NEPHROS INC
Form POS AM
April 08, 2013

As filed with the Securities and Exchange Commission on April 8, 2013

Registration No. 333-169728

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D. C. 20549

POST-EFFECTIVE AMENDMENT NO. 5

TO

FORM S-1

REGISTRATION STATEMENT

UNDER THE SECURITIES ACT OF 1933

NEPHROS, INC.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	**3841**	**13-3971809**
(State or Other Jurisdiction of Incorporation or Organization)	*(Primary Standard Industrial Classification Code Number)*	*(I. R. S. Employer Identification No.)*

41 Grand Avenue

River Edge, New Jersey 07661

(201) 343-5202

(Address, Including Zip Code, and Telephone Number,

Including Area Code, of Registrant's Principal Executive Offices)

John C. Houghton

President and Chief Executive Officer

Nephros, Inc.

41 Grand Avenue

River Edge, New Jersey 07661

(201) 343-5202

(Name, Address, Including Zip Code, and Telephone Number,

Including Area Code, of Agent for Service)

Copies to:

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Approximate date of commencement of proposed sale to the public: As promptly as practicable after this registration statement becomes effective and the satisfaction or waiver of certain other conditions described herein.

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, check the following box. x

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, 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, 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, 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 12b-2 of the Exchange Act.

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

EXPLANATORY NOTE

This Post-Effective Amendment No. 5 to Form S-1 (this "Post-Effective Amendment") is being filed pursuant to Section 10(a)(3) of the Securities Act of 1933, as amended, to update the Form S-1 Registration Statement (Registration No. 333-169728), which was previously declared effective by the Securities and Exchange Commission ("SEC") on January 31, 2011, to include the audited consolidated financial statements and the notes thereto included in the Registrant's Annual Report on Form 10-K for the fiscal year ended December 31, 2012 that have been filed with the SEC since the Post-Effective Amendment No. 3 to Form S-1 was declared effective by the SEC on April 5, 2012, and contains an updated prospectus relating to the offering and sale of the securities that were registered on Form S-1. As of the date of filing of this Post-Effective Amendment, no further offering will be made of the units registered on Form S-1. The rights offering was completed on March 10, 2011. Accordingly, this Post-Effective Amendment concerns only the exercise of the warrants underlying the units.

All applicable registration fees were paid at the time of the original filing of such Registration Statement on October 1, 2010.

The information in this prospectus is not complete and may be changed. We may not sell these securities until the Post-Effective Amendment No. 5 to the Registration Statement filed with the Securities and Exchange Commission is effective. This prospectus is not an offer to sell these securities or the solicitation of an offer to buy these securities in any state in which such offer, solicitation or sale is not permitted.

PRELIMINARY PROSPECTUS SUBJECT TO COMPLETION — DATED APRIL 8, 2013

NEPHROS, INC.

Issuance of up to 2,981,898 Shares of Common Stock upon Exercise of Warrants

We previously sold 4,964,854 units, each unit consisting of one share of our common stock and a warrant to purchase 4,590,171 shares of our common stock (the "Units"). The warrants are exercisable for a five-year term following the issue date of the warrants, which was March 10, 2011, and have an exercise price of $0.40 per share. This prospectus relates to the issuance of shares of common stock pursuant to the exercise of the warrants to purchase an aggregate of 2,981,898 shares of common stock.

All costs associated with this registration statement will be borne by us. Shares of our common stock are quoted on the OTC Bulletin Board under the ticker symbol "NEPH." On February 20, 2013, the closing sales price for our common stock was $1.00 per share. The shares of common stock issued upon the exercise of warrants will also be quoted on the OTC Bulletin Board under the same ticker symbol. Neither the warrants nor the subscription rights will be listed for trading on any stock exchange or market or quoted on the OTC Bulletin Board.

On March 10, 2011, we completed our rights offering and a private placement that together resulted in gross proceeds of approximately $3.2 million. The aggregate net proceeds were approximately $2.3 million, after deducting the estimated aggregate expenses of these transactions which approximated $200,000, the repayment of the $500,000 note, plus $26,650 of accrued interest thereon, issued to Lambda Investors, LLC, the payment of an 8% sourcing/transaction fee of $40,000 in respect of the note and an aggregate of $100,000 for reimbursement of Lambda Investors' legal fees incurred in connection with the loan and the rights offering.

After giving effect to the 1:20 reverse stock split on March 11, 2011, our stockholders subscribed for 4,964,854 units in the rights offering and we accepted all basic subscription rights and oversubscription privileges. The units were sold at a per unit purchase price of $0.40. Gross proceeds to us from the sale of these units in the rights offering was approximately $2.0 million. We issued an aggregate of 4,964,854 shares of our common stock and warrants to purchase an aggregate of approximately 4,590,171 shares of our common stock to stockholders who subscribed.

Simultaneously with the closing of the rights offering, Lambda Investors, LLC purchased in a private placement 3,009,711 units at the same per unit purchase price of $0.40, pursuant to a purchase agreement between us and Lambda Investors. We issued to Lambda Investors an aggregate of 3,009,711 shares of common stock and warrants to purchase an aggregate of 2,782,579 shares of common stock. Of the $3.2 million in gross proceeds from the rights offering and the private placement, we received approximately $1.2 million in gross proceeds from the sale of units to Lambda Investors.

We effected a reverse stock split, in which every 20 shares of our common stock issued and outstanding immediately prior to the effective time, which was 5:00 p.m. on March 11, 2011, were converted into one share of common stock. Fractional shares were not issued and stockholders who otherwise would have been entitled to receive a fractional share as a result of the reverse stock split received an amount in cash equal to $0.04 per pre-split share for such fractional interests. The number of shares of common stock issued and outstanding was reduced from approximately 201,300,000 pre-split to approximately 10,100,000 post-split. The reverse stock split was effected in connection with the rights offering and private placement.

The reverse stock split was approved by our stockholders at the annual meeting held on January 10, 2011. The number of shares of common stock subject to outstanding stock warrants and options, and the exercise prices and conversion ratios of those securities, were automatically proportionately adjusted for the 1-for-20 ratio provided for by the reverse stock split.

All of the share and per share amounts discussed in this Post-Effective Amendment have been adjusted to reflect the effect of this reverse split.

Investing in our common stock involves substantial risks. See " Risk Factors " beginning on page 6 of this prospectus to read about important factors you should consider before purchasing our common stock.

We do not intend to sell any more Units.

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

The date of this prospectus is April , 2013.

NEPHROS, INC.

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ABOUT THIS PROSPECTUS

We refer to Nephros, Inc. and its consolidated subsidiary as “Nephros”, the “Company”, “we”, “our”, and “us”. This prospectu is part of a registration statement that we have filed with the Securities and Exchange Commission, which we refer to as the SEC or the Commission, utilizing a registration process. It is important for you to read and consider all of the information contained in this prospectus and any applicable prospectus before making a decision whether to invest in the common stock. You should also read and consider the information contained in the exhibits filed with our registration statement, of which this prospectus is a part, as described in “Where You Can Find More Information” in this prospectus.

You should rely only on the information contained in this prospectus and any applicable prospectus supplement, including the information incorporated by reference. We have not authorized anyone to provide you with different information. We are not offering to sell or soliciting offers to buy, and will not sell, any securities in any jurisdiction where it is unlawful. You should assume that the information contained in this prospectus or any prospectus supplement, as well as information contained in a document that we have previously filed or in the future will file with the SEC is accurate only as of the date of this prospectus, the applicable prospectus supplement or the document containing that information, as the case may be.

PROSPECTUS SUMMARY

This summary highlights information contained in other parts of this prospectus. Because it is a summary, it does not contain all of the information that is important to you. For a more complete understanding of our business, you should read this summary together with the more detailed information and financial statements for the years ended December 31, 2012 and 2011, and related notes appearing elsewhere in this prospectus. You should read this entire prospectus carefully, including the “Risk Factors” section beginning on page 6 and the “Special Note Regarding Forward-Looking Statements” section beginning on page 17. This prospectus contains important information that you should consider when making your investment decision.

About the Company

Nephros is a commercial stage medical device company that develops and sells high performance liquid purification filters. Our filters, which we call ultrafilters, are primarily used in dialysis centers and healthcare facilities for the production of ultrapure water and bicarbonate. Because our ultrafilters capture contaminants as small as 0.005 microns in size, they eliminate a wide variety of bacteria, viruses, fungi, parasites, and endotoxins harmful to humans.

All of our ultrafilters use proprietary hollow fiber technology. We believe the hollow fiber design allows our ultrafilters to be the only commercially available filters for healthcare applications that optimize the three elements critical to filter performance:

- Filtration – as low as 0.005 microns
- Flow rate – minimal disruption
- Filter life – up to 12 months

By comparison, competitive filters on the market today are typically effective only to the 0.2 micron level and are prone to clog more quickly, thus reducing their useful lives.

We were founded in 1997 by healthcare professionals affiliated with Columbia University Medical Center/New York-Presbyterian Hospital to develop and commercialize an alternative method to hemodialysis (HD). In 2009, we began to extend our filtration technologies to meet the demand for liquid purification in other areas, in particular water purification.

Our Products

Presently, we offer seven types of ultrafilters for sale to customers in four markets:

- *Dialysis Centers – Water/Bicarbonate*: Treatment of both water and bicarbonate for the production of ultrapure dialysate
- *Hospitals and Other Healthcare Facilities*: Removal of infectious agents in drinking and bathing water, particularly in high risk patient areas

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- *Military*: Highly compact, individual water treatment devices used by soldiers to produce safe drinking water in the field

- *Dialysis Centers – Blood*: Clearance of toxins from blood using an alternative method to HD in patients with chronic renal failure

We have designed our ultrafilters as either in-line products, filters that are incorporated into the existing plumbing of healthcare facilities, or point-of-use products, filters that can be easily installed onto a faucet or as a replacement shower head or can be used stand-alone to purify small quantities of water immediately prior to use.

Our Target Markets

Dialysis Centers – Water/Bicarbonate. To perform hemodialysis, all dialysis clinics have dedicated water purification systems to produce pure water and bicarbonate. Water and bicarbonate are essential ingredients for making dialysate, the liquid that removes waste material from the blood. Within the U.S., there are approximately 5,700 clinics with 100,000 dialysis machines providing over 50 million dialysis treatments to 370,000 patients annually.

Medicare is the main payor for dialysis treatment in the U.S. To be eligible for Medicare reimbursement, dialysis centers must meet the minimum standards for water and bicarbonate quality set by the Association for the Advancement of Medical Instrumentation (AAMI), the American National Standards Institute (ANSI) and the International Standards Organization (ISO). We anticipate that the stricter standards approved by these organizations in 2009 will be adopted by Medicare in the near future.

Published studies have shown that the use of ultrapure dialysate can make patients healthier and reduce their dependence on erythropoietin (EPO), an expensive drug used in conjunction with HD. By reducing the level of dialysate contaminants, specifically cytokine-inducing substances that can pass into a patient's blood stream, cytokine levels within a patient stay low, thus reducing systemic inflammation. When inflammation is low, inflammatory morbidities are reduced and a patient's responsiveness to EPO is enhanced, consequently the overall need for the drug is reduced.

We believe that our ultrafilters are attractive to dialysis centers because they exceed currently approved and newly proposed standards for water/bicarbonate purity, assist in achieving those standards and help dialysis centers reduce costs associated with the amount of EPO required to treat a patient. Our in-line filters are easily installed into the fluid circuits supplying water and bicarbonate just prior to entering each dialysis machine.

Hospitals and Other Healthcare Facilities. According to the United States Centers for Disease Control and Prevention (CDC), healthcare acquired infections (HAIs) annually account for 1.7 million infections, 99,000 deaths, and $4.5 - $6.5 billion in extra costs in U.S. hospitals. At the root of many HAIs are waterborne pathogens such as *Legionella* and *Pseudomonas* which can thrive in aging or complex plumbing systems often found in healthcare facilities. According to the CDC, 23% of Legionella infections originate in healthcare facilities and Pseudomonas infections account for 10% of all water-related HAIs. These pathogens are most harmful to patients in intensive care, neonatal, burn, cancer, and transplant units.

The Affordable Care Act (ACA) which was passed in March 2010 puts in place comprehensive health insurance reforms that aim to lower costs and enhance quality of care. With its implementation, healthcare providers have substantial incentives to deliver better care or be forced to absorb the expenses associated with repeat medical procedures or complications like HAIs. The ACA encompasses HAIs and shifts the costs associated with their treatment back onto the healthcare provider. As a consequence, hospitals and other healthcare facilities are proactively implementing strategies to reduce the potential for HAIs.

Our ultrafilters are designed to reduce the risk of HAIs in the hospital/healthcare setting by treating water just prior to use. Our products can be used for reactive infection control. For example, during acute disease outbreaks (such as Legionnaires' disease), our ultrafilters have been used at hospitals and other healthcare facilities to quickly and efficiently assist in the control of such outbreaks. Our ultrafilters are also being used as a preventative measure in healthcare facilities, particularly in areas where high risk patients are being treated. Our point-of-use filters can be easily installed onto the end of faucets or as replacement shower heads.

