates in only one segment. The chief operating decision-maker and management use cash flows as the primary measure to manage the business and do not segment the business for internal reporting or decision making.

(c) **Unaudited pro forma information** The unaudited pro forma balance sheet as of June 30, 2013, gives effect to: the issuance of 58,817 shares of the Company s Series B-3 preferred, which the Company expects to occur on November 5, 2013 (note 14), for cash proceeds of $10,722; the conversion of all the convertible preferred stock, including the Series B-3, into shares of common stock upon the consummation of this proposed offering; the reclassification of the Series B purchase rights liability to additional paid-in capital; and the conversion of all outstanding warrants exercisable for shares of Series A-1, Series A-1A and Series B-1 preferred stock into warrants exercisable for shares of common stock, resulting in the preferred stock warrant liability being reclassified to additional paid-in capital. Unaudited pro forma net loss per share is computed using the weighted-average number of common stock equivalents outstanding after giving effect to the conversion of all the convertible preferred stock into shares of common stock as if such conversion had occurred at the beginning of the period presented, or the date of original issuance, if later.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO FINANCIAL STATEMENTS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(shares and dollars in thousands, except per share data)

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

(d) **Use of estimates** The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amount of revenues and expenses during the reporting period. Actual results could differ from these estimates.

(e) **Cash and cash equivalents** The Company considers all highly liquid investments with a maturity of 90 days or less at the time of purchase to be cash equivalents. Cash and cash equivalents include cash held in banks and money market accounts. Cash equivalents are carried at cost, which approximates fair value due to their short-term nature.

(f) **Restricted cash** The Company considers any cash legally set aside for a restricted purpose to be restricted cash. The restricted cash is recorded as current unless a related liability is classified as long-term. The balance sheet at June 30, 2012 includes $50 in cash equivalents restricted to secure the Company s credit card with a credit limit of the same amount. The collateral money market account paid interest on a monthly basis. The Company maintained the credit card on an unsecured basis as of June 30, 2013. The balance sheets at both June 30, 2012 and 2013 include $7 in accounts payable for credit card debt. The credit card balance is paid in full on a monthly basis.

(g) **Short-term investments** The Company considers all investments with a maturity of 91 to 360 days at the time of purchase to be short-term investments. Short-term investments include certificates of deposit with maturity within 91 to 360 days of date of purchase. Short-term investments are carried at cost, which approximates fair value due to their short-term nature.

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

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

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

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

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

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO FINANCIAL STATEMENTS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(shares and dollars in thousands, except per share data)

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

Items measured at fair value on a recurring basis include short-term investments, Series B purchase rights and warrant liabilities (Note 6).

(i) **Property and equipment** Property and equipment are recorded at cost. Depreciation is computed using the straight-line method over the estimated useful lives of the assets, which are generally three to seven years. The Company incurs maintenance costs on some of its major lab equipment. The maintenance contracts are prepaid and expensed over the life of the agreement, usually twelve months or less.

(j) **Intangible assets** Intangible assets consist primarily of licenses and patents. The Company obtains licenses from third parties and capitalizes the costs related to exclusive licenses that have alternative future use in multiple potential programs. The Company also capitalizes costs related to filing, issuance, and prosecution of patents. The Company reviews its capitalized costs periodically to determine that costs recorded include costs for patent applications that have future value. The Company evaluates costs related to patents that it is not actively pursuing and writes off any of these costs. Amortization expense is computed using the straight-line method over the estimated useful lives of the assets, which are generally eight to twenty years. The Company amortizes in-licensed patents and patent application from the date of the applicable license and internally developed patents and patent applications from the date of the initial application. Licenses and patents converted to research use only are expensed immediately.

(k) **Impairment of long-lived assets** The Company reviews its long-lived assets for impairment when impairment indicators are present. If impairment indicators exist, management determines whether impairment in value has occurred by comparing the estimated undiscounted cash flows from future operations with the carrying values of the assets. Management considers several indicators in assessing impairment, including trends and prospects, as well as the effects of obsolescence, demand, competition and other economic factors. For the fiscal years ended June 30, 2012 and 2013, the Company did not identify any indicators of impairment for its long-lived assets. The Company has not yet generated positive cash flows, and such cash flows may not materialize for a significant period in the future. As a result, future evaluations of long-lived assets may result in a conclusion that such assets have been impaired.

(l) **Warrants to purchase convertible preferred stock** In conjunction with various financing transactions, the Company issued warrants to purchase shares of the Company s Series A-1, Series A-1A and Series B-1 preferred stock. The Company s Series A-1, Series A-1A and Series B-1 preferred stock are subject to redemption under circumstances outside of the Company s control. Therefore, the associated shares are presented as temporary equity. Consequently, the warrants to purchase shares of Series A-1, Series A-1A and Series B-1 preferred stock are accounted for as liabilities and adjusted to fair value at the end of each reporting period. The fair value of the warrants classified as liabilities is estimated using the Black-Scholes option pricing model. The estimates in Black-Scholes option pricing model are based, in part, on subjective assumptions, including stock price volatility, term of the warrants, risk free interest rate, dividend yield, and fair value of the preferred stock underlying the warrants. Such assumptions could differ materially in the future. The gain or loss associated with the change in the fair value of the preferred stock warrant liability from the prior period is recognized as a component of other (expense) income, net.

(m) **Revenue recognition** The Company has primarily generated revenue through collaboration agreements, sponsored research arrangements with nonprofit organizations for the development and

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO FINANCIAL STATEMENTS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(shares and dollars in thousands, except per share data)

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

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

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

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

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

As required by GAAP, the Company recognizes the financial statement benefit of a tax position only after determining that the relevant tax authority would more likely than not sustain the position following an audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements is the largest benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the relevant tax authority. The Company files income tax returns in the U.S. federal jurisdiction and the state of Florida. As of June 30, 2012 and 2013, the Company does not have any significant uncertain tax positions.

(o) **Research and development** Research and development costs include costs incurred in identifying, developing and testing product candidates. Costs consist primarily of payroll expenses for research related employees, laboratory costs, animal and lab maintenance and supplies, rent, utilities, clinical and pre-clinical expenses, as well as payments for sponsored research, scientific and regulatory consulting fees and testing. Costs are charged to expense as incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and our clinical sites. When outside contracts for research products or testing require advance payments, they are recorded on the balance sheet as a prepaid item and expensed when the service is provided or reaches a specific milestone outlined in the

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO FINANCIAL STATEMENTS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(shares and dollars in thousands, except per share data)

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

contract. Advance payments related to research and development were $63 and $444, at June 30, 2012 and 2013, respectively, and are included in other current assets on the balance sheets.

(p) **Inventory** The Company expenses costs for clinical materials stored for master and working viral banks that remain at the sites in anticipation of their future use at those sites. Since the Company can use each of the raw materials in only a single product, each raw material is deemed to have no future economic value independent of the development status of that single drug.

(q) **Share-based compensation** The Company measures the cost of employee services received in exchange for an award of equity instruments based on the grant date fair value of the award. That cost is recognized on a straight-line basis over the period during which the employee is required to provide service in exchange for the award. The fair value of options on the date of grant is calculated using the Black-Scholes option pricing model based on key assumptions such as stock price, expected volatility and expected term. The Company s estimates of these assumptions are primarily based on third-party valuations, historical data, peer company data and judgment regarding future trends and factors. The Company accounts for stock options issued to non-employees in accordance with the provisions of ASC Subtopic 505-50, *Equity-Based Payments to Non-employees*, which requires valuing the stock options using the Black-Scholes option pricing model and measuring such stock options to their current fair value when they vest.

(r) **Net loss per share and unaudited pro forma net loss per share** Basic net loss per share is calculated by dividing net loss by the weighted average shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by adjusting weighted average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. For purposes of the diluted net loss per share calculation, preferred stock, stock options, and warrants are considered to be common stock equivalents but have been excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive for all periods presented. Therefore, basic and diluted net loss per share was the same for all periods presented. The calculations for the unaudited pro forma basic and diluted net loss per share assume the conversion of all outstanding shares of preferred stock into shares of common stock as if the conversions had occurred at the beginning of the period or the date of issuance, if later.

(3) Property and Equipment, Net:

Property and equipment consists of the following:

	June 30,	
	2012	**2013**
Lab equipment	$ 714	$ 945
Office equipment	88	79
Leasehold improvements	8	8

Software	19	32
Property and equipment, gross	829	1,064
Less: Accumulated depreciation and amortization	(776)	(723)
Property and equipment, net	$ 53	$ 341

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO FINANCIAL STATEMENTS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(shares and dollars in thousands, except per share data)

(3) Property and Equipment, Net: (Continued)

Depreciation and amortization expense was $54 and $64 for the fiscal years ended June 30, 2012 and 2013, respectively. Depreciation and amortization expense of $10 and $14 was included in general and administrative expenses for the years ended June 30, 2012 and 2013, respectively. Depreciation and amortization expense of $44 and $50 was included in research and development expenses for the fiscal years ended June 30, 2012 and 2013, respectively. The Company disposed of fully depreciated assets with a gross value of $117 in the fiscal year ended June 30, 2013.

(4) Intangible Assets, Net:

Intangible assets subject to amortization consist of the following:

	June 30,	
	2012	**2013**
Licenses	$ 991	$ 1,080
Patents	1,700	1,789
Other		2
Intangible assets, gross	2,691	2,871
Less: Accumulated amortization	(1,019)	(1,241)
Intangible assets, net	$ 1,672	$ 1,630

Amortization expense related to intangible assets for the years ended June 30, 2012 and 2013 was $208 and $221, respectively. All amortization expense related to intangible assets is included in research and development expenses on the statements of operations.

Estimated amortization expense for the next five years and thereafter is as follows:

Fiscal Year Ending June 30,	**Amount**
2014	$ 228
2015	222
2016	210
2017	207
2018	198
Thereafter	565
	$ 1,630

(5) Stock Option Plans:

The Company s 2001 Stock Option Plan was adopted effective July 30, 2001. The plan allows for the issuance of options to purchase shares of common stock as incentive and/or nonqualified stock options to certain employees and non-employees. On September 18, 2009, the board resolved to amend the plan to increase the allowed total of options available for issue from 121 to 160.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO FINANCIAL STATEMENTS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(shares and dollars in thousands, except per share data)

(5) Stock Option Plans: (Continued)

In August 2011, the Company approved a Stock Incentive Plan with an effective date of July 30, 2011. The plan allows for the issuance of options to purchase shares of common stock as incentive and/or nonqualified stock options to certain employees and non-employees. On April 6, 2013, the board resolved to amend the plan to increase the total of options available for issue to 846.