The plastic casing of our hospital ultrafilters contains BACTiglas™. BACTiglas™ releases silver ions at the surface of the plastic casing such that they are imparted to anything that touches it. Silver ions (through chemical bonding with amino acids) result in the killing of the bacteria that remains on the surface of the plastic. This enables our hospital ultrafilters to be bacteriocidal to any touch contamination or any growth on the surface of the plastic in addition to their water treatment effect.

Military. The military is heavily reliant on the use of bottled water to support its soldiers in the field. Bottled water is not always available, is very costly to move, resource intensive, and prone to constant supply disruptions. Soldiers conducting operations in isolated and rugged terrain must be able to use available local water sources when unable to resupply from bulk drinking water sources or bottled water. Therefore, the soldier needs the capability to purify water from indigenous water sources in the absence of available potable water. Soldiers must have the ability to remove microbiological contaminants in the water to Environmental Protection Agency specified levels; thereby reducing the effects of acute debilitating illnesses to soldiers.

We offer our individual water treatment device (IWTD), which allows a soldier in the field to derive biologically safe water from any fresh water source. Our IWTD is available in both in-line and point-of-use configurations. Our IWTD is one of the few portable filters that have been validated by the military to meet the NSF Protocol P248 standard. It has also been approved by U.S. Army Public Health Command (USAPHC) and U.S. Army Test and Evaluation Command (ATEC) for deployment. To date, we have received purchase orders for approximately 2,000 IWTDs from individual units of the U.S. armed forces and could become more widely used by soldiers in the future.

In January 2013, the U.S. Army issued a request for proposal (RFP) relating to an IWTD, Nephros submitted its response to this RFP on February 25th. The U.S. Army may award several, one or no contracts as a result of this solicitation. The maximum quantity of all contracts combined is not to exceed 450,000 units or $45,000,000 over a 3 year period. The RFP evaluation period may take up to 6 months before an award is made, if at all.

Dialysis Centers – Blood. The current standard of care in the U.S. for patients with chronic renal failure is HD, a process in which toxins are cleared via diffusion. Patients typically receive HD treatment at least 3 times weekly for 3-4 hours per treatment. HD is most effective in removing smaller, easily diffusible toxins. For patients with acute renal failure, the current standard of care in the U.S. is hemofiltration (HF), a process where toxins are cleared via convection. HF offers a much better removal of larger sized toxins when compared to HD. However, HF treatment is performed on a daily basis, and typically takes 12-24 hours.

Hemodiafiltration (HDF) is an alternative dialysis modality that combines the benefits of HD and HF into a single therapy by clearing toxins using both diffusion and convection. Though not widely used in the U.S., HDF is much more prevalent in Europe and is performed in approximately 16% of patients. Clinical experience and literature show the following multiple clinical and patient benefits of HDF:

·Enhanced clearance of middle and large molecular weight toxins
·Improved survival – up to a 35% reduction in mortality risk
·Reduction in the occurrence of dialysis-related amyloidosis
·Reduction in inflammation
·Reduction in medication such as EPO and phosphate binders
·Improved patient quality of life
·Reduction in number of hospitalizations and overall length of stay

However, like HF, HDF can be resource intensive and can require a significant amount of time to deliver one course of treatment.

We have developed a modified approach to HDF which is more patient-friendly, less resource-intensive, and can be used in conjunction with current HD machines. We refer to our approach as an on-line mid-dilution hemodiafiltration (mid-HDF) system and it consists of our OLpūr H2H Module and OLpūr MD220 Hemodiafilter. On April 30, 2012, we announced that we received clearance from the U.S. Food and Drug Administration to market the OLpūr H2H Module and OLpūr MD220 Hemodiafilter for use with a UF controlled hemodialysis machine that provides ultrapure dialysate in accordance with current ANSI/AAMI/ISO standards, for the treatment of patients with chronic renal failure in the United States. Like HD, on-line mid-HDF treatment is given to patients at least 3 times weekly for 3-4 hours per treatment. Our mid-HDF system is the only HDF system of its kind to be cleared by the FDA to date.

We are currently preparing our OLpūr H2H Modules and manufacturing our OLpūr MD220 Hemodiafilters in readiness for market release. We expect to place a mid-HDF system in a U.S. dialysis clinic in Q2. We have not begun to broadly market our mid-HDF system and plan to seek a commercialization partner in the U.S.

Immediate Need for Capital and Recent Loan from Lambda Investors LLC

As of December 31, 2012, we had cash and cash equivalents totaling approximately $47,000 and tangible assets of approximately $1,419,000.

Due to our dwindling cash position, on February 4, 2013, we issued a senior secured note to Lambda Investors LLC in the principal amount of $1,300,000. We expect that the proceeds from the note will allow us to fund our operations only through May 2013. The terms of the Lambda Investors note are discussed in more detail under the heading ‘‘Business— Recent Developments— Recent Loan from Lambda Investors LLC and Rights Offering.’’

As required under the terms of the note, we are conducting a rights offering to raise up to $3,000,000 from our existing stockholders and warrantholders. If we complete the rights offering, we must repay the principal and accrued interest on the note as well as fees and expenses associated with the note with the proceeds from the rights offering. Other conditions to the closing of the rights offering are discussed under the heading ‘‘Business— Recent Developments— Recent Loan from Lambda Investors LLC and Rights Offering.’’

As of the date of this prospectus, Lambda Investors is our largest stockholder and beneficially owns approximately 31% of our outstanding common stock and, on a fully-diluted basis, owns approximately 53% of our outstanding common stock. The warrants held by Lambda Investors have an exercise price of $0.40 per share and certain warrants have full ratchet anti-dilution protection.

The shares beneficially owned by Lambda Investors may be deemed beneficially owned by Wexford Capital LP, which is the managing member of Lambda Investors. Arthur H. Amron, a director of Nephros, is a partner and general counsel of Wexford Capital. Paul Mieyal, a director of Nephros, is a vice president of Wexford Capital.

Corporate Information

We were incorporated under the laws of the State of Delaware in April 1997. Our principal executive offices are located at 41 Grand Avenue, River Edge, New Jersey, 07661, and our telephone number is (201) 343-5202. We also have an office in Dublin, Ireland. For more information about Nephros, please visit our website at *www.nephros.com*.

Where You Can Find More Information

We make available on our website, *www.nephros.com* , our annual reports, quarterly reports, proxy statements and other filings made with the SEC. The registration statement on Form S-1, of which this prospectus is a part, and its exhibits, as well as our other reports filed with the SEC, can be inspected and copied at the SEC's public reference room at 100 F Street, N.E., Washington, D.C. 20549. The public may obtain information about the operation of the public reference room by calling the SEC at 1-800-SEC-0330. In addition, the SEC maintains a web site at *www.sec.gov* which contains our registration statement on Form S-1 and any amendments thereto and other reports, proxy and information statements and information regarding us that we file electronically with the SEC.

The Offering

The following summary describes the principal terms of the rights offering, but is not intended to be complete.

Securities Offered	2,981,898 shares of common stock issuable upon exercise of the warrants issued in connection with the Units sold on March 10, 2011.
Exercise Price and Term of Warrants	The warrants have an exercise price of $0.40 per share and are exercisable at any time prior to March 10, 2016. For a more complete description of the terms of the warrants, see "Description of Warrants."
Use of Proceeds	The proceeds of this offering consist solely of the payment by warrant holders of the exercise price. We plan to use the net proceeds of this offering to further develop our products and for general working capital purposes. For a more complete description of our intended use of proceeds from this offering, see "Use of Proceeds."
Risk Factors	The exercise of the warrants and the acquisition of our common stock involve substantial risks. See "Risk Factors" beginning on page 6 of this prospectus.
State Securities Law Matters	The issuance and exercise of warrants is subject to compliance with state securities laws and regulations. We reserve the right in some states to require stockholders, if they wish to participate, to state and agree upon exercise of their warrants that they are acquiring the shares for investment purposes only, and that they have no present intention to resell or transfer any shares acquired. This offering is not being made and our securities are not being offered in any jurisdiction where the offer is not permitted under applicable local laws. We have the right, in our sole discretion, to not effect registration or qualification of the shares underlying the warrants in any state or other jurisdiction, or take any other action required by any state or other jurisdiction to allow the offer to take place in that state or jurisdiction. If you reside in a state or other jurisdiction in which registration, qualification or other action is necessary with which we choose not to comply, you will not be eligible to participate in the offering.
Listing	The shares of our common stock issuable upon exercise of the warrants will be listed on the OTC Bulletin Board under the ticker symbol "NEPH."

Unless otherwise indicated, the information in this prospectus reflects a 1-for-20 reverse split of our common stock, which was effective on March 11, 2011.

RISK FACTORS

An investment in our securities involves a high degree of risk. You should consider carefully the following information about these risks, together with the other information contained in this prospectus, before you decide whether to buy our securities. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition and results of operations.

Risks Related to Our Company

Our independent registered public accounting firm, in its audit report related to our financial statements for the fiscal year ended December 31, 2012, expressed substantial doubt about our ability to continue as a going concern.

Our independent registered public accounting firm has included an explanatory paragraph in its report on our financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2012 expressing doubt as to our ability to continue as a going concern. The accompanying financial statements have been prepared assuming that we will continue as a going concern. However, there can be no assurance that we will be able to do so. Our recurring losses and difficulty in generating sufficient cash flow to meet our obligations and sustain our operations raise substantial doubt about our ability to continue as a going concern, and our consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty. Based on our current cash flow projections, we will need to raise additional funds through either the licensing or sale of our technologies or the additional public or private offerings of our securities. However, there is no guarantee that we will be able to obtain further financing, or do so on reasonable terms. If we are unable to raise additional funds on a timely basis, or at all, we would be materially adversely affected.

If we do not receive capital from the rights offering or from another source, we may be forced to cease operations.

We are in immediate need of capital. We expect that the $1.3 million in proceeds from the senior secured note issued to Lambda Investors LLC will allow us to fund our operations through May 2013. If we do not successfully complete a rights offering by May 2013, we expect that we will not have sufficient resources to fund our operations and may be required to cease and wind down operations unless we can find another source of financing at such time, which we believe would be difficult and may not be possible on acceptable terms or at all.

Our secured note with Lambda Investors LLC affects our business operations and contains provisions which restrict our ability to execute certain strategic transactions

On February 4, 2013, we issued a senior secured note to Lambda Investors LLC in the principal amount of $1.3 million. We expect that the proceeds from the note will allow us to fund our operations through May 2013. The note bears interest at the rate of 12% per annum and matures on August 4, 2013, at which time all principal and accrued interest will be due. If we do not pay principal and interest under the note when due, the interest rate increases to 16% per annum. The note is secured by a first priority lien on all of our property, including our intellectual property. In the event of a default, our outstanding indebtedness could become immediately due and payable and, if outstanding indebtedness is not immediately satisfied from cash resources, Lambda could realize on the collateral to secure such indebtedness. Currently, we do not have sufficient cash to satisfy the indebtedness.

As long as indebtedness remains outstanding under the senior secured note with Lambda Investors LLC, we will be subject to certain covenants which, among other items, restrict our ability to merge with another company, sell a material amount of our assets, incur any additional indebtedness, repay any existing indebtedness, or declare or pay any dividends in cash, property or securities. These restrictions significantly impact our future alternatives to enter into strategic transactions and limit our ability to obtain additional or other financing because our assets have been pledged as collateral for repayment of our indebtedness. We have agreed to prepay amounts due under the note with the cash proceeds from (a) a rights offering and an offering of a discounted exercise price to public warrantholders, each as further described in the note, (b) any other equity or debt financing, or (c) the issuance or incurrence of any other indebtedness or the sale of any assets outside the ordinary course of business, in each case prior to the maturity date. In addition, the net proceeds of any offering, financing, asset disposition or other external liquidity generating transaction would need to be first applied to our existing indebtedness which, while reducing our level of indebtedness, cannot be assured to be sufficient for our continuing cash requirements and cash needs.

In the event that we default under the senior secured note or we are unable to repay the indebtedness when it becomes due, Lambda could foreclose on all of our property and assets. If this were to occur, our stockholders could lose all or a portion of their investment in the Company.

We have a history of operating losses and a significant accumulated deficit, and we may not achieve or maintain profitability in the future.

We have not been profitable since our inception in 1997. As of December 31, 2012, we had an accumulated deficit of approximately $97,530,000, primarily as a result of historical operating losses. We expect to continue to incur additional losses for the foreseeable future as a result of a high level of operating expenses, significant up-front expenditures, including the cost of clinical trials, production and marketing activities and very limited revenue from the sale of our products. We began sales of our first product in March 2004, and we may never realize sufficient revenues from the sale of our products or be profitable. Each of the following factors, among others, may influence the timing and extent of our profitability, if any:

- the market acceptance of our technologies and products in each of our target markets;
- our ability to effectively and efficiently manufacture, market and distribute our products;
- our ability to sell our products at competitive prices which exceed our per unit costs; and
- our ability to continue to develop products and maintain a competitive advantage in our industry.

We face significant challenges in obtaining market acceptance of our products, which could adversely affect our potential sales and revenues.

Our products are new to the market, and we do not yet have an established market or customer base for our products. Acceptance of our products in the marketplace by both potential users, including chronic renal failure patients, and potential purchasers, including nephrologists, dialysis clinics and other health care providers, is uncertain, and our failure to achieve sufficient market acceptance will significantly limit our ability to generate revenue and be profitable. Market acceptance will require substantial marketing efforts and the expenditure of significant funds by us to inform dialysis patients and nephrologists, dialysis clinics and other health care providers of the benefits of using our products. We may encounter significant clinical and market resistance to our products and our products may never achieve market acceptance. We may not be able to build key relationships with physicians, clinical groups and government agencies, pursue or increase sales opportunities in Europe or elsewhere, or be the first to introduce hemodiafiltration therapy in the United States. Product orders may be cancelled, patients or customers currently using our products may cease to do so and patients or customers expected to begin using our products may not. Factors that may affect our ability to achieve acceptance of our chronic renal failure therapy products in the marketplace include

whether:

- such products will be safe for use;
- such products will be effective;
- such products will be cost-effective;
- we will be able to demonstrate product safety, efficacy and cost-effectiveness;
- there are unexpected side effects, complications or other safety issues associated with such products; and
- government or third party reimbursement for the cost of such products is available at reasonable rates, if at all.

Acceptance of our water filtration products in the marketplace is also uncertain, and our failure to achieve sufficient market acceptance and sell such products at competitive prices will limit our ability to generate revenue and be profitable. Our water filtration products and technologies may not achieve expected reliability, performance and endurance standards. Our water filtration products and technology may not achieve market acceptance, including among hospitals, or may not be deemed suitable for other commercial, military, industrial or retail applications.

Many of the same factors that may affect our ability to achieve acceptance of our chronic renal failure therapy products in the marketplace will also apply to our water filtration products, except for those related to side effects, clinical trials and third party reimbursement.

If we are not able to successfully scale-up production of our products, then our sales and revenues will suffer.

In order to commercialize our products, we need to be able to produce them in a cost-effective way on a large scale to meet commercial demand, while maintaining extremely high standards for quality and reliability. If we fail to successfully commercialize our products, then we will not be profitable.

We expect to rely on a limited number of independent manufacturers to produce our products. Our manufacturers' systems and procedures may not be adequate to support our operations and may not be able to achieve the rapid execution necessary to exploit the market for our products. Our manufacturers could experience manufacturing and control problems as they begin to scale-up our future manufacturing operations, if any, and we may not be able to scale-up manufacturing in a timely manner or at a commercially reasonable cost to enable production in sufficient quantities. If we experience any of these problems with respect to our manufacturers' initial or future scale-ups of manufacturing operations, then we may not be able to have our products manufactured and delivered in a timely manner. Our products are new and evolving, and our manufacturers may encounter unforeseen difficulties in manufacturing them in commercial quantities or at all.

If we cannot develop adequate distribution, customer service and technical support networks, then we may not be able to market and distribute our products effectively and/or customers may decide not to order our products and, in either case, our sales and revenues will suffer.

Our strategy requires us to distribute our products and provide a significant amount of customer service and maintenance and other technical service. To provide these services, we have begun, and will need to continue, to develop a network of distribution and a staff of employees and independent contractors in each of the areas in which we intend to operate. We cannot assure that we will be able to organize and manage this network on a cost-effective basis. If we cannot effectively organize and manage this network, then it may be difficult for us to distribute our products and to provide competitive service and support to our customers, in which case customers may be unable, or decide not, to order our products and our sales and revenues will suffer.

We have limited experience selling our products to healthcare facilities, and we might be unsuccessful in increasing our sales.

Our business strategy depends in part on our ability to sell our products to hospitals and other healthcare facilities that include dialysis clinics. We have limited experience with respect to sales and marketing. If we are unsuccessful at manufacturing, marketing and selling our products, our operations and potential revenues will be materially adversely affected.

We cannot sell our products, including certain modifications thereto, until we obtain the requisite regulatory approvals and clearances in the countries in which we intend to sell our products. If we fail to receive, or experience a significant delay in receiving, such approvals and clearances, then we may not be able to get our products to market and enhance our revenues.

Our business strategy depends in part on our ability to get our products into the market as quickly as possible. We have obtained a Conformité Européene, or CE, mark, which demonstrates compliance with the relevant European Union requirements and is a regulatory prerequisite for selling our products in the European Union and certain other countries that recognize CE marking (collectively, "European Community"), for our OLpur mid dilution hemodiafilter series product and our Dual Stage Ultrafilter ("DSU"). We have not yet obtained the CE mark for any of our other products. Recently, we received clearance from the FDA to market our OLpūr MD220 Hemodiafilter and OLpūr H2H Module for use with a hemodialysis machine that provides ultrapure dialysate in accordance with current ANSI/AAMI/ISO standards, for the treatment of chronic renal failure patients. We have not yet begun to market these products in the U.S.

There is no assurance that any existing products that have not yet been approved, or any new products developed by us in the future, will be approved for marketing. The clearance and/or approval processes can be lengthy and uncertain and each requires substantial commitments of our financial resources and our management's time and effort. We may not be able to obtain further CE marking or regulatory approval for any of our existing or new products in a timely manner or at all. Even if we do obtain regulatory approval, approval may be only for limited uses with specific classes of patients, processes or other devices. Our failure to obtain, or delays in obtaining, the necessary regulatory clearance and/or approvals would prevent us from selling our affected products in the applicable regions. If we cannot sell some of our products in such regions, or if we are delayed in selling while waiting for the necessary clearance and/or approvals, our ability to generate revenues from these products will be limited.

We intend to market our products globally. Requirements pertaining to the sale of our products vary widely from country to country. It may be very expensive and difficult for us to meet the requirements for the sale of our products in many countries. As a result, we may not be able to obtain the required approvals in a timely manner, if at all. If we cannot sell our products in a particular region, then the size of our potential market could be reduced, which would limit our potential sales and revenues.

Clinical studies that may be required for our products are costly and time-consuming, and their outcome is uncertain.

Before obtaining regulatory approvals for the commercial sale of any of our products, other than those for which we have already received marketing approval in the United States and elsewhere, we must demonstrate through clinical studies that our products are safe and effective.

For products other than those for which we have already received marketing approval, if we do not prove in clinical trials that our products are safe and effective, we will not obtain marketing approvals from the applicable regulatory authorities. In particular, one or more of our products may not exhibit the expected medical benefits, may cause harmful side effects, may not be effective in treating dialysis patients or may have other unexpected characteristics that preclude regulatory approval for any or all indications of use or limit commercial use if approved. The length of time necessary to complete clinical trials varies significantly and is difficult to predict. Factors that can cause delay or termination of our clinical trials include:

- slower than expected patient enrollment due to the nature of the protocol, the proximity of subjects to clinical sites, the eligibility criteria for the study, competition with clinical trials for similar devices or other factors;
- lower than expected retention rates of subjects in a clinical trial;
- inadequately trained or insufficient personnel at the study site to assist in overseeing and monitoring clinical trials;
- delays in approvals from a study site's review board, or other required approvals;
- longer treatment time required to demonstrate effectiveness;
- lack of sufficient supplies of the product;
- adverse medical events or side effects in treated subjects; and
- lack of effectiveness of the product being tested.

Even if we obtain positive results from clinical studies for our products, we may not achieve the same success in future studies of such products. Data obtained from clinical studies are susceptible to varying interpretations that could delay, limit or prevent regulatory approval. In addition, we may encounter delays or rejections based upon changes in regulatory policy for device approval during the period of product development and regulatory review of each submitted new device application. Moreover, regulatory approval may entail limitations on the indicated uses of the device. Failure to obtain requisite governmental approvals or failure to obtain approvals of the scope requested will delay or preclude our licensees or marketing partners from marketing our products or limit the commercial use of such products and will have a material adverse effect on our business, financial condition and results of operations.

In addition, some or all of the clinical trials we undertake may not demonstrate sufficient safety and efficacy to obtain the requisite regulatory approvals, which could prevent or delay the creation of marketable products. Our product development costs will increase if we have delays in testing or approvals, if we need to perform more, larger or different clinical trials than planned or if our trials are not successful. Delays in our clinical trials may harm our financial results and the commercial prospects for our products. Additionally, we may be unable to complete our clinical trials if we are unable to obtain additional capital.

We may be required to design and conduct additional clinical trials.

We may be required to design and conduct additional clinical trials to further demonstrate the safety and efficacy of our products, which may result in significant expense and delay. Regulatory agencies may require new or additional clinical trials because of inconclusive results from current or earlier clinical trials, a possible failure to conduct clinical trials in complete adherence to certain regulatory standards, the identification of new clinical trial endpoints, or the need for additional data regarding the safety or efficacy of our products. It is possible that regulatory authorities may not ultimately approve our products for commercial sale in any jurisdiction, even if we believe future clinical results are positive.

We cannot assure you that our medically approved products will be safe and we are required under applicable law to report any product-related deaths or serious injuries or product malfunctions that could result in deaths or serious injuries, and such reports could trigger recalls, class action lawsuits and other events that could cause us to incur expenses and may also limit our ability to generate revenues from such products.

We cannot assure you that our medically approved products will be safe. Under the Food, Drug and Cosmetic Act (FDC Act), we are required to submit medical device reports, or MDRs, to the FDA to report device-related deaths, serious injuries and product malfunctions that could result in death or serious injury if they were to recur. Depending on their significance, MDRs could trigger events that could cause us to incur expenses and may also limit our ability to generate revenues from such products, such as the following:

- information contained in the MDRs could trigger FDA regulatory actions such as inspections, recalls and patient/physician notifications;

- because the reports are publicly available, MDRs could become the basis for private lawsuits, including class actions; and

- if we fail to submit a required MDR to the FDA, the FDA could take enforcement action against us.

If any of these events occur, then we could incur significant expenses and it could become more difficult for us to gain market acceptance of our medically approved products and to generate revenues from sales. Other countries may impose analogous reporting requirements that could cause us to incur expenses and may also limit our ability to generate revenues from sales of our medically approved products.

Product liability associated with the production, marketing and sale of our products, and/or the expense of defending against claims of product liability, could materially deplete our assets and generate negative publicity which could impair our reputation.

The production, marketing and sale of kidney dialysis and water-filtration products have inherent risks of liability in the event of product failure or claim of harm caused by product operation. Furthermore, even meritless claims of product liability may be costly to defend against. Although we have acquired product liability insurance for our products, we may not be able to maintain or obtain this insurance on acceptable terms or at all. Because we may not be able to obtain insurance that provides us with adequate protection against all potential product liability claims, a successful claim in excess of our insurance coverage could materially deplete our assets. Moreover, even if we are able to obtain adequate insurance, any claim against us could generate negative publicity, which could impair our reputation and adversely affect the demand for our products, our ability to generate sales and our profitability.

Some of the agreements that we may enter into with manufacturers of our products and components of our products may require us:

- to obtain product liability insurance; or

- to indemnify manufacturers against liabilities resulting from the sale of our products.

For example, the agreement with our contract manufacturer, or CM, requires that we obtain and maintain certain minimum product liability insurance coverage and that we indemnify our CM against certain liabilities arising out of our products that they manufacture, provided they do not arise out of our CM's breach of the agreement, negligence or willful misconduct. If we are not able to obtain and maintain adequate product liability insurance, then we could be in breach of these agreements, which could materially adversely affect our ability to produce our products and generate

revenues. Even if we are able to obtain and maintain product liability insurance, if a successful claim in excess of our insurance coverage is made, then we may have to indemnify some or all of our manufacturers for their losses, which could materially deplete our assets.

If we violate any provisions of the FDC Act or any other statutes or regulations, then we could be subject to enforcement actions by the FDA or other governmental agencies.

We face a significant compliance burden under the FDC Act and other applicable statutes and regulations which govern the testing, labeling, storage, record keeping, distribution, sale, marketing, advertising and promotion of our medically approved products. If we violate the FDC Act or other regulatory requirements at any time during or after the product development and/or approval process, we could be subject to enforcement actions by the FDA or other agencies, including:

·fines;

·injunctions;

·civil penalties;

·recalls or seizures of products;

·total or partial suspension of the production of our products;

·withdrawal of any existing approvals or pre-market clearances of our products;

·refusal to approve or clear new applications or notices relating to our products;

·recommendations that we not be allowed to enter into government contracts; and

·criminal prosecution.

Any of the above could have a material adverse effect on our business, financial condition and results of operations.