(a) Incentive stock options Incentive stock options are granted to employees at the discretion of the board of directors of the Company. The exercise price of the options must at least be equal to 100% of the stock s fair market value on the date of the award.

(b) Nonqualified stock options Nonqualified stock options can be granted to employees or non-employees at the discretion of the board of directors of the Company.

Incentive stock options

Options issued to employees are exercisable at a price ranging from $0.35 to $3.50 per share. Based upon third-party valuations, historical data, peer company data and judgment regarding future trends and factors, management has determined the per share price equals or exceeds fair market value. There is currently no active market for the Company s stock. The employee options generally vest ratably over four years, with 25% vesting one full year after the grant date and 1/48th of each month thereafter, until vested in full. The options expire ten years from the date of the award.

A summary of the employee option activity is as follows:

	Fiscal Year Ended June 30,			
	2012		2013	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Outstanding, beginning of year	69	$ 3.85	69	$ 3.50
Granted	4	3.50	193	0.35
Exercised				
Terminated	(4)	12.53		
Outstanding, end of year	69	$ 3.50	262	$ 1.18
Exercisable, end of year	58		85	
Weighted average fair value of options granted during the year	$ 1.75		$ 0.35	

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO FINANCIAL STATEMENTS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(shares and dollars in thousands, except per share data)

(5) Stock Option Plans: (Continued)

The following table summarizes information about incentive stock options outstanding:

	June 30,			
	2012		2013	
Exercise Price	Number	Weighted Average Contractual Life Remaining	Number	Weighted Average Contractual Life Remaining
$0.35			193	9.53
$3.50	69	5.49	69	4.49
	69		262	

The following table summarizes information about incentive stock options exercisable:

	June 30,			
	2012		2013	
Exercise Price	Number	Weighted Average Contractual Life Remaining	Number	Weighted Average Contractual Life Remaining
$0.35			20	9.53
$3.50	58	5.10	65	4.38
	58		85	

As of June 30, 2013, options to purchase 65 and 20 shares were exercisable at $3.50 and $0.35 per share, respectively, and options to purchase 467 shares remain available to be granted. As of June 30, 2012 and 2013, there was $16 and $30, respectively, of total unrecognized compensation cost related to non-vested incentive stock options.

Share-based compensation cost related to employee incentive stock options included in expense amounted to $18 and $19 for the years ended June 30, 2012 and 2013, respectively. The expense was allocated as follows:

	Fiscal Year Ended June 30,	
	2012	2013
Research and development	$ 9	$ 11
General and administrative	9	8
	$ 18	$ 19

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO FINANCIAL STATEMENTS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(shares and dollars in thousands, except per share data)

(5) Stock Option Plans: (Continued)

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

	Fiscal Year Ended June 30,	
Assumption	**2012**	**2013**
Dividend yield	0.00%	0.00%
Expected term	6.25 years	6.25 years
Risk-free interest rate	1.39%	1.37% to 1.40%
Expected volatility	65.02%	63.23%

The dividend yield is based upon the assumption that the Company will not declare a dividend over the life of the options. Since adopting ASC 718, the Company has been unable to use historical employee exercise and option expiration data to estimate the expected term assumption for the Black-Scholes grant-date valuation. The Company therefore has utilized the simplified method, as prescribed by the SEC s Staff Accounting Bulletin No. 107, *Share-Based Payment*, to estimate on a formula basis the expected term of our stock options considered to have plain vanilla characteristics. The risk-free interest rate is based on the U.S. Treasury yield curve on the date of the grant. The Company computes volatility under the calculated value method of ASC 718 by utilizing the average of a peer group comprised of publicly-traded companies and expect to continue to do so until the Company has adequate historical data regarding the volatility of the Company s traded stock price. The peer group was determined based upon companies considered to be direct competition or having been presented by independent parties as a comparable company based upon market sector. In determining a comparable, the Company has excluded large-cap entities. Forfeitures are estimated at the time of the grant and revised, if necessary, in subsequent periods if actual forfeitures differ from those estimates. Share-based compensation expense recognized in the statement of operations for the years ended June 30, 2012 and 2013 does not record tax related effects on stock-based compensation given the Company s historical and anticipated operating losses and offsetting changes in its valuation allowance that fully reserves against potential deferred tax assets.

The fair value of the shares of common stock that underlie the stock options the Company has granted has historically been determined by the Company s board of directors based upon information available to it at the time of grant. The Company s board of directors considered numerous objective and subjective factors in the assessment of fair value, including reviews of the Company s business and financial condition, the conditions of the industry in which the Company operates and the markets that the Company serves and general economic, market and United States and global capital market conditions, the lack of marketability of its common stock, the likelihood of achieving a liquidity event for the shares of common stock underlying these stock options, the preferences and privileges of the preferred stock over the rights of the common stock, the status of the clinical trials and preclinical studies relating to its product candidates and third-party valuations of its common stock. The Company s board has generally considered the most persuasive evidence of fair value to be the prices at which the Company s securities were sold in actual arms length transactions.

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(5) Stock Option Plans: (Continued)

Nonqualified stock options issued to non-employees

Options to non-employees are exercisable at fixed prices ranging from $0.35 to $3.50 per share. Management has determined the exercise price equals or exceeds fair market value. There is currently no active market for the Company s stock. The non-employee options vest variably over three to four years and expire ten years from the date of the award. A summary of non-employee option activity follows:

	Fiscal Year Ended June 30,			
	2012		2013	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Outstanding, beginning of year	64	$ 3.50	64	$ 3.50
Granted	1	3.50	54	0.35
Terminated	(1)	7.67		
Outstanding, end of year	64	$ 3.50	118	$ 2.06
Exercisable, end of year	59		67	
Weighted average fair value of options granted during the year	$ 1.75		$ 0.35	

In accounting for stock options to non-employees, the value of goods and services related to the options granted are recognized as the awards vest, which is generally consistent with receipt of services. Therefore, vested portions vary based upon services and terms of each option. The Company revalues non-vested, non-employee options each reporting period using the estimated fair value of the Company s common stock as of the last day of each reporting period. Share-based consulting cost amounted to $6 for the years ended June 30, 2012 and 2013 and was allocated to general and administrative expense.

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(6) Fair Value of Financial Instruments and Investments:

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

Description	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets:				
June 30, 2013				
Short-term investments	$ 14,000	$	$ 14,000	$
Liabilities:				
June 30, 2012				
Warrant liabilities	$ 80	$	$	$ 80
June 30, 2013				
Series B purchase rights	$ 2,096	$	$	$ 2,096
Warrant liabilities	110			110
Total	$ 2,206	$	$	$ 2,206

Short-term investments Short-term investments consist of certificates of deposit placed through an account registry service, with maturities up to one year, for which the fair market value is measured based on level 2 inputs (quoted prices for identical assets in markets that are not active).

Warrant liabilities In connection with various financing transactions that were consummated in periods prior to June 30, 2013, the Company issued warrants for the purchase of up to 384, 94, and 1,422 shares of the Company s Series A-1, Series A-1A and Series B-1 convertible preferred stock, respectively, to certain investors and lenders. Each warrant was immediately exercisable and generally expires approximately 5 or 10 years from the original date of issuance. The warrants to purchase shares of the Company s convertible preferred stock have an exercise price equal to the estimated fair value of the underlying instrument as of the initial date such shares were issued. Each warrant is exercisable on either a physical settlement or net share settlement basis.

There were no exercises, cancellations, or expirations of warrants during the fiscal years ended June 30, 2012 and 2013. All warrants were fully vested and exercisable as of June 30, 2012 and 2013.

The terms and accounting treatment for the warrants outstanding are summarized below:

	June 30,					
	2012			2013		
Warrants to purchase:	Shares	Exercise Price	Expiration	Shares	Exercise Price	Expiration

Series A-1 Convertible Preferred Stock	384	$ 0.9658	October 3, 2013 - July 5, 2017	384	$ 0.9658	October 3, 2013 - July 5, 2017
Series A-1A Convertible Preferred Stock	94	$ 0.9658	October 3, 2013 - July 5, 2017	94	$ 0.9658	October 3, 2013 - July 5, 2017
Series B-1 Convertible Preferred Stock	1,145	$ 0.1297	May 2, 2017	1,422	$ 0.1297	May 2, 2017 - August 31, 2019
	1,623			1,900		

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(6) Fair Value of Financial Instruments and Investments: (Continued)

All warrants have been classified in the accompanying balance sheets as liabilities.

The fair value of the warrants on the date of issuance, and on each financial reporting date for those warrants classified as liabilities, is estimated using the Black-Scholes option pricing model. The significant assumptions used in preparing the option pricing model for valuing the Company s warrants include:

	Fiscal Year Ended June 30,	
Assumption	**2012**	**2013**
Exercise price	$ 0.1297 to $0.9658	$ 0.1297 to $0.9658
Fair value of preferred shares	$0.1297	$0.1485
Expected life (in years)	1.26 to 7.17	0.26 to 7.00
Risk-free interest rate	0.27% to 1.39%	0.07% to 1.69%
Expected volatility	65.02%	63.23%

Series B purchase rights

In November 2012, the Company entered into a Series B-1, B-2 and B-3 Preferred Stock Purchase Agreement (the Series B Purchase Agreement), which authorized the sale of up to 290,782 shares of convertible preferred stock in three separate tranches of Series B-1, Series B-2 and Series B-3 preferred stock, respectively. Simultaneously with the execution of the Series B Purchase Agreement, the Company issued and sold an aggregate of 66,147 shares of Series B-1 preferred stock at a price per share of $0.1297. The Series B Purchase Agreement provided that the holders of the Series B-1 shares (Series B holders) were also entitled to purchase up to an aggregate of 140,542 shares of Series B-2 preferred stock for an aggregate purchase price equal to $18,228 (the second tranche) and up to an aggregate of 82,670 shares of Series B-3 preferred stock for an aggregate purchase price equal to $10,722 (the third tranche). The price per share and number of shares to be issued in exchange for such amount was to be determined separately for each tranche by reference to which, if any, of three milestones specified in the Series B Purchase Agreement had been satisfied by the Company.

The purchase rights were legally separable and exercisable apart from the Series B-1 shares and, because representatives of the Series B holders hold a majority of the seats on the board of directors, the decision to complete the second and third tranche was deemed to be outside the control of the Company. The Company therefore recorded, at the time of entry into the Series B Purchase Agreement, a Series B purchase right liability of $1,723 for the fair value of the Company s obligation to sell the Series B-2 and Series B-3 preferred stock in the second and third tranches. The Series B purchase right liability was valued separately for each series using the Black-Scholes option-pricing method to assign a value to the purchase right relating to that series under each of the possible applicable valuation scenarios, depending on which milestones were met, with each scenario being assigned an estimated probability as of the valuation date. The aggregate of these probability-weighted valuations was assigned as the value of the purchase right for each tranche. The initial fair value of the Series B purchase rights was estimated to be $612 for the second tranche and $1,111 for the third tranche. The total value allocated to the Series B purchase rights reduced the amount allocated to the carrying value of the Series B-1 preferred stock on the Company s balance sheet.

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(6) Fair Value of Financial Instruments and Investments: (Continued)

The significant assumptions used as inputs in the Black-Scholes valuation were as follows:

Assumption	Fiscal Year Ended June 30, 2013
Exercise price	$ 0.1297 to $0.1823
Years to maturity	0.37 to 1.87
Risk-free interest rate	0.04% to 0.25%
Volatility	40.0% to 60.0%

The most significant and judgmental inputs driving the fair value of the Company s Series B purchase rights are the assumptions regarding the fair value of the underlying preferred shares and the volatility factor. With all other inputs constant, an increase or decrease in the assumed fair value of the preferred shares would result in a higher or lower estimate of the fair value of the Series B purchase rights, respectively, although there would not be a direct correlation. Similarly, an increase or decrease in the assumed volatility factor would result in a higher or lower estimate of the fair value of the Series B purchase rights, respectively.

In April 2013, following the satisfaction by the Company of the first milestone, the Series B holders exercised their rights with respect to the second tranche and purchased an aggregate of 122,750 shares of Series B-2 preferred stock at a price per share of $0.1485, for gross cash proceeds of $18,228. During fiscal year 2013, a change in value of the Series B purchase right liability of $1,207 was recorded to other expense, and the $834 balance of the value allocated to the Series B-2 purchase right immediately prior to the closing of the second tranche was recorded as proceeds of the issuance of the Series B-2 preferred stock.

The Company reports the change in fair value during each period as a non-operating gain or loss recorded as a component of other (expenses) income in the statement of operations. The table presented below is a summary of changes in the fair value of the Company s Level 3 valuation for warrant liabilities and Series B purchase rights for the fiscal years ended June 30, 2012 and 2013:

	Warrant liabilities	Series B purchase rights
Beginning balance as of July 1, 2011	$ 205	$
Fair value of warrants issued	79	
Change in fair value of during period	(204)	
Ending balance as of June 30, 2012	80	
Fair value of warrants issued	22	
Fair value of Series B purchase rights issued		1,723
Change in fair value of during period	8	1,207
Series B purchase rights converted to Series B-2 convertible preferred stock		(834)
Ending balance as of June 30, 2013	$ 110	$ 2,096

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(shares and dollars in thousands, except per share data)

(7) **Debt and Capital Lease:**

Debt and capital lease are summarized as follows:

	June 30,	
	2012	2013
Term loans, net of original issue discount	$ 336	$
Convertible notes payable, net of original issue discount	683	
Capital lease	4	1
	1,023	1
Less current portion	(1,007)	(1)
Debt and capital lease, net of current portion	$ 16	$

Term loans In July 2010, the Company entered into a loan and security agreement with Square 1 Bank. Under the terms of this agreement, the Company borrowed $800 in July 2010 in exchange for the issuance of a promissory note. The note carried a fixed interest rate of 7%. Interest-only payments were paid monthly through January 2011, followed by 30 equal installments of principal and interest. In consideration of this agreement, the Company issued warrants to purchase 52 shares of Series A-1 preferred stock with an exercise price of $0.9658 per share. The warrants are exercisable upon issuance and will automatically convert upon expiration, seven years after issuance, if not already exercised. The estimated fair value of the warrants at issuance was $32 which was recorded as a discount to the note payable. This discount was amortized over the original life of the loan using the effective interest rate method. Upon early repayment concurrent with the Series B-2 financing, the loss on extinguishment was not material and was classified as interest expense. The loan was collateralized by all assets of the Company except intellectual property.

In August 2012, the Company entered into an amended loan and security agreement with Square 1 Bank to provide additional financing in the form of a second term loan. Under the terms of this amended agreement, in September 2012, the Company borrowed $507 in exchange for the issuance of a promissory note. The note carried an interest rate of 9% through December 2012 and 7% thereafter. Interest-only payments were paid monthly through December 2012, followed by 24 equal installments of principal and interest. In consideration of this amended agreement, the Company issued warrants to purchase 277 shares of Series B-1 preferred stock with an exercise price of $0.1297 per share. The warrants are exercisable upon issuance and will automatically convert upon expiration, seven years after issuance, if not already exercised. The estimated fair value of the warrants at issuance was $22 which was recorded as a discount to the note payable. This discount was amortized over the original life of the loan using the effective interest rate method. Upon early repayment concurrent with the Series B-2 financing, the loss on extinguishment was not material and was classified as interest expense. The loan was collateralized by all assets of the Company except intellectual property.

Interest expense for both notes for the years ended June 30, 2012 and 2013, including non-cash amortization of the discount, was $46 and $70, respectively. The amended loan and security agreement was terminated in April 2013 upon payment of the note balances.

Capital lease In September 2011, the Company converted an operating lease for its phone system into a capital lease agreement in the amount for $7. The lease is repayable monthly over 24 months, beginning October 2011. During fiscal year 2012, these assets were capitalized as office equipment and are being depreciated accordingly. As of June 30, 2013, the outstanding capital lease balance was $1.

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(7) Debt and Capital Lease: (Continued)

Convertible note In May 2012, the Company entered into a convertible note and warrant purchase agreement with existing investors in exchange for gross cash proceeds of $750. The term notes had an interest rate of 8.0% per annum, with principal and interest payable at the stated date of May 2, 2013 (the Maturity Date) or earlier due to a liquidity event or a financing with gross proceeds to the Company of at least $10,000 (a Trigger Financing). The note holders could extend the maturity date with a rate of 10.0% per annum beyond the stated due date. The warrants issued were for the purchase of Series B-1 preferred stock with an exercise price of $0.1297 per share. The estimated fair value of the warrants at issuance was $79 which was recorded as a discount to the note payable. This discount was amortized over the original life of the loan using the effective interest rate method until these notes converted into Series B preferred stock in November 2012 as described further below.

All unpaid principal and accrued but unpaid interest on these convertible notes would be converted automatically into preferred stock securities (New Preferred) issued by the Company in a Trigger Financing closed on or prior to the due date. The number of shares of New Preferred to be issued upon such conversion would be equal to the quotient obtained by dividing (i) the outstanding principal and accrued but unpaid interest under the note by (ii) the price per share equal to the original price per share of such New Preferred, and the issuance of such shares upon such conversion would be upon the same terms and subject to the conditions applicable to the Trigger Financing.

In the event that (i) the Company had not consummated the Trigger Financing by the Maturity Date, or (ii) prior to the closing of the Trigger Financing, there occurred any transaction or series of related transactions resulting in the (a) acquisition of greater than 50% of the voting equity interests of the Company by means of stock purchase, share exchange or other form of corporate reorganization, (b) acquisition, consolidation, merger or like transaction involving the Company in which the shareholders of the Company immediately prior to such transaction own less than 50% of the voting power of the surviving entity, or (c) sale, lease, license, transfer or other conveyance of all or substantially all of the assets of the Company (a Liquidity Event), the holder of each convertible note could, at its option, convert the note into shares of the Company s Series A-1 Preferred Stock (or, at the holder s election, shares of the Company s Series A-1A Preferred Stock) (the Existing Preferred). The number of shares of Existing Preferred to be issued upon such conversion would be equal to the quotient obtained by dividing (i) the outstanding principal and accrued but unpaid interest under the note by (ii) the price per share obtained by dividing (A) $42,208 by (B) the number of fully diluted shares of the Company as of the date of such conversion.

In conjunction with the Series B-1, B-2 and B-3 Preferred Stock Purchase Agreement entered into in November 2012 (see Note 11), the Company converted the notes payable with a carrying value of $709 and related accrued interest of $32 into 5,970 shares of Series B-1 Preferred Stock. The notes payable included a contingent beneficial conversion feature related to a Trigger Financing. The issuance of the Series B preferred stock met the definition of a Trigger Financing and the contingency was resolved. Accordingly, upon conversion of the notes payable, the Company recognized a beneficial conversion feature charge of $72. This beneficial conversion charge has been included as a component of interest expense in the statement of operations. Interest expense for these notes for the years ended June 30, 2012 and 2013, including non-cash amortization of the discount and the beneficial conversion charge discussed above, was $21 and $121, respectively.

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(7) Debt and Capital Lease: (Continued)

The aggregate future maturities of the Company s capital lease as of June 30, 2013 were as follows:

Fiscal Year Ending June 30,	Amount
2014	$ 1
Less: amount representing interest	
Total future maturities	$ 1

(8) Commitments and Contingencies:

Operating leases The Company leases office equipment, office space, and lab space under operating leases expiring through December 2014. For the years ended June 30, 2012 and 2013, rent expense under these and other operating leases was $82 and $102, respectively. Minimum future lease payments under non-cancelable operating leases as of June 30, 2013 in the aggregate are:

Fiscal Year Ending June 30,	Amount
2014	$ 81
2015	23
Total minimum future lease payments	$ 104

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

The Company is also party to various agreements entered into in the ordinary course of its business, principally relating to licensed technology, that require future payments relating to milestones or royalties on future sales of specified products. At June 30, 2013, the Company had nine license agreements with six different entities, including five with the University of Florida Research Foundation. Several of these entities are stockholders of the Company. The Company is required to pay minimum annual royalty and license maintenance for all licenses until such time when the license is terminated by either expiration of underlying patents or voluntary termination by either party per the agreement. Once a product reaches commercialization, the above-mentioned minimum annual payments will be replaced by annual royalties ranging from 0.5% to 4.0% on net sales. The Company is responsible for all costs related to preparation, filing, issuance, prosecution and maintenance of the underlying patents covered in the license agreements. As of June 30, 2013, the Company held one license where certain milestones have been met that requires additional royalty payments. The Company may terminate its license agreements with zero to ninety days written notice depending upon the terms of each specific agreement. The Company paid annual royalty and license maintenance payments of $41 and $61 for

the fiscal years ended June 30, 2012 and 2013, respectively. All royalty and license maintenance payments are included in research and development expenses on the statement of operations.