Significant additional governmental regulation could subject us to unanticipated delays which would adversely affect our sales and revenues.

Our business strategy depends in part on our ability to get our products into the market as quickly as possible. Additional laws and regulations, or changes to existing laws and regulations that are applicable to our business may be enacted or promulgated, and the interpretation, application or enforcement of the existing laws and regulations may change. We cannot predict the nature of any future laws, regulations, interpretations, applications or enforcements or the specific effects any of these might have on our business. Any future laws, regulations, interpretations, applications or enforcements could delay or prevent regulatory approval or clearance of our products and our ability to market our products. Moreover, changes that result in our failure to comply with the requirements of applicable laws and regulations could result in the types of enforcement actions by the FDA and/or other agencies as described above, all of which could impair our ability to have manufactured and to sell the affected products.

Protecting our intellectual property in our technology through patents may be costly and ineffective. If we are not able to adequately secure or enforce protection of our intellectual property, then we may not be able to compete effectively and we may not be profitable.

Our future success depends in part on our ability to protect the intellectual property for our technology through patents. We will only be able to protect our products and methods from unauthorized use by third parties to the extent that our products and methods are covered by valid and enforceable patents or are effectively maintained as trade secrets. Our 16 granted U.S. patents will expire at various times from 2018 to 2026, assuming they are properly maintained.

The protection provided by our patents, and patent applications if issued, may not be broad enough to prevent competitors from introducing similar products into the market. Our patents, if challenged or if we attempt to enforce them, may not necessarily be upheld by the courts of any jurisdiction. Numerous publications may have been disclosed by, and numerous patents may have been issued to, our competitors and others relating to methods and devices for dialysis of which we are not aware and additional patents relating to methods and devices for dialysis may be issued to our competitors and others in the future. If any of those publications or patents conflict with our patent

rights, or cover our products, then any or all of our patent applications could be rejected and any or all of our granted patents could be invalidated, either of which could materially adversely affect our competitive position.

Litigation and other proceedings relating to patent matters, whether initiated by us or a third party, can be expensive and time-consuming, regardless of whether the outcome is favorable to us, and may require the diversion of substantial financial, managerial and other resources. An adverse outcome could subject us to significant liabilities to third parties or require us to cease any related development, product sales or commercialization activities. In addition, if patents that contain dominating or conflicting claims have been or are subsequently issued to others and the claims of these patents are ultimately determined to be valid, then we may be required to obtain licenses under patents of others in order to develop, manufacture, use, import and/or sell our products. We may not be able to obtain licenses under any of these patents on terms acceptable to us, if at all. If we do not obtain these licenses, we could encounter delays in, or be prevented entirely from using, importing, developing, manufacturing, offering or selling any products or practicing any methods, or delivering any services requiring such licenses.

If we file patent applications or obtain patents in foreign countries, we will be subject to laws and procedures that differ from those in the United States. Such differences could create additional uncertainty about the level and extent of our patent protection. Moreover, patent protection in foreign countries may be different from patent protection under U.S. laws and may not be as favorable to us. Many non-U.S. jurisdictions, for example, prohibit patent claims covering methods of medical treatment of humans, although this prohibition may not include devices used for such treatment.

If we are not able to secure and enforce protection of our trade secrets through enforcement of our confidentiality and non-competition agreements, then our competitors may gain access to our trade secrets, we may not be able to compete effectively and we may not be profitable. Such protection may be costly and ineffective.

We attempt to protect our trade secrets, including the processes, concepts, ideas and documentation associated with our technologies, through the use of confidentiality agreements and non-competition agreements with our current employees and with other parties to whom we have divulged such trade secrets. If these employees or other parties breach our confidentiality agreements and non-competition agreements, or if these agreements are not sufficient to protect our technology or are found to be unenforceable, then our competitors could acquire and use information that we consider to be our trade secrets and we may not be able to compete effectively. Policing unauthorized use of our trade secrets is difficult and expensive, particularly because of the global nature of our operations. The laws of other countries may not adequately protect our trade secrets.

If we are not able to maintain sufficient quality controls, then the approval or clearance of our products by the European Union, the FDA or other relevant authorities could be withdrawn, delayed or denied and our sales and revenues will suffer.

Approval or clearance of our products could be withdrawn, delayed or denied by the European Union, the FDA and the relevant authorities of other countries if our manufacturing facilities do not comply with their respective manufacturing requirements. The European Union imposes requirements on quality control systems of manufacturers, which are inspected and certified on a periodic basis and may be subject to additional unannounced inspections. Failure by our manufacturers to comply with these requirements could prevent us from marketing our products in the European Community. The FDA also imposes requirements through quality system requirements, or QSR, regulations, which include requirements for good manufacturing practices, or GMP. Failure by our manufacturers to comply with these requirements could prevent us from obtaining FDA approval of our products and from marketing such products in the United States. Although the manufacturing facilities and processes that we use to manufacture our OLpur MDHDF filter series have been inspected and certified by a worldwide testing and certification agency (also referred to as a notified body) that performs conformity assessments to European Union requirements for medical devices, they have not been inspected by the FDA. A “notified body” is a group accredited and monitored by governmental agencies that inspects manufacturing facilities and quality control systems at regular intervals and is authorized to carry out unannounced inspections. We cannot be sure that any of the facilities or processes we use will comply or continue to comply with their respective requirements on a timely basis or at all, which could delay or prevent our obtaining the approvals we need to market our products in the European Community and the United States.

To market our products in the European Community, the United States and other countries, where approved, manufacturers of such products must continue to comply or ensure compliance with the relevant manufacturing requirements. Although we cannot control the manufacturers of our products, we may need to expend time, resources and effort in product manufacturing and quality control to assist with their continued compliance with these requirements. If violations of applicable requirements are noted during periodic inspections of the manufacturing

facilities of our manufacturers, then we may not be able to continue to market the products manufactured in such facilities and our revenues may be materially adversely affected.

We may face significant risks associated with international operations, which could have a material adverse effect on our business, financial condition and results of operations.

We expect to manufacture and to market our products globally. Our international operations are subject to a number of risks, including the following:

- fluctuations in exchange rates of the United States dollar could adversely affect our results of operations;
- we may face difficulties in enforcing and collecting accounts receivable under some countries' legal systems;
- local regulations may restrict our ability to sell our products, have our products manufactured or conduct other operations;
- political instability could disrupt our operations;
- some governments and customers may have longer payment cycles, with resulting adverse effects on our cash flow; and
- some countries could impose additional taxes or restrict the import of our products.

Any one or more of these factors could increase our costs, reduce our revenues, or disrupt our operations, which could have a material adverse effect on our business, financial condition and results of operations.

Risks Related to Our Common Stock and Warrants

There currently is a limited trading market for our Common Stock.

Our Common Stock currently does not meet all of the requirements for initial listing on a registered stock exchange. Our Common Stock is quoted on the OTC Bulletin Board. Trading in our Common Stock on the OTC Bulletin Board has been very limited. As a result, an investor may find it difficult to dispose of or to obtain accurate quotations as to the market value of our Common Stock, and our Common Stock may be less attractive for margin loans, for investment by financial institutions, as consideration in future capital raising transactions or other purposes. There is no guarantee that we will ever become listed on the Nasdaq Capital Market, or any other exchange, or that a liquid trading market for our Common Stock will develop.

Our Common Stock could be further diluted as a result of the issuance of additional shares of Common Stock, warrants or options.

In the past we have issued Common Stock and warrants in order to raise money. We have also issued stock options as compensation for services and incentive compensation for our employees, directors and consultants. We have shares of Common Stock reserved for issuance upon the exercise of certain of these securities and may increase the shares reserved for these purposes in the future. Our issuance of additional Common Stock, convertible securities, options and warrants could affect the rights of our stockholders, could reduce the market price of our Common Stock or could result in adjustments to exercise prices of outstanding warrants (resulting in these securities becoming exercisable for, as the case may be, a greater number of shares of our Common Stock), or could obligate us to issue additional shares of Common Stock.

Market sales of large amounts of our Common Stock, or the potential for those sales even if they do not actually occur, may have the effect of depressing the market price of our Common Stock, the supply of Common Stock available for resale could be increased which could stimulate trading activity and cause the market price of our Common Stock to drop, even if our business is doing well. Furthermore, the issuance of any additional shares of our Common Stock or securities convertible into our Common Stock could be substantially dilutive to holders of our Common Stock if they do not invest in future offerings.

As previously disclosed, we expect to commence a rights offering in March 2013. Holders of our common stock and public warrants that choose not to fully exercise their basic subscription privilege will be diluted as a result of the rights offering if other shareholders and/or warrantholders fully exercise their basic subscription privilege, and such affected holders' voting and other rights will likewise be diluted.

The prices at which shares of the Common Stock trade have been and will likely continue to be volatile.

In the two years ended December 31, 2012, our Common Stock has traded at prices ranging from a high of $3.19 to a low of $0.40 per share, after giving effect to the 1:20 reverse stock split effected on March 11, 2011. Due to the lack of an active trading market for our Common Stock, you should expect the prices at which our Common Stock might trade to continue to be highly volatile. The expected volatile price of our stock will make it difficult to predict the value of your investment, to sell your shares at a profit at any given time, or to plan purchases and sales in advance. A variety of other factors might also affect the market price of our Common Stock. These include, but are not limited to:

- achievement or rejection of regulatory approvals by our competitors or us;
- publicity regarding actual or potential clinical or regulatory results relating to products under development by our competitors or us;
- delays or failures in initiating, completing or analyzing clinical trials or the unsatisfactory design or results of these trials;
- announcements of technological innovations or new commercial products by our competitors or us;
- developments concerning proprietary rights, including patents;
- regulatory developments in the United States and foreign countries;
- economic or other crises and other external factors;
- period-to-period fluctuations in our results of operations;
- threatened or actual litigation;
- changes in financial estimates by securities analysts; and
- sales of our Common Stock.

We are not able to control many of these factors, and we believe that period-to-period comparisons of our financial results will not necessarily be indicative of our future performance.

In addition, the stock market in general, and the market for biotechnology companies in particular, has experienced extreme price and volume fluctuations in recent years that might have been unrelated or disproportionate to the operating performance of individual companies. These broad market and industry factors might seriously harm the market price of our Common Stock, regardless of our operating performance. Securities class action litigation has often been instituted against companies following periods of volatility in the overall market and in the market price of a company's securities. This litigation, if instituted against us, could result in very substantial costs, divert our management's attention and resources and harm our business, operating results and financial condition.

We have never paid dividends and do not intend to pay cash dividends.

We have never paid dividends on our Common Stock and currently do not anticipate paying cash dividends on our Common Stock for the foreseeable future. Consequently, any returns on an investment in our Common Stock in the foreseeable future will have to come from an increase in the value of the stock itself. As noted above, the lack of an active trading market for our Common Stock will make it difficult to value and sell our Common Stock. While our dividend policy will be based on the operating results and capital needs of our business, it is anticipated that all earnings, if any, will be retained to finance our future operations.

Because we are subject to the "penny stock" rules, you may have difficulty in selling our Common Stock.

Our Common Stock is subject to regulations of the SEC relating to the market for penny stocks. Penny stock, as defined by the Penny Stock Reform Act, is any equity security not traded on a national securities exchange that has a market price of less than $5.00 per share. The penny stock regulations generally require that a disclosure schedule explaining the penny stock market and the risks associated therewith be delivered to purchasers of penny stocks and impose various sales practice requirements on broker-dealers who sell penny stocks to persons other than established customers and accredited investors. The broker-dealer must make a suitability determination for each purchaser and receive the purchaser's written agreement prior to the sale. In addition, the broker-dealer must make certain mandated disclosures, including the actual sale or purchase price and actual bid offer quotations, as well as the compensation to be received by the broker-dealer and certain associated persons. The regulations applicable to penny stocks may severely affect the market liquidity for your Common Stock and could limit your ability to sell your securities in the secondary market.

Several provisions of the Delaware General Corporation Law, our fourth amended and restated certificate of incorporation, as amended, and our second amended and restated bylaws could discourage, delay or prevent a

merger or acquisition, which could adversely affect the market price of our Common Stock.

Several provisions of the Delaware General Corporation Law, our fourth amended and restated certificate of incorporation, as amended, and our second amended and restated bylaws could discourage, delay or prevent a merger or acquisition that stockholders may consider favorable, and the market price of our Common Stock could be reduced as a result. These provisions include:

- authorizing our board of directors to issue "blank check" preferred stock without stockholder approval;
- providing for a classified board of directors with staggered, three-year terms;
- prohibiting us from engaging in a "business combination" with an "interested stockholder" for a period of three years after the date of the transaction in which the person became an interested stockholder unless certain provisions are met;
- prohibiting cumulative voting in the election of directors;
- limiting the persons who may call special meetings of stockholders; and
- establishing advance notice requirements for nominations for election to our board of directors or for proposing matters that can be acted on by stockholders at stockholder meetings.