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(8) Commitments and Contingencies: (Continued)

Minimum annual royalty and license maintenance payments under these agreements are as follows:

Fiscal Year Ending June 30,	Amount
2014	$ 81
2015	$ 101
2016 and every fiscal year thereafter	$ 141

The Company enters into standard indemnification agreements in the ordinary course of business. Pursuant to the agreements, the Company indemnifies, holds harmless, and agrees to reimburse the indemnified party for losses suffered or incurred by the indemnified party, generally the Company s business partners or customers, in connection with any U.S. patent or any copyright or other intellectual property infringement claim by any third party with respect to the Company s products. The term of these indemnification agreements is generally perpetual. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is unlimited. The Company has never incurred costs to defend lawsuits or settle claims related to these indemnification agreements. From time to time, the Company is involved in various claims and legal actions that arise in the normal course of business. Management believes that the outcome of such legal actions will not have a significant adverse effect on the Company s financial position, results of operations or cash flows.

(9) Concentrations:

The Company has demand deposits and money market funds in a regional bank that are insured by the FDIC up to FDIC limits. In addition, the Company has short-term investments in certificates of deposits at various financial institutions that are 100% FDIC insured.

All of the Company s grant receivables at June 30, 2012 and 2013 are derived or due from government grants. Any future changes in the availability of grants for such research would have a significant impact on the Company s operations.

(10) Income Taxes:

For the fiscal years ended June 30, 2012 and 2013, the Company did not record a current or deferred income tax expense or benefit.

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(10) Income Taxes: (Continued)

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

	June 30,	
	2012	2013
Deferred tax assets:		
Net operating loss carryforwards	$ 10,848	$ 11,724
Research and development credit carryforwards	184	218
Accruals and other	47	40
Gross deferred tax assets	11,079	11,982
Deferred tax asset valuation allowance	(11,019)	(11,928)
Total deferred tax assets	$ 60	$ 54
Deferred tax liabilities:		
Depreciation and amortization	$ (60)	$ (54)
Total deferred tax liabilities	$ (60)	$ (54)
Net deferred tax asset (liability)	$	$

At June 30, 2013, the Company has net operating losses of approximately $46,900 that may be applied against future taxable income and expire in various years from 2022 to 2033. At June 30, 2013, the Company also has research and development tax credits of approximately $873 that may provide future tax benefits and expire from 2027 to 2042.

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on the Company s history of operating losses, the Company has concluded that it is more likely than not that the benefit of its deferred tax assets will not be realized. Therefore, any tax benefits to be realized in future years as a result of the utilization of the Company s net operating loss carry forwards as of June 30, 2013, computed based on statutory federal and state rates, are completely offset by valuation allowances established since realization of the deferred tax benefits are not considered more likely than not. The valuation allowance increased approximately $909 during the fiscal year ended June 30, 2013, due primarily to net operating losses generated during the period.

A reconciliation of income tax expense computed at the statutory federal income tax rate to income taxes as reflected in the financial statements is as follows:

	Fiscal Year Ended June 30,	
	2012	2013
Federal income tax benefit at statutory rate	(34)%	(34)%
State income tax, net of federal benefit	(5)	(5)
Permanent differences	(8)	27
Research and development credit	10	8
Other	9	3
Change in valuation allowance	28	1
Effective income tax rate	0%	0%

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(10) Income Taxes: (Continued)

Under the provisions of the Internal Revenue Code, the Company s net operating loss and tax credit carry forwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. Net operating loss and tax credit carry forwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50 percent, as defined under Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. The Company has completed several financings since its inception which may have resulted in a change in control as defined by Sections 382 and 383 of the Internal Revenue Code, or could result in a change in control in the future.

For fiscal years through June 30, 2013, the Company generated research credits but has not conducted a study to document the qualified activities. This study may result in an adjustment to the Company s research and development credit carry forwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position for these two years. A full valuation allowance has been provided against the Company s research and development credits and, if an adjustment is required, this adjustment would be offset by an adjustment to the deferred tax asset established for the research and development credit carry forwards and the valuation allowance.

The Company files income tax returns in the United States and in the state of Florida. The federal and state returns are generally subject to tax examinations for the tax years ended June 30, 2009 through June 30, 2013. To the extent the Company has tax attribute carry forwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service, or state authorities, to the extent utilized in a future period.

The Company s policy is to recognize interest and penalties related to uncertain tax positions in income tax expense. As of June 30, 2012 and 2013, the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Company s statements of operations.

(11) Convertible Preferred Stock and Stockholders (Deficit) Equity:

Common Stock As of June 30, 2012, the Company s common stock consisted of 45,102 authorized shares. In November 2012, the Company amended and restated its Certificate of Incorporation to increase the number of shares authorized to be issued to 410,000 shares of $0.001 par value common stock. At June 30, 2012 and 2013, the Company had 109 shares issued and outstanding.

The following shares of common stock are reserved for future issuance:

	June 30, 2013
Conversion of preferred stock and preferred stock warrants	7,509
Stock options issued and outstanding	380
Authorized for future grant under the 2011 Stock Incentive Plan	467
	8,356

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(11) Convertible Preferred Stock and Stockholders (Deficit) Equity: (Continued)

Convertible preferred stock as of June 30, 2012 and 2013 consists of the following:

	Shares Authorized	Original Issue Price per Share	Shares Issued and Outstanding	Aggregate Liquidation Amount	Carrying Value
Series A-1	29,737	$ 0.9658	22,466	$ 21,698	$ 21,526
Series A-1A	11,572	$ 0.9658	11,479	11,086	10,998
Balance at June 30, 2012	41,309		33,945	$ 32,784	$ 32,524
Series A-1	29,737	$ 0.9658	22,466	$ 21,698	$ 21,526
Series A-1A	11,572	$ 0.9658	11,479	11,086	10,998
Series B-1	67,570	$ 0.1297	66,147	8,579	6,539
Series B-2	140,542	$ 0.1485	122,750	18,228	19,040
Series B-3	82,670				
Balance at June 30, 2013	332,091		222,842	$ 59,591	$ 58,103

Rights and privileges of preferred stock In November 2012, in connection with the transactions contemplated by the Series B Purchase Agreement, the Company amended and restated its certificate of incorporation. The material rights and privileges of the Company s preferred stock as set forth in the Company s amended and restated certificate of incorporation are as follows:

Authorized Shares. The Company is authorized to issue 332,091 shares of preferred stock, par value $0.001 per share, of which 29,737 are designated Series A-1 Preferred Stock, 11,572 are designated Series A-1A Preferred Stock, 67,570 are designated Series B-1 Preferred Stock, 140,542 are designated Series B-2 Preferred Stock and 82,670 are designated Series B-3 Preferred Stock. The Series A-1 and Series A-1A Preferred Stock are referred to collectively as the Series A Preferred, the Series B-1, B-2 and B-3 Preferred Stock are referred to collectively as the Series B Preferred and the Series A Preferred and the Series B Preferred are referred to collectively as the Preferred Stock.

Dividends. Holders of shares of all series of Preferred Stock are entitled to receive cash dividends at the rate of eight percent (8%) of the applicable purchase price of each such share. Such dividends are payable only when, as and if declared by the board of directors of the Company and are non-cumulative. Upon the automatic conversion of Preferred Stock to common stock in the event a successful initial public offering, any dividends declared and unpaid on the Preferred Stock shall be paid. To date, no dividends have been declared or paid.

Liquidation Preference. Upon any liquidation, dissolution, or winding up of the Company, whether voluntary or involuntary (a Liquidation Event), before any distribution or payment shall be made to the holders of any Series A Preferred or common stock, the holders of shares of the Series B Preferred shall be entitled to be paid, out of the assets of the Company legally available for distribution (or the consideration received in any Deemed Liquidation Event, as defined in the certificate of incorporation), an amount equal to the purchase price of such Series B Preferred share plus all declared and unpaid dividends on the Series B-1 Preferred, Series B-2 Preferred or Series B-3 Preferred, as the case may be (the Series B Liquidation Preference). If, upon any such Liquidation Event, the assets of the Company (or the consideration received in the Deemed Liquidation Event) shall be insufficient to make payment in full to all holders of Series B Preferred of the Series B Liquidation Preference, then such assets (or consideration) shall be distributed among the holders of Series B Preferred at the time outstanding, ratably in proportion to

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO FINANCIAL STATEMENTS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(shares and dollars in thousands, except per share data)

(11) Convertible Preferred Stock and Stockholders (Deficit) Equity: (Continued)

the amounts to which they would be entitled with respect to such shares of Series B Preferred if sufficient assets were available to make such payment in full.

Upon any Liquidation Event, after the payment in full of the Series B Liquidation Preference to the holders of Series B Preferred, but before any distribution or payment shall be made to the holders of any common stock, the holders of shares of the Series A Preferred shall be entitled to be paid, out of the assets of the Company legally available for distribution (or the consideration received in the Deemed Liquidation Event), for each share of Series A Preferred, an amount equal to the purchase price of such Series A Preferred share plus all declared and unpaid dividends on the Series A-1 Preferred and Series A-1A Preferred, as the case may be (the Series A Liquidation Preference). If, upon any such Liquidation Event, the assets of the Company (or the consideration received in the Deemed Liquidation Event) shall be insufficient to make payment in full to all holders of Series A Preferred of the Series A Liquidation Preference, then such assets (or consideration) shall be distributed among the holders of Series A Preferred at the time outstanding, ratably in proportion to the amounts to which they would be entitled with respect to such shares of Series A Preferred if sufficient assets were available to make such payment in full.

Upon any Liquidation Event, after the payment in full of the Series B Liquidation Preference and the Series A Liquidation Preference, the assets of the Company legally available for distribution in such Liquidation Event (or the consideration received in the Deemed Liquidation Event), if any, shall be distributed ratably to the holders of the common stock and the Preferred Stock on an as-if-converted to common stock basis.