As a smaller reporting company with little or no name recognition and with several risks and uncertainties that could impair our business operations, we are not likely to generate widespread interest in our Common Stock. Without widespread interest in our Common Stock, our Common Stock price may be highly volatile and an investment in our Common Stock could decline in value.

Unlike many companies with publicly traded securities, we have little or no name recognition in the investment community. We are a relatively new company and very few investors are familiar with either our company or our products. We do not have an active trading market in our Common Stock, and one might never develop, or if it does develop, might not continue.

Additionally, the market price of our Common Stock may fluctuate significantly in response to many factors, many of which are beyond our control. Risks and uncertainties, including those described elsewhere in this "Risk Factors" section could impair our business operations or otherwise cause our operating results or prospects to be below expectations of investors and market analysts, which could adversely affect the market price of our Common Stock. As a result, investors in our Common Stock may not be able to resell their shares at or above their purchase price and could lose all of their investment.

Securities class action litigation is often brought against public companies following periods of volatility in the market price of such company's securities. We may become subject to this type of litigation in the future. Litigation of this type could be extremely expensive and divert management's attention and resources from running our company.

If we fail to maintain an effective system of internal controls over financial reporting, we may not be able to accurately report our financial results, which could have a material adverse effect on our business, financial condition and the market value of our securities.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports. If we cannot provide reliable financial reports, our reputation and operating results may be harmed. If management is unable to express a favorable opinion on the effectiveness of our internal controls, we could lose investor confidence in the accuracy and completeness of our financial reports. Any failure to achieve and maintain effective internal controls could have an adverse effect on our business, financial position and results of operations.

Our directors, executive officers and Lambda Investors LLC control a significant portion of our stock and, if they choose to vote together, could have sufficient voting power to control the vote on substantially all corporate matters.

As of December 31, 2012, our directors, executive officers and Lambda Investors LLC, our largest stockholder, beneficially owned approximately 31% of our outstanding Common Stock, representing approximately 55% on a fully-diluted basis. As previously disclosed, we expect to commence a rights offering in March 2013. Holders of our common stock and public warrants that choose not to fully exercise their basic subscription privilege will be diluted as a result of the rights offering if Lambda fully exercises its subscription privilege, and, consequently, such affected holders' voting and other rights will likewise be diluted. If our stockholders and/or warrantholders do not exercise their subscription privilege in full, and Lambda elects to purchase such shares in the rights offering by exercising an oversubscription right, Lambda would increase its ownership percentage and obtain greater voting power.

As a result of this ownership, Lambda Investors has the ability to exert significant influence over our policies and affairs, including the election of directors. Lambda Investors, whether acting alone or acting with other stockholders, could have the power to elect all of our directors and to control the vote on substantially all other corporate matters without the approval of other stockholders. Furthermore, such concentration of voting power could enable Lambda Investors, whether acting alone or acting with other stockholders, to delay or prevent another party from taking control of our company even where such change of control transaction might be desirable to other stockholders. The interests of Lambda Investors in any matter put before the stockholders may differ from those of any other stockholder.

Future sales of our Common Stock could cause the market price of our Common Stock to decline.

The market price of our Common Stock could decline due to sales of a large number of shares in the market, including sales of shares by Lambda Investors or any other large stockholder, or the perception that such sales could occur. These sales could also make it more difficult or impossible for us to sell equity securities in the future at a time and price that we deem appropriate to raise funds through future offerings of Common Stock. Future sales of our Common Stock by stockholders could depress the market price of our Common Stock.

Shares eligible for future sale may adversely affect the market.

From time to time, certain of our stockholders may be eligible to sell all or some of their shares of Common Stock by means of ordinary brokerage transactions in the open market pursuant to Rule 144 promulgated under the Securities Act, subject to certain limitations. In general, pursuant to Rule 144, non-affiliate stockholders may sell freely after holding their shares for six months and affiliates may sell freely after holding their shares for one year, in each case, subject to current public information, notice and other requirements. Any substantial sales of our Common Stock pursuant to Rule 144 may have a material adverse effect on the market price of our Common Stock.

The market price of our common stock may fall below the exercise price of the warrants issued in connection with the rights offering.

The warrants are currently exercisable and will expire on March 10, 2016. The market price of our common stock may fall below the exercise price for these warrants prior to their expiration. Any warrants not exercised by their date of expiration will expire worthless and we will be under no further obligation to the holders of warrants.

If an effective registration is not in place and a current prospectus is not available when an investor desires to exercise warrants, such investor may be unable to exercise his, her or its warrants, causing such warrants to expire worthless.

We will not be obligated to issue shares of common stock upon exercise of warrants unless, at the time such holder seeks to exercise such warrant, we have a registration statement under the Securities Act in effect covering the shares of common stock issuable upon the exercise of the warrants and a current prospectus relating to the common stock. We intend to use our best efforts to keep a registration statement in effect covering shares of common stock issuable upon exercise of the warrants and to maintain a current prospectus relating to the common stock issuable upon exercise of the warrants until the expiration of the warrants. However, we cannot assure you that we will be able to do so, and if we do not maintain a current prospectus related to the common stock issuable upon exercise of the warrants, holders will be unable to exercise their warrants and we will not be required to settle any such warrant exercise. If the prospectus relating to the common stock issuable upon the exercise of the warrants is not current, the warrants held by public stockholders may have no value, we will have no obligation to settle the warrants for cash, the market for such warrants may be limited, such warrants may expire worthless and, as a result, an investor may have paid the full price solely for the shares of common stock included in the Units.

An investor will only be able to exercise a warrant if the issuance of common stock upon such exercise has been registered or qualified or is deemed exempt under the securities laws of the state of residence of the holder of the warrants.

No warrants will be exercisable and we will not be obligated to issue shares of common stock unless the shares of common stock issuable upon such exercise have been registered or qualified or deemed to be exempt under the securities laws of the state of residence of the holder of the warrants. Because the exemptions from qualification in certain states for resales of warrants and for issuances of common stock by the issuer upon exercise of a warrant may be different, a warrant may be held by a holder in a state where an exemption is not available for issuance of common stock upon an exercise and the holder will be precluded from exercise of the warrant. As a result, the warrants may be deprived of any value, the market for the warrants may be limited, the holders of the warrants may not be able to exercise their warrants and they may expire worthless if the common stock issuable upon such exercise is not qualified or exempt from qualification in the jurisdictions in which the holders of the warrants reside.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains certain "forward-looking statements." Such statements include statements regarding the efficacy and intended use of our technologies under development, the timelines for bringing such products to market and the availability of funding sources for continued development of such products and other statements that are not historical facts, including statements which may be preceded by the words "intends," "may," "will," "plans," "expects," "anticipates," "projects," "predicts," "estimates," "aims," "believes," "hopes," "potential" or similar words. Forward-looking st are not guarantees of future performance are based on certain assumptions and are subject to various known and unknown risks and uncertainties, many of which are beyond our control. Actual results may differ materially from the expectations contained in the forward-looking statements. Factors that may cause such differences include, but are not limited to, the risks that:

- we may not be able to continue as a going concern;
- we may not be able to obtain funding if and when needed or on terms favorable to us in order to continue operations;
- a default under the terms of the secured note with Lambda Investors LLC would result in the lender foreclosing upon substantially all of our assets and could result in our inability to continue business operations;
- we may not be able to complete the contemplated rights offering which could result in our inability to continue business operations;
- even if we are able to complete the rights offering, we may not have sufficient capital to successfully implement our business plan;
- restrictions in the secured note and related security agreement which require the prior consent of the lender may restrict our ability to operate our business, sell the company or sell our assets;
- we may not be able to effectively market our products;
- we may not be able to sell our water filtration products or chronic renal failure therapy products at competitive prices or profitably;
- we may encounter problems with our suppliers and manufacturers;
- we may encounter unanticipated internal control deficiencies or weaknesses or ineffective disclosure controls and procedures;
- we may not obtain appropriate or necessary regulatory approvals to achieve our business plan;
- products that appeared promising to us in research or clinical trials may not demonstrate anticipated efficacy, safety or cost savings in subsequent pre-clinical or clinical trials;
- we may not be able to secure or enforce adequate legal protection, including patent protection, for our products; and

- we may not be able to achieve sales growth in key geographic markets.

More detailed information about the Company and the risk factors that may affect the realization of forward-looking statements, including the forward-looking statements in this prospectus and in our Annual Report on Form 10-K for the year ended December 31, 2012, is set forth in our filings with the SEC, including our other periodic reports filed with the SEC. We urge investors and security holders to read those documents free of charge at the SEC's web site at www.sec.gov. We do not undertake to publicly update or revise our forward-looking statements as a result of new information, future events or otherwise, except as required by law.

Any information contained in this prospectus relating to the contemplated rights offering previously disclosed on a Form 8-K filed on February 5, 2013 is preliminary in nature. The securities that are to be offered in the rights offering described therein may not be sold, nor may offers to buy be accepted, prior to the time the registration statement relating to the rights offering becomes effective. This communication shall not constitute an offer to sell or the solicitation of an offer to buy in the rights offering, nor shall there be any sale of the securities in the rights offering, in any state in which such offer, solicitation or sale would be unlawful prior to their registration or qualification under the securities laws of any such state.

USE OF PROCEEDS

We received proceeds from the offer and sale of the Units, net of discounts, commissions and expense, of approximately $2,300,000. In the event of full exercise of all of the warrants, we will receive additional net proceeds of approximately $1,836,068. The actual exercise of any of the warrants, however, is beyond our control and depends on a number of factors, including the market price of our common stock. There can be no assurance that any of the warrants will be exercised.

While we have no specific plan for the proceeds, we expect to use the net proceeds of this offering, if any, to further develop our products and for general working capital purposes. The principal reason for this offering is to provide shares of common stock issuable upon conversion of our outstanding warrants issued in connection with the offer and sale of the Units.

DETERMINATION OF OFFERING PRICE

The exercise price of $0.40 was not based on any discount to the market price of our common stock. The exercise price is not necessarily related to our book value, net worth or any other established criteria of value and may or may not be considered the fair value of our common stock included in the warrants. We did not consult with any financial or other advisor in determining the exercise price. After the date of this prospectus, our common stock may trade at prices above or below the exercise price. You should not consider the exercise price as an indication of value of our company or our common stock. You should not assume or expect that our shares of common stock will trade at or above the exercise price in any given time period. The market price of our common stock may decline during or after this offering, and you may not be able to exercise or sell the shares of our common stock. You should obtain a current quote for our common stock before exercising and make your own assessment of our business and financial condition, our prospects for the future, and the terms of the warrants. On February 20, 2013, the closing sale price of our common stock on the OTC Bulletin Board was $1.00 per share.

DILUTION

Our net tangible book value as of December 31, 2012 was approximately ($595,000) or approximately ($0.05) per share. Net tangible book value per share represents the amount of our total tangible assets, less our total liabilities divided by the number of outstanding shares of common stock. Dilution in net tangible book value per share represents the difference between the amount per share paid by the purchaser of shares of common stock upon the exercise of warrants and the net tangible book value per share of common stock immediately after the exercise of warrants.

After giving effect to the exercise of 3,306,399 warrants that remained outstanding at December 31, 2012 at an exercise price of $0.40, which would have resulted in 3,057,190 common shares being issued, our pro forma net tangible book value as of December 31, 2012 would have been $627,876 or $0.04 per share. This represents an immediate increase in net tangible book value of $0.09 per share to existing stockholders and an immediate dilution in net tangible book value of $0.36 per share to warrants exercised from this offering.

The shares outstanding as of December 31, 2012 used to calculate the information in this section exclude:

- 2,294,714 shares issuable upon the exercise of stock options outstanding on December 31, 2012; and
- 14,679,971 shares issuable upon the exercise of warrants outstanding on December 31, 2012.

Unless otherwise indicated, the information in this prospectus reflects a 1-for-20 reverse split of our common stock, which was effective on March 11, 2011.

DIVIDEND POLICY

We have neither paid nor declared dividends on our common stock since our inception. We do not anticipate paying any dividends on our common stock in the foreseeable future. We expect to retain future earnings, if any, for use in our development activities and the operation of our business. The payment of any future dividends will be subject to the discretion of our board of directors and will depend, among other things, upon our results of operations, financial condition, cash requirements, prospects and other factors that our board of directors may deem relevant. Additionally, our ability to pay future dividends may be restricted by the terms of any debt financing, tax considerations and applicable law.

MARKET FOR OUR COMMON STOCK

Our common stock is quoted on the Over the Counter (OTC) Bulletin Board under the symbol “NEPH.” The following table sets forth the high and low bid and ask prices for our common stock as reported on the OTC Bulletin Board for each quarter listed. All prices have been adjusted to reflect the effect of the reverse split effective March 11, 2011. Such over the counter market quotations reflect inter-dealer prices, without retail mark-up, mark-down or commission and may not necessarily represent actual transactions.