Conversion Rights. Each share of Preferred Stock is convertible, at the option of the holder thereof, at any time and from time to time, and without the payment of additional consideration by the holder thereof, into fully-paid and nonassessable shares of common stock. As of June 30, 2013, the number of shares of common stock to which a holder of shares of Preferred Stock was entitled upon conversion was as follows: Series A Preferred, 0.06 shares; and Series B Preferred, 0.03 shares.

The conversion ratios applicable to the Preferred Stock are subject to adjustment in the event that the Company effects any subdivision or reverse split or declares any dividend or distribution in shares of common stock, in each case in respect of its common stock, or if the common stock issuable upon the conversion of the Preferred Stock is changed into the same or a different number of shares of any class or classes of stock, whether by recapitalization, reclassification, merger, consolidation or otherwise. The conversion ratios applicable to the Preferred Stock are also subject to anti-dilution adjustment in the event of certain dilutive issuances by the Company of its common stock.

Each share of Preferred Stock shall automatically be converted into shares of common stock, based on the then-effective applicable conversion ratio (i) at any time upon the written consent of the holders of at least a majority of the then outstanding shares of Preferred Stock, voting as a single class or (ii) immediately upon the closing of a firmly underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended, covering the offer and sale of common stock for the account of the Company in which (x) the per share price is at least three (3) times the Series B-3 Purchase Price (as adjusted for stock splits, dividends, recapitalizations and the like after the filing date hereof), or, in the event that the closing of the third tranche has not yet occurred and the Series B-3 Purchase Price has not been determined, then the per share price is at least $0.7425 (as adjusted for stock splits, dividends, recapitalizations and the like after the filing date hereof) and (y) the gross cash proceeds to the Company (before underwriting discounts, commissions and fees) are at least $40,000.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO FINANCIAL STATEMENTS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(shares and dollars in thousands, except per share data)

(11) Convertible Preferred Stock and Stockholders (Deficit) Equity: (Continued)

Voting Rights. Each holder of shares of the Preferred Stock is entitled to the number of votes equal to the number of shares of common stock into which such shares of Preferred Stock could be converted immediately after the close of business on the record date for voting. Except as otherwise provided in the certificate of incorporation or as required by law, holders of shares of Preferred Stock vote together with the holders of shares of common stock and not as a separate class.

Subject to supermajority votes for some matters, matters submitted to a vote of the Company s stockholders shall be decided by the affirmative vote of the stockholders having a majority in voting power of the votes cast by the stockholders present or represented and voting on such matter.

As long as at least 10% of the authorized shares of Series B Preferred remain outstanding, the holders of the outstanding shares of Series B Preferred, voting as a separate class, shall be entitled to elect five (5) members of the board of directors and to remove from office such directors and to fill any vacancy caused by the resignation, death or removal of such directors;

As long as at least 10% of the authorized shares of Series A Preferred remain outstanding, the holders of record of the then outstanding shares of Series A Preferred, voting as a separate class on an as-if-converted to common stock basis, shall be entitled to elect three (3) members of the board of directors at each meeting or pursuant to each consent of the Company s stockholders for the election of directors, and to remove from office such directors and to fill any vacancy caused by the resignation, death or removal of such directors; and

The holders of record of the then outstanding shares of common stock and Preferred Stock, voting together as a single class on an as-if-converted to common stock basis, shall be entitled to elect the remaining member of the board of directors, which member shall be the Company s chief executive officer and to remove from office such director and to fill any vacancy caused by the resignation, death or removal of such director.

The vote or written consent of the holders of at least a majority of the then outstanding shares of Preferred Stock, voting together as a single class is required for the Company to, among other things: liquidate or dissolve; amend, alter or repeal any provision of its certificate of incorporation or bylaws; authorize or issue any other security convertible into or exercisable for any equity security having rights, preferences or privileges senior to or on parity with the Series B Preferred, or increase or decrease the authorized number of shares of Preferred Stock; with certain exceptions, redeem or declare any dividend on any shares of capital stock of the Company; incur more than $2,000 of indebtedness; acquire any minority-owned subsidiary or dispose of any capital stock or assets of any subsidiary; increase or decrease the authorized number of members of the Company s board of directors; take any action that would limit, change or alter the rights, preferences or privileges of any series of the Preferred Stock; or make any acquisition of the capital stock or assets of another entity or enter into any strategic alliance, technology or intellectual property licensing arrangement, or other corporate partnership with any entity involving the payment, contribution or assignment by the Company of more than $1,000.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO FINANCIAL STATEMENTS

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(shares and dollars in thousands, except per share data)

(12) Accrued Expenses:

Accrued expenses consist of the following:

	June 30,	
	2012	2013
Research and development-related	$ 151	$ 61
Compensation-related	207	298
Other	11	
	$ 369	$ 359

(13) 401(k) Plan:

The Company has a 401(k) Plan (the Plan) through the Company s staff leasing company. Employees may elect to defer up to 25% of their compensation. The Company matches employee contributions up to 4%. Total matching contributions to the Plan for the years ended June 30, 2012 and 2013 were approximately $34 and $40, respectively.

(14) Subsequent Events:

(a) The Company has completed an evaluation of all subsequent events through November 4, 2013, to ensure appropriate disclosure of events both recognized in the financial statements as of June 30, 2013, and events which occurred subsequently but were not recognized in the financial statements. The Company has concluded that no subsequent event has occurred that requires disclosure except the following:

In October 2013, the Series B holders notified the Company of their election to exercise their rights with respect to the third tranche to purchase an aggregate of 58,817 shares of Series B-3 preferred stock at a price per share equal to $0.1823, for gross proceeds of $10,722. The Company expects to complete the sale of the shares of Series B-3 preferred stock on November 5, 2013.

(b) On March 4, 2014, the Company effected a 1-for-35 reverse stock split of its common stock, whereby each share of common stock, $0.001 par value, outstanding immediately prior to that date was combined, reclassified and changed into one thirty-fifth (1/35) of a fully paid and non-assessable share of common stock. All common share and common per share information in the accompanying financial statements has been retroactively adjusted to reflect the reverse stock split and adjustment of the preferred stock conversion ratios for all periods presented.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

BALANCE SHEETS (UNAUDITED)

(in thousands, except per share data)

	June 30, 2013	December 31, 2013	Pro forma December 31, 2013
ASSETS			
Current assets:			
Cash and cash equivalents	$ 8,893	$ 8,998	$ 8,998
Short-term investments	14,000	19,000	19,000
Grants receivable	143	366	366
Other current assets	475	1,386	1,386
Total current assets	23,511	29,750	29,750
Property and equipment, net	341	352	352
Intangible assets, net	1,630	1,603	1,603
Other assets	8	4	4
Total assets	$ 25,490	$ 31,709	$ 31,709
LIABILITIES, CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS (DEFICIT) EQUITY			
Current liabilities:			
Accounts payable	$ 792	$ 832	$ 832
Accrued expenses	359	812	812
Deferred revenue	212		
Current portion of debt and capital lease	1		
Series B purchase rights	2,096		
Total current liabilities	3,460	1,644	1,644
Long-term liabilities:			
Warrant liabilities	110	215	
Total liabilities	3,570	1,859	1,644
Commitments and contingencies			
Series A-1 convertible preferred stock, par value $0.001 per share, 29,737 shares authorized at June 30, 2013 and December 31, 2013, 22,466 and 22,467 shares issued and outstanding at June 30, 2013 and December 31, 2013, respectively, and no shares issued and outstanding pro forma (aggregate liquidation preference of $21,699)	21,526	21,527	
Series A-1A convertible preferred stock, par value $0.001 per share, 11,572 shares authorized, 11,479 shares issued and outstanding at June 30, 2013 and December 31, 2013, and no shares issued and outstanding pro forma (aggregate liquidation preference of $11,086)	10,998	10,998	
Series B-1 convertible preferred stock, par value $0.001 per share, 67,570 shares authorized, 66,147 shares issued and outstanding at June 30, 2013 and December 31, 2013, and no shares issued and outstanding pro forma (aggregate liquidation preference of $8,579)	6,539	6,539	
Series B-2 convertible preferred stock, par value $0.001 per share, 140,542 shares authorized, 122,750 shares issued and outstanding at June 30, 2013 and December 31, 2013, and no shares issued and outstanding pro forma (aggregate liquidation preference of $18,228)	19,040	19,040	
Series B-3 convertible preferred stock, par value $0.001 per share, 82,670 shares authorized, no shares issued and outstanding at June 30, 2013 and pro forma, 58,817 shares issued and outstanding December 31, 2013 (aggregate liquidation preference of $10,722)		15,617	
Stockholders (deficit) equity:			

Common stock, par value $.001 per share, 410,000 shares authorized, 109 shares issued and outstanding at June 30, 2013 and December 31, 2013, and 9,229 shares issued and outstanding pro forma			
Additional paid-in capital	12,243	12,354	86,281
Accumulated deficit	(48,426)	(56,225)	(56,225)
Total stockholders (deficit) equity	(36,183)	(43,871)	30,065
Total liabilities, convertible preferred stock and stockholders (deficit) equity	$ 25,490	$ 31,709	$ 31,709

The accompanying notes to financial statements

are an integral part of these statements.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

STATEMENTS OF OPERATIONS (UNAUDITED)

(in thousands, except per share data)

	Six Months Ended December 31,	
	2012	**2013**
Revenue:		
Grant revenue	$ 293	$ 466
Sponsored research revenue	156	307
Total revenue	449	773
Operating expenses:		
Research and development	1,156	3,673
General and administrative	649	1,971
Total operating expenses	1,805	5,644
Loss from operations	(1,356)	(4,871)
Other income (expense):		
Interest income		15
Interest expense	(152)	
Fair value adjustments to warrant liabilities	5	(105)
Fair value adjustments to Series B purchase rights	(437)	(2,838)
Total other income (expense)	(584)	(2,928)
Net loss	$ (1,940)	$ (7,799)
Net loss per share, basic and diluted	$ (17.80)	$ (71.55)
Weighted -average shares outstanding, basic and diluted	109	109
Pro forma net loss per share, basic and diluted		$ (1.13)
Weighted -average pro forma shares outstanding, basic and diluted		6,885

The accompanying notes to financial statements

are an integral part of these statements.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

STATEMENTS OF CASH FLOWS (UNAUDITED)

(in thousands)