Quarter Ended	High	Low
March 31, 2011	$.53	$.40
June 30, 2011	$.98	$.30
September 30, 2011	$2.19	$.70
December 31, 2011	$1.90	$.41
March 31, 2012	$1.09	$.44
June 30, 2012	$3.19	$.80
September 30, 2012	$1.98	$1.15
December 31, 2012	$1.40	$1.02

As of February 20, 2013, there were approximately 20 holders of record and approximately 1,000 beneficial holders of our common stock.

On February 20, 2013, the last reported sale price of our common stock on the OTC Bulletin Board was $1.00 per share.

PLAN OF DISTRIBUTION

Pursuant to the terms of the warrants, the shares of common stock will be distributed to those warrant holders who surrender their warrant certificate with their subscription form, together with the payment of the exercise price, to our warrant agent, Continental Stock Transfer & Trust Company.

MANAGEMENT’S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION

AND RESULTS OF OPERATIONS

The following discussion includes forward-looking statements about our business, financial condition, and results of operations, including discussions about management's expectations for our business. These statements represent projections, beliefs and expectations based on current circumstances and conditions and in light of recent events and trends, and you should not construe these statements either as assurances of performances or as promises of a given course of action. Instead, various known and unknown factors are likely to cause our actual performance and management's actions to vary, and the results of these variances may be both material and adverse. A list of the known material factors that may cause our results to vary, or may cause management to deviate from its current plans and expectations, is included herein under "Risk Factors" and Item 1A "Risk Factors" of our Annual Report on Form 10-K for the year ended December 31, 2012. The following discussion should also be read in conjunction with the consolidated financial statements and notes included herein.

Going Concern

Our independent registered public accounting firm has included an explanatory paragraph in their report on our financial statements included in this prospectus which expressed doubt as to our ability to continue as a going concern. The accompanying financial statements have been prepared assuming that we will continue as a going concern, however, there can be no assurance that we will be able to do so. Our recurring losses and difficulty in generating sufficient cash flow to meet our obligations and sustain our operations raise substantial doubt about our ability to continue as a going concern, and our consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty .

Business Overview

Nephros is a commercial stage medical device company that develops and sells high performance liquid purification filters. Our filters, which we call ultrafilters, are primarily used in dialysis centers and healthcare facilities for the production of ultrapure water and bicarbonate. Because our ultrafilters capture contaminants as small as 0.005 microns in size, they eliminate a wide variety of bacteria, viruses, fungi, parasites, and endotoxins harmful to humans.

All of our ultrafilters use proprietary hollow fiber technology. We believe the hollow fiber design allows our ultrafilters to be the only commercially available filters for healthcare applications that optimize the three elements critical to filter performance:

- Filtration – as low as 0.005 microns
- Flow rate – minimal disruption
- Filter life – up to 12 months

By comparison, competitive filters on the market today are typically effective only to the 0.2 micron level and are prone to clog more quickly, thus reducing their useful lives.

We were founded in 1997 by healthcare professionals affiliated with Columbia University Medical Center/New York-Presbyterian Hospital to develop and commercialize an alternative method to hemodialysis (HD). In 2009, we began to extend our filtration technologies to meet the demand for liquid purification in other areas, in particular water purification.

We have not begun to broadly market our mid-HDF system and plan to seek a commercialization partner in the U.S.

The following trends, events and uncertainties may have a material impact on our potential sales, revenue and income from operations:

- the market acceptance of our products in the United States and of our technologies and products in each of our target markets;
- our ability to effectively and efficiently manufacture, market and distribute our products;

- our ability to sell our products at competitive prices which exceed our per unit costs;
- the consolidation of dialysis clinics into larger clinical groups; and
- the current U.S. healthcare plan is to bundle reimbursement for dialysis treatment which may force dialysis clinics to change therapies due to financial reasons.

To the extent we are unable to succeed in accomplishing the foregoing, our sales could be lower than expected and dramatically impair our ability to generate income from operations.

Recently Adopted Accounting Pronouncements

In June 2011, the FASB issued ASU No. 2011-05, "Comprehensive Income (ASC Topic 220): Presentation of Comprehensive Income," ("ASU 2011-05") which amends current comprehensive income guidance. This accounting update eliminates the option to present the components of other comprehensive income as part of the statement of shareholders' equity. Instead, we must report comprehensive income in either a single continuous statement of comprehensive income which contains two sections, net income and other comprehensive income, or in two separate but consecutive statements. ASU 2011-05 will be effective for public companies during the interim and annual periods beginning after Dec. 15, 2011 with early adoption permitted. We adopted this guidance as of January 1, 2012 and since this relates to presentation only, the adoption of this guidance did not have any other effect on our consolidated financial statements.

Critical Accounting Policies and Estimates

Our discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States. The preparation of financial statements in accordance with generally accepted accounting principles in the United States requires application of management’s subjective judgments, often requiring the need to make estimates about the effect of matters that are inherently uncertain and may change in subsequent periods. Our actual results may differ substantially from these estimates under different assumptions or conditions. While our significant accounting policies are described in more detail in the notes to consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2012, we believe that the following accounting policies require the application of significant judgments and estimates.

Revenue Recognition

Revenue is recognized in accordance with Accounting Standards Codification ("ASC") Topic 605. Four basic criteria must be met before revenue can be recognized: (i) persuasive evidence that an arrangement exists; (ii) delivery has occurred or services have been rendered; (iii) the fee is fixed or determinable; and (iv) collectability is reasonably assured.

We recognize revenue related to product sales when delivery is confirmed by our external logistics provider and the other criteria of ASC Topic 605 are met. Product revenue is recorded net of returns and allowances. All costs and duties relating to delivery are absorbed by us. Shipments for all products are currently received directly by our customers.

We recognize the fixed license revenue under the Bellco license agreement on a straight line basis over the forty-two month expected obligation period which ends on December 31, 2014. Any difference between payments received and recognized revenue is reported as deferred revenue.

Deferred revenue on the accompanying December 31, 2012 consolidated balance sheet is approximately $1,414,000 and is related to the Bellco license agreement. We have recognized approximately $1,045,000 of revenue related to this license agreement to date and approximately $680,000 for the twelve months ended December 31, 2012, resulting in $1,414,000 being deferred over the remainder of the expected obligation period. We amortize the deferred revenue monthly over the expected obligation period which ends on December 31, 2014. This will result in expected recognized revenue of approximately $707,000 in each of the years ended December 31, 2013 and 2014.

The final guaranteed fixed payment of approximately $791,000 is due in January 2013 and is included in current trade receivables on the accompanying December 31, 2012 consolidated balance sheet.

Stock-Based Compensation

We account for stock-based compensation in accordance with ASC 718 by recognizing the fair value of stock-based compensation in net income. The fair value of our stock option awards are estimated using a Black-Scholes option valuation model. This model requires the input of highly subjective assumptions and elections including expected stock price volatility and the estimated life of each award. In addition, the calculation of compensation costs requires that we estimate the number of awards that will be forfeited during the vesting period. The fair value of stock-based awards is amortized over the vesting period of the award. For stock awards that vest based on performance conditions

(e.g. achievement of certain milestones), expense is recognized when it is probable that the condition will be met.

Accounts Receivable

We provide credit terms to our customers in connection with purchases of our products. We periodically review customer account activity in order to assess the adequacy of the allowances provided for potential collection issues and returns. Factors considered include economic conditions, each customer's payment and return history and credit worthiness. Adjustments, if any, are made to reserve balances following the completion of these reviews to reflect our best estimate of potential losses.

Inventory Reserves

Our inventory reserve requirements are based on factors including the products' expiration date and estimates for the future sales of the product. If estimated sales levels do not materialize, we will make adjustments to our assumptions for inventory reserve requirements.

Accrued Expenses

We are required to estimate accrued expenses as part of our process of preparing financial statements. This process involves identifying services which have been performed on our behalf, and the level of service performed and the associated cost incurred for such service as of each balance sheet date in our financial statements. Examples of areas in which subjective judgments may be required include costs associated with services provided by contract organizations for the preclinical development of our products, the manufacturing of clinical materials, and clinical trials, as well as legal and accounting services provided by professional organizations. In connection with such service fees, our estimates are most affected by our understanding of the status and timing of services provided relative to the actual levels of services incurred by such service providers. The majority of our service providers invoice us monthly in arrears for services performed. In the event that we do not identify certain costs, which have begun to be incurred, or we under- or over-estimate the level of services performed or the costs of such services, our reported expenses for such period would be too low or too high. The date on which certain services commence, the level of services performed on or before a given date and the cost of such services are often determined based on subjective judgments. We make these judgments based upon the facts and circumstances known to us in accordance with generally accepted accounting principles.

Results of Operations

Fluctuations in Operating Results

Our results of operations have fluctuated significantly from period to period in the past and are likely to continue to do so in the future. We anticipate that our annual results of operations will be impacted for the foreseeable future by several factors including the progress and timing of expenditures related to our research and development efforts, marketing expenses related to product launches, timing of regulatory approval of our various products and market acceptance of our products. Due to these fluctuations, we believe that the period to period comparisons of our operating results are not a good indication of our future performance.

The Fiscal Year Ended December 31, 2012 Compared to the Fiscal Year Ended December 31, 2011

Revenues

Total revenues for the year ended December 31, 2012 were approximately $1,807,000 compared to approximately $2,214,000 for the year ended December 31, 2011. Total revenues decreased approximately $407,000, or 18% as a

result of decreases of approximately $733,000 related to our MD filters in Europe, $346,000 related to the Office of Naval Research, whose contract ended as of March 2012, and approximately $33,000 related to the STERIS project. These decreases were partially offset by an increase of approximately $315,000 related to the Bellco license agreement as well as a 63% increase in water filter sales, which increased from $620,000 in 2011 to $1,010,000 in 2012.

Revenues were not significantly impacted by inflation or changing prices for the years ended December 31, 2012 or 2011.

Cost of Goods Sold

Cost of goods sold was approximately $737,000 for the year ended December 31, 2012 compared to approximately $1,346,000 for the year ended December 31, 2011. The decrease of approximately $609,000 or 45%, in cost of goods sold is primarily related to a $583,000 reduction in cost of goods sold of our MD filters in Europe. Additional decreases include approximately $208,000 related to the Office of Naval Research, approximately $15,000 related to DSU sales for the year ended December 31, 2012 compared to the same period in 2011 and a decrease of approximately $29,000 related to the STERIS project. These decreases were partially offset by an increase in cost of goods sold of approximately $226,000 related to filters sold to the military during the year ended December 31, 2012, a 100% increase compared to the same period in 2011. Cost of goods sold includes increases in inventory reserves of approximately $82,000 and $218,000 for the years ended December 31, 2012 and 2011, respectively.

Research and Development

Research and development expenses were approximately $632,000 and $451,000 respectively, for the years ended December 31, 2012 and December 31, 2011. This increase of approximately $181,000 or 40% is primarily due to an increase in research and development personnel related costs of approximately $136,000 during the year ended December 31, 2012 compared to the year ended December 31, 2011.

Depreciation and Amortization Expense

Depreciation and amortization expense was approximately $151,000 for the year ended December 31, 2012 compared to approximately $91,000 for the year ended December 31, 2011, an increase of 66%. The increase of approximately $60,000 is primarily due to amortization of approximately $142,000 related to the asset recognized in conjunction with the License and Supply Agreement offset partially by several assets having been fully depreciated as of year-end 2011 resulting in no depreciation expense for those assets during the year ended December 31, 2012.

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Selling, General and Administrative Expenses

Selling, general and administrative expenses were approximately $3,620,000 for the year ended December 31, 2012 compared to approximately $2,636,000 for the year ended December 31, 2011, an increase of $984,000 or 37%. The increase is primarily due to $489,000 of salary expense, an increase in legal expenses of approximately $330,000, an increase in stock compensation expense of $159,000, and $171,000 of travel related expenses during the year ended December 31, 2012 compared to the year ended December 30, 2011. These increases were partially offset by a reduction in bonus expense of approximately $165,000 for the year ended December 31, 2012 compared to the year ended December 31, 2011.

Interest Income

Interest income was approximately $2,000 for the year ended December 31, 2012 compared to approximately $4,000 for the year ended December 31, 2011. The decrease of $2,000 reflects the impact of having less cash on hand in 2012 compared to 2011.

Interest Expense

Interest expense for the year ended December 31, 2012 was $0 compared to $12,000 for the year ended December 31, 2011. Interest expense for the year ended December 31, 2011 relates to interest accrued on the $500,000 senior secured note issued to Lambda Investors LLC, which was paid in March 2011.

Amortization of Debt Issuance Costs

We account for debt issuance costs in accordance with ASC 835, which requires that these costs be reported in the balance sheet as deferred charges and amortized over the term of the associated debt. Amortization of debt issuance costs of $0 and $40,000 for the years ended December 31, 2012 and 2011, respectively, were associated with the senior secured note issued to Lambda Investors LLC. The note was paid in March 2011 and these capitalized costs were fully amortized by the first quarter of 2011.