	Six Months Ended December 31,	
	2012	2013
Cash flows from operating activities		
Net loss	$ (1,940)	$ (7,799)
Adjustments to reconcile net loss to net cash used in operating activities:		
Share-based compensation	10	111
Depreciation and amortization	125	161
Non-cash interest expense	140	
Fair value adjustments to warrant liabilities	(5)	105
Fair value adjustments to Series B purchase rights	437	2,838
Change in operating assets and liabilities		
Increase in grant receivable	(12)	(223)
Increase in other current assets	(210)	(911)
Increase in accounts payable	450	40
Decrease in deferred revenues		(212)
(Decrease) increase in accrued expenses	(89)	453
Net cash used in operating activities	(1,094)	(5,437)
Cash flows from investing activities		
Purchase of property and equipment	(205)	(54)
Purchase of and costs related to intangible assets	(115)	(87)
Maturity of short-term investments		15,500
Purchase of short-term investments		(20,500)
Net cash used in investing activities	(320)	(5,141)
Cash flows from financing activities		
Proceeds from exercise of convertible preferred stock warrants		1
Proceeds from issuance of preferred stock and Series B purchase rights, net of issuance costs	7,524	10,683
Proceeds from issuance of bank term note and warrants	507	
Payment of bank term notes and capital lease	(75)	(1)
Net cash provided by financing activities	7,956	10,683
Net increase in cash and cash equivalents	6,542	105
Cash and cash equivalents, beginning of period	774	8,893
Cash and cash equivalents, end of period	$ 7,316	$ 8,998
Supplemental disclosure of cash flow information		
Cash paid for interest	$ 20	$
Supplemental disclosure of non-cash financing activities		
Conversion of notes payable and accrued interest to Series B-1 convertible preferred stock	$ 741	$
Conversion of Series B purchase rights to Series B-3 convertible preferred stock	$	$ 4,934

The accompanying notes to financial statements

are an integral part of these statements.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO UNAUDITED FINANCIAL STATEMENTS

FOR THE SIX MONTHS ENDED DECEMBER 31, 2012 AND 2013

(shares and dollars in thousands, except per share data)

(1) Organization and Operations:

Applied Genetic Technologies Corporation (the Company or AGTC) was incorporated as a Florida corporation on January 19, 1999 and reincorporated as a Delaware corporation on October 24, 2003. The Company is a clinical-stage biotechnology company developing gene therapy products designed to transform the lives of patients with severe inherited orphan diseases in ophthalmology.

The Company has devoted substantially all of its efforts to research and development, including clinical trials. The Company has not completed the development of any products. The Company has generated revenue from collaboration agreements, sponsored research payments and grants, but has not generated product revenue to date and is subject to a number of risks similar to those of other early stage companies in the biotechnology industry, including dependence on key individuals, the difficulties inherent in the development of commercially viable products, the need to obtain additional capital necessary to fund the development of its products, development by the Company or its competitors of technological innovations, risks of failure of clinical studies, protection of proprietary technology, compliance with government regulations and ability to transition to large-scale production of products. As of December 31, 2013, the Company had an accumulated deficit of $56,225. The Company has financed its operations to date primarily through private placements of its convertible preferred stock, collaborations, bank debt, convertible debt financings, grant funding and payments for sponsored research. The Company expects to continue to incur losses for the foreseeable future. At December 31, 2013, the Company had capital resources consisting of cash, cash equivalents and short-term investments of $27,998 and believes that these resources will be sufficient to allow the Company to fund its current operating plan for at least the next 12 months.

(2) Summary of Significant Accounting Policies:

The Company s significant accounting policies are more fully described in Note 2 of the Notes to the audited financial statements as of June 30, 2012 and 2013 included in the prospectus of which these financial statements are a part.

(a) **Basis of Presentation** The accompanying financial information as of December 31, 2013 and for the six months ended December 31, 2012 and 2013 has been prepared by the Company, without audit, pursuant to the rules and regulations of the Securities and Exchange Commission. Certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. generally accepted accounting principles have been condensed or omitted pursuant to such rules and regulations. The June 30, 2013 balance sheet was derived from the Company s audited financial statements. The financial information as of December 31, 2013 and for the six months ended December 31, 2012 and 2013 should be read in conjunction with the June 30, 2013 audited annual financial statements and notes thereto included elsewhere in the prospectus.

In the opinion of management, the unaudited financial information as of December 31, 2013 and for the six months ended December 31, 2012 and 2013 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of the Company s financial position, results of operations and cash flows. The results of operations for the six months ended December 31, 2013 are not necessarily indicative of the operating results to be expected for the full fiscal year or any future period.

(b) **Pro forma information** The pro forma balance sheet as of December 31, 2013, gives effect to: the conversion of all the convertible preferred stock into shares of common stock upon the consummation of the initial public offering contemplated by the prospectus of which these financial statements are a

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO UNAUDITED FINANCIAL STATEMENTS

FOR THE SIX MONTHS ENDED DECEMBER 31, 2012 AND 2013

(shares and dollars in thousands, except per share data)

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

part; and the conversion of all outstanding warrants exercisable for shares of Series A-1, Series A-1A and Series B-1 preferred stock into warrants exercisable for shares of common stock, resulting in the preferred stock warrant liability being reclassified to additional paid-in capital. Unaudited pro forma net loss per share is computed using the weighted-average number of common stock equivalents outstanding after giving effect to the conversion of all the convertible preferred stock into shares of common stock as if such conversion had occurred at the beginning of the period presented, or the date of original issuance, if later.

(c) **Use of estimates** The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amount of revenues and expenses during the reporting period. Actual results could differ from these estimates.

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

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

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

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

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

Items measured at fair value on a recurring basis include short-term investments, Series B purchase rights and warrant liabilities (Note 4).

(e) **Warrants to purchase convertible preferred stock** In conjunction with various financing transactions, the Company issued warrants to purchase shares of the Company s Series A-1, Series A-1A and Series B-1 preferred stock. The Company s Series A-1,

Series A-1A and Series B-1 preferred stock are subject to redemption under circumstances outside of the Company s control. Therefore, the associated shares are presented as temporary equity. Consequently, the warrants to purchase shares of Series A-1, Series A-1A and Series B-1 preferred stock are accounted for as liabilities and adjusted to fair value at the end of each reporting period. The fair value of the warrants classified as liabilities is

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO UNAUDITED FINANCIAL STATEMENTS

FOR THE SIX MONTHS ENDED DECEMBER 31, 2012 AND 2013

(shares and dollars in thousands, except per share data)

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

estimated using the Black-Scholes option pricing model. The estimates in the Black-Scholes option pricing model are based, in part, on subjective assumptions, including stock price volatility, term of the warrants, risk free interest rate, dividend yield, and fair value of the preferred stock underlying the warrants. Such assumptions could differ materially in the future. The gain or loss associated with the change in the fair value of the preferred stock warrant liability from the prior period is recognized as a component of other (expense) income, net.

(f) **Share-based compensation** The Company measures the cost of employee services received in exchange for an award of equity instruments based on the grant date fair value of the award. That cost is recognized on a straight-line basis over the period during which the employee is required to provide service in exchange for the award. The fair value of options on the date of grant is calculated using the Black-Scholes option pricing model based on key assumptions such as stock price, expected volatility and expected term. The Company s estimates of these assumptions are primarily based on third-party valuations, historical data, peer company data and judgment regarding future trends and factors. The Company accounts for stock options issued to non-employees in accordance with the provisions of ASC Subtopic 505-50, *Equity-Based Payments to Non-employees*, which requires valuing the stock options and measuring such stock options to their current fair value when they vest.

(g) **Deferred issuance costs** The Company capitalizes certain legal, accounting and other third-party fees that are directly associated with in-process probable equity financings as other assets until such financings are consummated. After consummation of an in-process probable equity financing, these costs are recorded in stockholders equity as a reduction of additional paid-in capital generated as a result of the offering. As of December 31, 2013, the Company recorded deferred financing costs of $894 in other current assets in the accompanying balance sheet in contemplation of a probable equity financing. Should the equity financing no longer be considered probable of being consummated, the deferred financing costs would be expensed immediately as a charge to operating expenses in the statement of operations.

(h) **New Accounting Pronouncements** In July 2013, the FASB issued amended guidance on the financial statement presentation of an unrecognized tax benefit when a net operating loss carryforward, similar tax loss, or tax credit carryforward exists. The guidance requires an unrecognized tax benefit, or a portion of an unrecognized tax benefit, to be presented as a reduction of a deferred tax asset when a net operating loss carryforward, similar tax loss, or tax credit carryforward exists, with certain exceptions. This accounting guidance is effective prospectively for the Company beginning in the first quarter of fiscal year 2015, with early adoption permitted. While the Company is currently evaluating the impact, its adoption is not expected to have a material impact on the Company s financial statements.

(3) Stock Option Plans:

On September 18, 2013, the Company s board of directors approved a grant of 372 incentive stock options and 31 nonqualified stock options under the Company s 2011 Stock Incentive Plan. Effective upon the closing of the Company s sale of shares of Series B-3 preferred stock on November 5, 2013, the Company s stockholders approved an amendment to the 2011 Stock Incentive Plan to increase the total number of shares available for issue under the plan to 909.

(a) **Incentive stock options** Incentive stock options are granted to employees at the discretion of the board. The exercise price of the options must at least be equal to 100% of the stock s fair market value on the date of the award.

(b) **Nonqualified stock options** Nonqualified stock options can be granted to employees or non-employees at the discretion of the board.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO UNAUDITED FINANCIAL STATEMENTS

FOR THE SIX MONTHS ENDED DECEMBER 31, 2012 AND 2013

(shares and dollars in thousands, except per share data)

(3) Stock Option Plans: (Continued)

Incentive stock options

A summary of the employee option activity is as follows:

	Six Months Ended December 31,			
	2012		2013	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Outstanding, June 30	69	$ 3.50	262	$ 1.18
Granted			372	4.90
Exercised				
Terminated				
Outstanding, December 31	69	$ 3.50	634	$ 3.36
Exercisable, December 31	61		125	
Weighted average fair value of options granted during the period			$ 4.90	

As of June 30, 2013 and December 31, 2013, there was approximately $30 and $855, respectively of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the Company s stock incentive plans.

Nonqualified stock options issued to non-employees

A summary of non-employee option activity follows:

	Six Months Ended December 31,			
	2012		2013	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Outstanding, June 30	64	$ 3.50	118	$ 2.06
Granted			31	4.90
Exercised				
Terminated				

Outstanding, December 31	64	$ 3.50	149	$ 2.74
Exercisable, December 31	60		77	
Weighted average fair value of options granted during the period			$ 4.90	

In accounting for stock options to non-employees, the value of goods and services related to the options granted is recognized as the awards vest, which is generally consistent with receipt of services. Therefore, vested portions vary based upon services and terms of each option. The Company revalues non-vested, non-employee options each reporting period using the estimated fair value of the Company s common stock as of the last day of each reporting period.

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO UNAUDITED FINANCIAL STATEMENTS

FOR THE SIX MONTHS ENDED DECEMBER 31, 2012 AND 2013

(shares and dollars in thousands, except per share data)

(4) Fair Value of Financial Instruments and Investments:

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

Description	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets:				
June 30, 2013				
Short-term investments	$ 14,000	$	$ 14,000	$
December 31, 2013				
Short-term investments	$ 19,000	$	$ 19,000	$
Liabilities:				
June 30, 2013				
Series B purchase rights	$ 2,096	$	$	$ 2,096
Warrant liabilities	110			110
Total	$ 2,206	$	$	$ 2,206
December 31, 2013				
Series B purchase rights	$	$	$	$
Warrant liabilities	215			215
Total	$ 215	$	$	$ 215

Short-term investments Short-term investments consist of certificates of deposit placed through an account registry service, with maturities up to one year, for which the fair market value is measured based on level 2 inputs (quoted prices for identical assets in markets that are not active).

Warrant liabilities The fair value of the warrants on the date of issuance, and on each financial reporting date for those warrants classified as liabilities, is estimated using the Black-Scholes option pricing model. The significant assumptions used in preparing the option pricing model for valuing the Company s warrants include:

Assumption	Six Months Ended December 31, 2013
Exercise price	$0.1297 to $0.9658
Fair value of preferred shares	$0.23
Expected life (in years)	0.12 to 5.67
Risk-free interest rate	0.01% to 1.75%
Expected volatility	70.00%

Series B purchase rights

In October 2013, the holders of the Series B-1 and B-2 shares (Series B holders) exercised their rights with respect to the optional third tranche and the Company entered into a First Amendment to Series B-1, B-2 and B-3 Preferred Stock Purchase Agreement (the Series B Amendment) to amend the terms under which the Company could sell Series B-3 Shares. In November 2013, the Company issued and sold an aggregate of 58,817 shares of Series B-3 preferred stock at a price per share of $0.1823. The Series B

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APPLIED GENETIC TECHNOLOGIES CORPORATION

NOTES TO UNAUDITED FINANCIAL STATEMENTS

FOR THE SIX MONTHS ENDED DECEMBER 31, 2012 AND 2013

(shares and dollars in thousands, except per share data)

(4) Fair Value of Financial Instruments and Investments: (Continued)

Amendment provides that if the two remaining milestones specified in the Series B Purchase Agreement entered into in November 2012 are not satisfied by the Company by September 2014, Series B holders who still hold their Series B-3 shares will be entitled to receive up to an aggregate of 13,387 additional shares of Series B-3 preferred stock. The automatic conversion of the Company s preferred stock to common stock upon the consummation of the Company s initial public offering will extinguish this right. During the six months ended December 31, 2013, a change in value of the Series B purchase right liability of $2,838 was recorded to other expense, and $4,934 allocated to the Series B-3 purchase right immediately prior to the closing of the third tranche was reallocated to the carrying value of the Series B-3 preferred stock on the Company s balance sheet.

The significant assumptions used as inputs in the Black-Scholes valuation were as follows:

Assumption	Six Months Ended December 31, 2013
Exercise price	$ 0.1485 to $0.1823
Years to maturity	0.00 to 1.00
Risk-free interest rate	0.06% to 0.10%
Volatility	55.00% to 85.00%

The Company reports the change in fair value during each period as a non-operating gain or loss recorded as a component of other (expense) income, net in the statement of operations. The table presented below is a summary of changes in the fair value of the Company s Level 3 valuation for warrant liabilities and Series B purchase rights for the fiscal year ended June 30, 2013 and the six months ended December 31, 2013:

	Warrant liabilities	Series B purchase rights
Beginning balance as of July 1, 2012	$ 80	$
Fair value of warrants issued	22	
Fair value of Series B purchase rights issued		1,723
Change in fair value during the period	8	1,207
Series B purchase rights converted to Series B-2 convertible preferred stock		(834)
Ending balance as of June 30, 2013	110	2,096
Change in fair value during the period	105	2,838
Series B purchase rights converted to Series B-3 convertible preferred stock		(4,934)
Ending balance as of December 31, 2013	$ 215	$

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(shares and dollars in thousands, except per share data)

(5) Accrued Expenses:

Accrued expenses consist of the following:

	Fiscal Year Ended June 30, 2013	Six Months Ended December 31, 2013
Research and development-related	$ 61	$ 537
Compensation-related	298	275
	$ 359	$ 812

(6) Subsequent Events:

The Company has completed an evaluation of all subsequent events through March 4, 2014, to ensure appropriate disclosure of events both recognized in the financial statements as of December 31, 2013, and events which occurred subsequently but were not recognized in the financial statements.

On March 4, 2014, the Company effected a 1-for-35 reverse stock split of its common stock, whereby each share of common stock, $0.001 par value, outstanding immediately prior to that date was combined, reclassified and changed into one thirty-fifth (1/35) of a fully paid and non-assessable share of common stock. All common share and common per share information in the accompanying financial statements has been retroactively adjusted to reflect the reverse stock split and adjustment of the preferred stock conversion ratios for all periods presented.

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Applied Genetic Technologies Corporation

3,571,000 Shares

Common Stock

Prospectus

, 2014

Through and including , 2014 (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This 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.

BMO Capital Markets Wedbush PacGrow Life Sciences

Cantor Fitzgerald & Co. Roth Capital Partners

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PART II

INFORMATION NOT REQUIRED IN PROSPECTUS

Item 13. Other Expenses of Issuance and Distribution.

The following table indicates the expenses to be incurred in connection with this offering described in this Registration Statement, other than underwriting discounts and commissions, all of which will be paid by us. All amounts are estimated except the Securities and Exchange Commission registration fee, the FINRA filing fee and the Nasdaq Global Market listing fee.

	Amount
Securities and Exchange Commission registration fee	$ 9,016
FINRA filing fee	11,000
NASDAQ Global Market listing fee	125,000
Accountants fees and expenses	300,000
Blue Sky fees and expenses	10,000
Legal fees and expenses	1,000,000
Transfer agent s fees and expenses	10,000
Printing and engraving expenses	200,000
Miscellaneous	34,984
Total Expenses	$ 1,650,000

* To be provided by amendment.

Item 14. Indemnification of Directors and Officers.

Section 102 of the Delaware General Corporation Law permits a corporation to eliminate the personal liability of directors of a corporation to the corporation or its stockholders for monetary damages for a breach of fiduciary duty as a director, except where the director breached his duty of loyalty, failed to act in good faith, engaged in intentional misconduct or knowingly violated a law, authorized the payment of a dividend or approved a stock repurchase in violation of Delaware corporate law or obtained an improper personal benefit. Upon the closing of this offering, our certificate of incorporation will provide that none of our directors shall be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duty as director, notwithstanding any provision of law imposing such liability, except to the extent that the Delaware General Corporation Law prohibits the elimination or limitation of liability of directors for breaches of fiduciary duty.

Section 145 of the Delaware General Corporation Law provides that a corporation has the power to indemnify a director, officer, employee or agent of the corporation and certain other persons serving at the request of the corporation in related capacities against expenses (including attorneys fees), judgments, fines and amounts paid in settlements actually and reasonably incurred by the person in connection with an action, suit or proceeding to which he is or is threatened to be made a party by reason of such position, if such person acted in good faith and in a manner he reasonably believed to be in or not opposed to the best interests of the corporation and, in any criminal action or proceeding, had no reasonable cause to believe his conduct was unlawful, except that, in the case of actions brought by or in the right of the corporation, no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, such person is fairly and reasonably entitled to indemnity for such expenses which the Court of Chancery or such other court shall deem proper.

Upon the closing of this offering, our certificate of incorporation will provide that we will indemnify each person who was or is a party or threatened to be made a party to or is involved in any threatened, pending or completed action, suit or proceeding by reason of the fact that he or she is or was a director or officer of AGTC,

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or is or was serving at our request as a director or officer of another corporation, partnership, joint venture, trust or other enterprise to the fullest extent permitted by the Delaware General Corporation Law. Upon the closing of this offering, our certificate of incorporation will provide that expenses must be advanced to these indemnitees under certain circumstances.

The indemnification provisions contained in our certificate of incorporation that will be effective as of the closing date of this offering are not exclusive. In addition, we intend to enter into indemnification agreements with each of our directors. Each indemnification agreement will provide that we will indemnify the director to the fullest extent permitted by law for claims arising in his capacity as a director, provided that he acted in good faith and in a manner that he reasonably believed to be in, or not opposed to, our best interests and, with respect to any criminal proceeding, had no reasonable cause to believe that his conduct was unlawful. In the event that we do not assume the defense of a claim against a director, we are required to advance his expenses in connection with his defense, provided that he undertakes to repay all amounts advanced if it is ultimately determined that he is not entitled to be indemnified by us.

In addition, we maintain standard policies of insurance under which coverage is provided to our directors and officers against losses arising from claims made by reason of breach of duty or other wrongful act, and to us with respect to payments which may be made by us to such directors and officers pursuant to the above indemnification provisions or otherwise as a matter of law. In any underwriting agreement we enter into in connection with the sale of common stock being registered hereby, the underwriters will agree to indemnify, under certain conditions, us, our directors, our officers and persons who control us within the meaning of the Securities Act of 1933, as amended, against certain liabilities.

Item 15. Recent Sales of Unregistered Securities.

Options and restricted stock

At various times since November 1, 2010, we have granted options to purchase 658,925 shares of common stock to our employees, directors, and consultants pursuant to our 2011 Stock Incentive Plan and 2001 Stock Option Plan at exercise prices ranging from $0.35 to $4.50. During this time, we also issued 57,794 shares of our common stock upon the exercise of options issued pursuant to our 2001 Stock Incentive Plan for aggregate consideration of $196,728. The issuance of these options and shares was exempt from registration under Section 4(a)(2) of the Securities Act, as a sale not involving a public offering, and pursuant to Rule 701 of the Securities Act of 1933, as securities issued pursuant to a compensatory benefit plan.