Other Income/Expense

Other income in the amount of approximately $69,000 for the year ended December 31, 2012 was primarily due to approximately $55,000 arising from the sale of fully depreciated manufacturing equipment sold to Medica in October 2012. In addition, approximately $18,000 was related to the write-offs of vendor invoices which are no longer due. Other income was partially offset by $4,000 related to foreign currency losses on invoices paid to an international supplier.

Other expense in the amount of approximately $2,000 for the year ended December 31, 2011 was due to foreign currency loss on invoices paid to an international supplier.

Off-Balance Sheet Arrangements

We did not engage in any off-balance sheet arrangements during the periods ended December 31, 2012 and December 31, 2011.

Liquidity and Capital Resources

Our future liquidity sources and requirements will depend on many factors, including:

- receipt of scheduled payments per the Bellco S.r.l. license agreement;
- the availability of additional financing, through the sale of equity securities or otherwise, on commercially reasonable terms or at all;
- the market acceptance of our products, and our ability to effectively and efficiently produce and market our products;
- the continued progress in and the costs of clinical studies and other research and development programs;
- the costs involved in filing and enforcing patent claims and the status of competitive products; and

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 m

We do not expect to independently conduct all aspects of our vector production, product manufacturing, protocol development, and monitoring and mana and clinical programs. Although we intend to use a portion of the proceeds of this offering to expand our manufacturing capabilities and, in particular, manufacture of materials for our clinical trials, we currently rely, and expect to continue to rely, to a significant degree, on third parties for the production 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 arrangemer 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 required regulations and study and trial protocols. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or co 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 cor preclinical studies and clinical trials required to support future IND submissions and approval of our product candidates. In some such cases we may need relationship, which may not be readily available or on acceptable terms, which would cause additional delay with respect to the approval of our product 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 candi

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, inclu 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 pro the basis for FDA action, including injunction, recall, seizure or total or partial suspension of product manufact

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We and our contract manufacturer are subject to significant regulatory oversight with respect to manufacturing our products. The manufacturing fa 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 can regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accorda govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assu products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadverte product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in suppo to the FDA s GMP requirements enforced by the FDA through its facilities inspection program. Our facilities and quality systems and the facilities and c contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candida at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or the associated quality systems for comp activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will

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 m identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of su regulatory authority may require remedial measures that may be costly and/or time-consuming for us or our third-party manufacturers to implement and suspension of a clinical trial or commercial sales or the temporary or permanent closure of a manufacturing facility. Any such remedial measures imposed 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 thin a new product candidate, or revocation of a pre-existing approval. Such an occurrence may cause our business, financial condition and results o

Additionally, if supply from an approved manufacturer is interrupted, there could be a significant disruption in commercial supply of our products. We do n product candidate supply for clinical trials or commercial sale. An alternative manufacturer would need to be qualified through a BLA supplement which agencies may also require additional trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substant 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 commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more re 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

We expect to rely on academic research institutions and other CROs along with clinical trial sites to ensure our clinical trials are conducted properly an governing their activities,

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we will have limited influence over their actual performance and will control only certain aspects of our CROs activities. Nevertheless, we will be respon conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on the CROs does not relieve

We and our CROs are required to comply with the FDA s and other regulatory authorities GCP, GMP and good laboratory practice, or GLP, requirem 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 c protected. The FDA enforces these requirements through periodic inspections of study sponsors, principal investigators and clinical trial sites. If we or o requirements, the clinical data generated in our future clinical trials may be deemed unreliable and the FDA may require us to perform additional clin applications. Upon inspection, the FDA may determine that our clinical trials did not comply with GCP requirements, which may render the data generated clinical trials will require a sufficient number of test subjects to evaluate the safety and effectiveness of our product candidates. Accordingly, if our CROs recruit a sufficient number of patients, we may be required to repeat such clinical trials, which would delay the regulatory a

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 mon and resources to our clinical and nonclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, trials or other drug development activities that could harm our competitive position. If our CROs do not successfully carry out their contractual duties or o 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 f extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates. As a re prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenues cou

Switching or adding CROs involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period wh delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships wi 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, 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 fa clinical development, regulatory review or marketing approval of our product candidates or commercialization of our products, if approved, producing a product revenue.

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

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 t Genzyme became responsible for all future clinical and commercial development of the licensed wet AMD product candidate. Genzyme recently info HSV-based manufacturing technology to produce the AAV vector being used for the wet AMD product. Our license agreement with Genzyme was further We do not currently expect to derive

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substantial revenue from our license arrangement with Genzyme, but an unsuccessful outcome in pending and future clinical trials for which Genzyme
perception and prospects of our gene therapy platform. Our license relationship with Genzyme, and any future collaboration we enter into in the future
following:

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

collaborators may not perform their obligations as expected;

collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect no
commercialization programs based on clinical trial results, changes in the collaborators strategic focus or available funding, or external fact
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 cand
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 pro
competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attracti

product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates
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 comm
distribution of any such product candidate;

disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of developm
delays or termination of the research, development or commercialization of such product candidates, might lead to additional responsibilities
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
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
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
funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our developmen
candidates could be delayed and we may need additional resources to develop product candidates and gene therapy platform. All of the risks relating to p
commercialization described in this prospectus also apply to the activities of our therapeutic program collaborators

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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 Decem Genzyme, these restrictions no longer apply to the field of treatments for ocular neovascularization disorders, including AMD. In addition, under that co 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 diseas restrictions, and any similar restrictions contained in future collaborations, may have the effect of preventing us from undertaking development and other

Additionally, subject to its contractual obligations to us, if one of our collaborators is involved in a business combination, the collaborator might deer 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 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 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 business. In addition, we could face significant competition in seeking appropriate collaborators and the negotiation process is time-consuming and comple agreement will depend, among other things, upon our assessment of the collaborator s resources and expertise, the terms and conditions of the propose evaluation of a number of factors. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements f and development pipeline may be insufficient, our product candidates may be deemed to be at too early of a stage of development for collaborative effo candidates as having the requisite potential to demonstrate safety and efficacy. If we license product candidates, we may not be able to realize the ben successfully integrate them with our existing operations and company culture. We cannot be certain that, following a strategic transaction or license, we w 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 developmer development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or market undertake development or commercialization activities at our own expense. If we elect to fund and undertake development or commercialization activities 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 su necessary development and commercialization activities, we may not be able to further develop our product candidates or bring them to market or continu 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 trad

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

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Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases th our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our p know-how and trade secrets, a competitor s discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position business.

In addition, these agreements typically restrict the ability of our collaborators, advisors, employees and consultants to publish data potentially relating to 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 in collaboration. In other cases, publication rights are controlled exclusively by us, although in some cases we may share these rights with other parties. We programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. Despite our effor may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secre otherwise protected rights at the time of publication. A competitor s discovery of our trade secrets would impair our competitive position and ha

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 currently have no sales and marketing organization and have no experience selling and marketing our product candidates. To successfully commerc 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 to market any products we may develop will be expensive and time-consuming, particularly to the extent that we seek to commercialize any product patient population significantly larger than those addressed by our current lead product candidates, and could delay any product launch. Moreover, we cann develop this capability. We may enter into collaborations with other entities to utilize their mature marketing and distribution capabilities, but we may be 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 c 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 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 Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully agai

We face intense competition and rapid technological change and the possibility that our competitors may develop therapies that are more advanced or e 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 proprieta successfully develop and commercialize will have to compete with existing therapies and new therapies that may become available in the future. While we and scientific expertise in the gene therapy field provide us with competitive advantages, we face potential competition from many different sources, inclu specialty pharmaceutical and biotechnology

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companies, as well as from academic institutions and governmental agencies and public and private research institutions that may develop potential

Currently there are no approved products for any of our lead orphan ophthalmology indications of XLRS, ACHM and XLRP. We believe the key compet product candidates, if approved, are likely to be their efficacy, safety, convenience of administration and delivery, price, the level of generic competitio 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., BioMar Corp., Audentes Therapeutics, GenSight Biologics, ReGenX Biosciences, LLC, or ReGenX, Avalanche Biotechnologies, Inc., or Avalanche, Regeneron P or Spark, Voyager Therapeutics, Inc., Dimension Therapeutics, Inc., Sangamo Biosciences, Inc. and Hemera Biosciences, Inc., or Hermera. We believe th field of orphan ophthalmology on which we are currently focused include Genzyme and Spark, whose programs are at the clinical stage, Avalanche, GenS and ReGenX, as well as two smaller, early-stage companies, RetroSense Therapeutics, LLC and Eos Neuroscience, Inc., all of whose programs we believe 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 discovery and development of product candidates, obtaining FDA and other regulatory approvals of treatments and the commercialization of those tr biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. Our c eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, wh 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 reim 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 regul reimbursement by governmental and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of our proc domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit an or reimbursed by government authorities, private health coverage insurers and other third-party payors. Coverage and reimbursement by a third-party p 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 scientific, clinical and

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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 reimburs available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, th high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investme

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. In the United States, third-party payors, in as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbu increasingly are used as models for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs and b have been approved for coverage and reimbursement by the Centers for Medicare & Medicaid Services, or CMS, the agency responsible for administerin predict what CMS will decide with respect to coverage and reimbursement for fundamentally novel products such as ours, as there is no body of establi products. Moreover, reimbursement agencies in Europe may be more conservative than CMS. For example, a number of cancer drugs have been approved 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 t candidates.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and cost-containment initiatives in Europe, Canada, and other countries has and will continue to put pressure on the pricing and usage of our product candida products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are sub countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may b 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 orga reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to expe sale of any of our product candidates, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and ad pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, 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 candid 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 approve 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 specializing in the treatment of those diseases that our product candidates target prescribing treatments that involve the use of our product candidates in lie are already familiar with and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would ha condition and may delay or impair the development and

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commercialization of our product candidates or demand for any products we may develop. For example, trials using early versions of lentiviral vectors, w
cell s DNA, have led to several well-publicized adverse events, including reported cases of leukemia. Although none of our current product candidates uti
a viral delivery system. Adverse events in our clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity coul
unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those prod
in demand for any such product candidates.

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

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 P
amended by the Health Care and Education Reconciliation Act, or PPACA, was passed, which substantially changes the way health care is financed by
significantly impacts the U.S. pharmaceutical industry. The PPACA, among other things, subjects biologic products to potential competition by lower-cos
which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or in
owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care orga
manufacturers of certain branded prescription drugs, and subjects additional drugs to lower pricing under the 340B drug pricing program by a

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 C
measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of
2021, was unable to reach required goals, thereby triggering the legislation s automatic reduction to several government programs. This includes aggrega
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 adopte
amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product ca

The commercial success of any of our product candidates will depend upon its degree of market acceptance by physicians, patients, third-party pay

Ethical, social and legal concerns about gene therapy and genetic research could result in additional regulations restricting or prohibiting the products and
approvals from the FDA in the United States and other government bodies internationally, the commercial success of our product candidates will depend in
third-party payors acceptance of gene therapy products in general, and our product candidates in particular, as medically necessary, cost-effective, and sa
not gain market acceptance by physicians, patients, third-party payors and others in the medical community. If these products do not achieve an adequa
significant product revenue and may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale,

the efficacy and safety of such product candidates as demonstrated in clinical trials;

the potential and perceived advantages of product candidates over alternative treatments;

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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

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;

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 n efforts to educate the medical community and third-party payors on the benefits of the product candidates may require significant resources and may ne marketplace may require more resources than are required by the conventional technologies marketed by our competitors. If any of our product candid acceptance among physicians, patients, or health care payors, we will not be able to generate significant revenues from such product, which could hav 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 c

If any of our product candidates are approved for commercialization, we may enter into agreements with third parties to market them on a worldwide basi expect that we will be subject to additional risks related to entering into international business relationships, inclu

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 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 intel
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 doi

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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, f

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

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 platfo are currently in clinical or preclinical development, we may fail to identify other potential product candidates for clinical development for a number of rea may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects or may l 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 ma potentially cause us to cease operations. Research programs to identify new product candidates require substantial technical, financial and human resourc potential programs or product candidates that ultimately prove to be unsuccessful.

Risks related to our business operations

We incur significant increased costs as a result of operating as a public company, and our management devotes substantial time to

As a public company, we have incurred and will continue to incur significant legal, accounting and other expenses that we did not incur as a private compa as rules subsequently implemented by the Securities and Exchange Commission, or SEC, and The NASDAQ Global Market impose various requireme Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and exe Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as say on pay and proxy access. Recent legislation per implement many of these requirements over a longer period and up to five years from the date of our initial public offering, which was March 26, 2014 accorded to us by this legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and t activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulat to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently

We may not be successful in complying with these obligations, and compliance with these obligations could be time-consuming and expensive. If the management and personnel from other business concerns, they could have a material adverse effect on our business, financi

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results of operations. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time
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
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
increase the prices of our products or services.