The following table provides information regarding the number of options and shares of common stock issued upon the exercise of options in each calendar year during this period.

Year	Options issued (#)	Weighted average exercise price of issued options ($)	Total shares of stock issued upon exercise of outstanding options (#)	Weighted average exercise price of exercised options ($)
November 1, 2010 October 31, 2011	4,284	$ 3.50	658	$ 3.50
November 1, 2011 October 31, 2012	4,824	$ 3.50		
November 1, 2012 October 31, 2013	650,084	$ 3.17		
November 1, 2013 March 10, 2014			57,156	$ 3.40

Convertible promissory notes and warrants

In May 2012, we issued and sold convertible promissory notes, which we refer to as the May 2012 notes, in an aggregate principal amount of $0.7 million to five of our existing accredited investors. These promissory notes converted into an aggregate of 5,970,277 shares of our Series B-1 preferred stock in November 2012. In connection with the issuance of the May 2012 notes, we issued warrants to purchase either (i) shares of the series of preferred stock issued in our next equity financing, at an exercise price equal to the amount per share paid by

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investors in such next equity financing, or (ii) at any time prior to such next equity financing, shares of our Series A-1 preferred stock, at an exercise price to be determined based on our fully-diluted capitalization at the time of exercise. These warrants are currently exercisable for an aggregate of 1,421,918 shares of our Series B-1 preferred stock at an exercise price of $0.1297 per share.

These issuances of the May 2012 notes and warrants were exempt from registration under Section 4(a)(2) of, and Rule 506 promulgated under, the Securities Act, as sales not involving a public offering.

Preferred stock

In November 2012, pursuant to a Series B-1, B-2 and B-3 Preferred Stock Purchase Agreement, dated November 15, 2012, we issued and sold 66,147,709 shares of our Series B-1 preferred stock to nine of our existing accredited investors for aggregate consideration of $7.8 million in cash plus the conversion of all outstanding principal and accrued interest on the May 2012 notes. In April 2013, as part of the same financing, we issued and sold an additional 122,749,634 shares of our Series B-2 preferred stock to nine of our existing accredited investors for aggregate consideration of $18.2 million.

Pursuant to the Series B Purchase Agreement, the holders of our Series B preferred stockholders were entitled to purchase an aggregate of 58,816,897 shares of our Series B-3 preferred stock for an aggregate of $10.7 million. The Series B holders exercised this right and we completed the purchase and sale of these Series B-3 shares on November 5, 2013.

In September 2013, we issued and sold 1,247 shares of our series A-1 preferred stock upon the exercise of warrants for aggregate consideration of $1,204.

These issuances of preferred stock were exempt from registration under Section 4(a)(2) of, and Rule 506 promulgated under, the Securities Act, as sales not involving a public offering.

Item 16. Exhibits and Financial Statement Schedules.

The exhibits to the registration statement are listed in the Exhibit Index attached hereto and incorporated by reference herein.

Item 17. Undertakings.

Insofar as indemnification for liabilities arising under the Securities Act of 1933 may be permitted to directors, officers and controlling persons of the registrant pursuant to the foregoing provisions, or otherwise, the registrant has been advised that in the opinion of the Securities and Exchange Commission such indemnification is against public policy as expressed in the Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the registrant of expenses incurred or paid by a director, officer or controlling person of the registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Act and will be governed by the final adjudication of such issue.

The undersigned registrant hereby undertakes that:

For purposes of determining any liability under the Securities Act of 1933, the information omitted from the form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in a form of prospectus filed by the registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this registration statement as of the time it was declared effective.

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For the purpose of determining any liability under the Securities Act of 1933, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial *bona fide* offering thereof.

The undersigned registrant hereby undertakes to provide to the underwriter at the closing specified in the underwriting agreements, certificates in such denominations and registered in such names as required by the underwriter to permit prompt delivery to each purchaser.

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SIGNATURES

Pursuant to the requirements of the Securities Act, the registrant has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of Alachua, State of Florida, on the 12th day of March, 2014.

APPLIED GENETIC TECHNOLOGIES CORPORATION

By: /s/ Susan B. Washer
Susan B. Washer
President and Chief Executive Officer

In accordance with the requirements of the Securities Act of 1933, this Registration Statement has been signed by the following persons in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Susan B. Washer **Susan B. Washer**	Chief Executive Officer, President and Director (Principal Executive Officer)	March 12, 2014
/s/ Lawrence E. Bullock **Lawrence E. Bullock**	Chief Financial Officer (Principal Financial and Accounting Officer)	March 12, 2014
* **Scott Koenig**	Director	March 12, 2014
* **Jill Carroll**	Director	March 12, 2014
* **Ed Hurwitz**	Director	March 12, 2014
* **Arnold Oronsky**	Director	March 12, 2014
* **James Rosen**	Director	March 12, 2014
* **Sam Wu**	Director	March 12, 2014

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* The undersigned, by signing her name hereto, does sign and execute this Registration Statement as attorney-in-fact pursuant to the powers of attorney executed by the above-named officers and directors of the registrant, which powers of attorney were included in the signature page to the Registration Statement of Applied Genetic Technologies Corporation on Form S-1 (File No. 333-193309) filed with the Securities and Exchange Commission on January 10, 2014.

/s/ Susan B. Washer
Susan B. Washer

Attorney-in-fact

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EXHIBIT INDEX

Exhibit number	Description
1.1	Form of Underwriting Agreement
3.1***	Fourth Amended and Restated Certificate of Incorporation of the Registrant
3.2***	Certificate of Amendment to Fourth Amended and Restated Certificate of Incorporation of the Registrant, effective April 10, 2013.
3.3***	Restated Certificate of Incorporation of the Registrant, to be effective upon the closing of this offering
3.4***	Bylaws of the Registrant, as amended
3.5***	Amended and Restated Bylaws of the Registrant, to be effective upon the closing of this offering
3.6***	Certificate of Amendment of Fourth Amended and Restated Certificate of Incorporation, filed on March 4, 2014
4.1	Specimen certificate evidencing shares of common stock
5.1	Opinion of Foley Hoag LLP
10.1***	Lease Agreement made as of September 19, 2011, by and between Thomson-Davis Enterprises, LLC and Applied Genetic Technologies Corporation
10.2 ***	Exclusive License Agreement with Sublicensing Terms, effective as of September 25, 2001, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation
10.3 ***	Restated Amendment to License Agreement made and, effective as of January 31, 2005, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation
10.4 ***	First Amendment After Restated Amendment to License Agreement, made and effective as of November 28, 2007, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation
10.5 ***	Standard Exclusive License Agreement with Sublicensing Terms, effective as of October 7, 2003, by and between the University of Florida Research Foundation, Inc., Johns Hopkins University and Applied Genetic Technologies Corporation
10.6 ***	First Amendment to Standard Exclusive License Agreement with Sublicensing Terms, made as of November 2004, by and between the University of Florida Research Foundation, Inc., Johns Hopkins University and Applied Genetic Technologies Corporation
10.7 ***	Second Amendment to Standard Exclusive License Agreement with Sublicensing Terms, made as of February 25, 2009, by and among Applied Genetic Technologies Corporation, the University of Florida Research Foundation, Inc. and Johns Hopkins University
10.8 ***	Non-Exclusive License Agreement with Sublicensing Terms, made as of January 19, 2006, by and between The UAB Research Foundation, Inc. and Applied Genetic Technologies Corporation
10.9 ***	Standard Exclusive License Agreement with Know How, effective as of September 18, 2012, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation
10.10 ***	Standard Non-Exclusive License Agreement, effective as of September 18, 2012, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation

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Exhibit number	Description
10.11 ***	Standard Exclusive License Agreement with Know How, effective as of November 5, 2012, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation
10.12***	Amended and Restated Investor Rights Agreement, dated as of November 15, 2012
10.13***	Applied Genetic Technologies Corporation 2001 Stock Option Plan, as amended**
10.14***	Applied Genetic Technologies Corporation 2011 Stock Incentive Plan, as amended, and forms of Incentive Stock Option Agreement and Nonstatutory Stock Option Agreement thereunder**
10.15***	Applied Genetic Technologies Corporation 2013 Equity And Incentive Plan**
10.16***	Applied Genetic Technologies Corporation 2013 Employee Stock Purchase Plan**
10.17***	Form of Applied Genetic Technologies Corporation Warrant to Purchase Shares of Series A-1 Preferred Stock
10.18***	Form of Applied Genetic Technologies Corporation Warrant to Purchase Shares of Series B-1 Preferred Stock
10.19***	Warrant to Purchase Shares of Series A-1 Preferred Stock of Applied Genetic Technologies Corporation issued to Silicon Valley Bank and effective on September 23, 2005
10.20***	Warrant to Purchase Shares of Series A-1 Preferred Stock of Applied Genetic Technologies Corporation issued to Silicon Valley Bank and effective on June 30, 2006
10.21***	Warrant to Purchase Shares of Series A-1 Preferred Stock of Applied Genetic Technologies Corporation issued to Square 1 Bank on July 6, 2010
10.22***	Warrant to Purchase Shares of Series B-1 Preferred Stock of Applied Genetic Technologies Corporation issued to Square 1 Bank on August 31, 2012
10.23***	Form of Indemnification Agreement for Directors Associated with an Investment Fund
10.24***	Form of Indemnification Agreement for Directors Not Associated with an Investment Fund
10.25 ***	Second Amendment After Restated Amendment to License Agreement, made and effective as of January 10, 2014, by and between the University of Florida Research Foundation, Inc. and Applied Genetic Technologies Corporation.
10.26 ***	Fourth Amendment to Standard Exclusive License Agreement with Sublicensing Terms, made as of December 17, 2013 by and between the University of Florida Research Foundation, Inc., Johns Hopkins University and Applied Genetic Technologies Corporation
23.1	Consent of McGladrey LLP
23.2	Consent of Foley Hoag LLP (included in Exhibit 5.1)
24.1***	Power of Attorney (included on signature page)

** Management contract or compensatory plan or arrangement
*** Previously filed
Confidential treatment requested as to certain portions, which portions have been omitted and filed separately with the Securities and Exchange Commission