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

Our management has determined that as of June 30, 2013, we had material weaknesses in our internal control over financial reporting, which relate to the d
reporting processes and our accounting for debt, equity and convertible instruments. We have concluded that these material weaknesses in our internal co
that we do not have the appropriate resources with the appropriate level of experience and technical expertise to oversee our closing and financial reportin
financial reporting requirements related to our issuances of convertible notes, preferred stock warrants, stock options, preferred stock and preferred stock p
not yet been remediated.

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 misstatemen
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. Ou
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 re
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

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

If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial and other systems and resources to m
development activities, and, in the longer term, build a sales force and commercial infrastructure to support commercialization of any of our product candi
would impose significant added responsibilities on members of management. It is possible that our management, finance, development personnel, syster
adequate to support this future growth. Our need to effectively manage our operations, growth and products requires that we continue to develop more robu
and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement thes
not achieve our research, development and growth goals.

***We may enter into or seek to enter into business partnerships, combinations and/or acquisitions which may be difficult to integrate, disrupt our busi
stockholder value.***

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

- the difficulty of integrating the operations and personnel of the acquired companies;
- the potential disruption of our ongoing business and distraction of management;
- potential unknown liabilities and expenses;

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the failure to achieve the expected benefits of the combination or acquisition;

the maintenance of acceptable standards, controls, procedures and policies; and

the impairment of relationships with employees as a result of any integration of new management and other personnel.

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

Our future success depends on our ability to retain key employees, consultants and advisors and to attract, retain and motivat

We are highly dependent on our executive officers, the loss of whose services may adversely impact the achievement of our objectives. Recruiting and re and advisors for our business, including scientific and technical personnel, will also be critical to our success. There is currently a shortage of skilled exec 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 r competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in preclin challenging to recruit and retain qualified personnel. The inability to recruit or loss of the services of any executive, key employee, consultant or adviso 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. Th 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

Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us. officers or other key employees and our inability to find suitable replacements could potentially harm our business, prospects, financial condition or resul 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 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 resource the industry than we do. They may also provide more diverse opportunities and better chances for career advancement. Some of these characteristics may 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 a limited.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other impr

We are exposed to the risk that our employees, CROs, principal investigators, consultants and commercial partners may engage in fraudulent conduct or unauthorized

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activities to us. Misconduct by these parties could include intentional, reckless and/or negligent failures to comply

the laws and regulations of the FDA and non-U.S. regulators, including those laws requiring the reporting of true, complete and accurate info

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 abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, c arrangements. Such misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regula 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 misconduc prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or othe 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 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 have not fully complied, with such laws, we could face substantial penalties.

Our operations may be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse law product candidates and begin commercializing those products in the United States, many of these laws will become more directly applicable to our operat Health Care Program Anti-Kickback Statute, the federal civil and criminal False Claims Acts and Physician Payments Sunshine Act and regulations. The proposed sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states i 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 willfull remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind in return for, the purchas 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 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 executing a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., pu

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulat omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under

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HITECH and the Genetic Information Nondiscrimination Act; Other Modifications to HIPAA, published in January 2013, which imposes cer security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such health care providers;

federal transparency laws, including the federal Physician Payment Sunshine Act that requires disclosure of payments and other transfers of v hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and

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

federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm co

federal government price reporting laws, which require us to calculate and report complex pricing metrics to government programs, where su calculation of reimbursement and/or discounts on our marketed drugs, when and if approved; participation in these programs and compliance us to potentially significant discounts on our products, when and if approved, increased infrastructure costs and potentially limit our ability to

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 rein commercial insurers; state laws that require pharmaceutical companies to comply with the industry s voluntary compliance guidelines and th by the federal government, or otherwise restrict certain payments that may be made to healthcare providers and other potential referral source report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; an of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, th circumstances, such as specific disease states.

In addition, any sale of our products or product candidates, if commercialized outside of the United States, may also subject us to foreign laws governing p including laws similar to the U.S. healthcare laws mentioned above. Because of the breadth of these laws and the narrowness of the statutory exceptions an 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 other things, amends the intent requirements of the federal Anti-Kickback Statute and the criminal statute governing healthcare fraud. A person or enti Anti-Kickback Statute and the federal criminal healthcare fraud statute without actual knowledge of the statute or specific intent to violate it. In addition, assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim fo

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 subj penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, disgorgement, contractual dan future earnings, imprisonment, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our

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

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 li brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. candidate we develop allegedly causes injury or is found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such pro defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability, and a breach of warranties. Clai protection acts. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. In addition, regardless of me may result in:

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

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could preve we develop. While we believe our product liability insurance coverage is sufficient in light of our current clinical programs, The amount of the product liab 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 num candidates in these trials. The amount of our product liability coverage as of March 31, 2014 was $10.0 million. This amount may increase or decrease i 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 c 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 marke to expand our insurance coverage to include the commercial sale of our products; however, we may be unable to obtain product liability insurance on co 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 succe brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our resu

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 of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. O products. We generally

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contract with third parties for the disposal of these materials and wastes. Although we believe that our procedures for using, handling, storing and dispo prescribed standards, we cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from o liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil

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

We rely on our relationship with a professional employer organization for our human relations function and as a co-employer of our personnel, and if under that relationship, our relations with our employees could be damaged and we could incur liabilities that could have a material a

All of our personnel, including our executive officers, are co-employees of AGTC and a professional employer organization, TriNet HR Corporation, or TriNet is the formal employer of all of our personnel, and is responsible for administering all payroll, including tax withholding, and providing health insu 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 obligation our employees could be damaged. We could, under certain circumstances, be held liable for a failure by TriNet to appropriately pay, or withhold and employees. In such a case, our potential liability could be significant and could have a material adverse effect on our

We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans disaster.

Substantially all of our operations are conducted from our headquarters located near Gainesville, Florida. Hurricanes or other natural disasters could severe facilities or destroy stored research materials that could be difficult to replace, and otherwise have a material adverse effect on our business, results of op addition, despite the implementation of security measures, our internal computer systems and those of our current and any future CROs and other contr vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. If such a 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 or a significant portion of our headquarters, that damaged critical infrastructure or that otherwise disrupted our operations or the operations of our third-par 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 t approval efforts and significantly increase our costs to recover or reproduce the data. If our security measures, disaster recovery and business continuity breach, serious disaster or similar event, we could incur substantial expenses and the further development and commercialization of our product candidates adverse effect on our business.

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

Our product candidates are manufactured using technically complex processes requiring specialized facilities, highly specific raw materials and other pr processes, as well as strict company and government standards for the manufacture and storage of our products, subjects us to production risks. While proc for commercialization undergo sample testing, some defects may only be identified following product release. In addition, process deviations or unanticip result in these intermediate products not complying with stability requirements or specifications. Most of our product candidates must be stored and transp these environmental conditions deviate, our product candidates remaining shelf-lives could be impaired or their efficacy and safety could become advers use. The occurrence or suspected occurrence of production and distribution difficulties can lead to lost inventories, and in some cases product recalls, with of product liability. The investigation and remediation of any identified problems can cause production delays, substantial expense, lost sales and delays o supply of finished products or the loss thereof could hinder our ability to timely distribute our products and satisfy customer demand. Any unforeseen failu could delay our clinical trials and, if our product candidates are approved, result in a loss of our market share and negatively affect ou

We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or pr 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 lat Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on programs for product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target mar relinquish valuable rights to that product candidate through strategic collaboration, licensing or other royalty arrangements in cases in which it would hav development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in w 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 car 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 5 limitation may significantly reduce the utilization of our net operating loss carryforwards before they expire. The closing of this offering, alone or toget 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 c 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 offeri 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 co change has occurred, or whether there have been multiple ownership changes since our inception, due to the significant costs and complexities associated 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, Section 382, which could limit the amount of net operating loss carryforwards that could be utilized annually in the future to offset o

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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 suff and commercialize technology and products similar or identical to ours, and our ability to successfully commercialize our technology c

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 prop protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies an

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applicatio is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Morec right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third pa 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 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 l treatment of the human body more than United States law does. Publications of discoveries in the scientific literature often lag behind the actual discoveri 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 whe 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 resu 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 which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of th 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 partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any s reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, witho manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our p could dissuade companies from collaborating with us to license, develop or commercialize current or future product c

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, pr otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar 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 c United States and

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abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in w
stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology an
for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such
owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products sim

Third parties may initiate legal proceedings alleging claims of intellectual property infringement, the outcome of which would be uncertain and could h
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 o
States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuit
reexamination proceedings before the United States Patent and Trademark Office and corresponding foreign patent offices. Numerous United States an
applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmace
issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of th

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications
of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to
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 c
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 cand
manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candi
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 meth
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
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
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 commer
Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources
claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys fees for willful infringement, pay roy
one or more licenses from third parties, which may be impossible or require substantial time and monetary expen

We may not be successful in obtaining or maintaining necessary rights to gene therapy product components and processes for our development pip

Presently we have rights to the intellectual property to develop our gene therapy product candidates. Because a key element of our business strategy is to
acquisitions for additional product candidates that may require the use of proprietary rights held by third parties, the

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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 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-pa that we identify on terms that we find acceptable, or at all. The licensing and acquisition of third-party intellectual property rights is a competitive area, an also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may hav 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 unde 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 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 property rights to other parties, potentially blocking our ability to pursue our program.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire t 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 prope 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 experie 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 b industry. We are a party to intellectual property license agreements with the University of Florida Research Foundation, an affiliate of the University of Research Foundation, an affiliate of The University of Alabama at Birmingham and the Trustees of the University of Pennsylvania, each of which is impor additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various di 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 t 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 fro 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 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 patents do not exist which might be enforced against our current product candidates or future products, resulting in either an injunction prohibiting our sale 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 oth 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 intellectual property. In certain cases, we control the

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prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur signific
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 agreemen

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 pa

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
commercialize the affected product candidates.

***We may become involved in lawsuits to protect or enforce our patents or other intellectual property or the patents or other intellectual property of
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 req
expensive and time-consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us, alleging
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 pate
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 def
patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not iss

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
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 co
offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in su
other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries
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 con
disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or de
perceive these results to be negative, it could have a material adverse effect on the price of our common stoc

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

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 defend
our product

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candidate is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonp an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceabilit connected with prosecution of the patent withheld relevant information from the United States Patent and Trademark Office, or made a misleading statem raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-exa 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 following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we could l protection on one or more of our product candidates. Such a loss of patent protection could have a material adverse impact

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information 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 p 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 b consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary 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 payin intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigat distraction to management and other employees.

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

We may be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patents or other intellectual p employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to u agreement with each party who in fact develops intellectual property that we regard as our own. We could be subject to ownership disputes arising, fo consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challe defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or r 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 subst and other employees.

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

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceed or investors perceive these results to be

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negative it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operati development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such liti competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. U continuation of patent litigation or other proceedings could compromise our ability to compete in the marketpla

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

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 Un 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 ou patent agencies. The United States Patent and Trademark Office and various non-U.S. governmental patent agencies require compliance with a number of p similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply, and in many cases, an 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 lap 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 cir on our business.

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

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

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or d 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 change that affect the way patent applications are prosecuted and may also affect patent litigation. The United States Patent and Trademark Office recently develo 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 fir 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-Smit uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could hav financial condition.

Moreover, recent United States Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the 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 on decisions by the United States Congress, the federal courts, and the United States Patent and Trademark Office, the laws and regulations governing pa would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in

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

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, o typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA objects to we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable tradem 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 intellectu 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 right the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or fro inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United S products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from c

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal syste developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotech for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce 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 interpr 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 rem meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial ad develop or license.

Risks related to this offering and ownership of our common stock

An active trading market for our common stock may not be sustained.

Although we have listed our common stock on The NASDAQ Global Market, an active trading market for our common stock may not be sustained. In th common stock, you may not be able to sell your common stock at or above the public offering price or at the time that you would like to sell. An inactive raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by usi

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

Fluctuations in the price of our common stock could contribute to the loss of all or part of your investment. The public offering price for the shares of our c that will prevail in the trading market following this offering. Since our initial public offering in March 2014, the trading price of our common stock has continue to be, highly volatile and could be

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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 ad
stock and our common stock may trade at prices significantly below the public offering price in this offering. In such circumstances the trading price of
experience a further decline.

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

our failure to develop and commercialize our product candidates;

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

changes in the market s expectations about our operating results;

adverse results or delays in 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 p

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

changes in financial estimates and recommendations by securities analysts concerning our company, the gene therapy market, or the biotechn

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

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 fo
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;

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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

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

Broad market and industry factors may materially harm the market price of our common stock irrespective of our operating performance. The stock marke
and the market for biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or dispr
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
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, c
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 comm

The trading market for our common stock relies in part on the research and reports that industry or financial analysts publish about us, our business, our m
these analysts. As a newly public company, we have only limited coverage by securities analysts. If securities analysts do not continue to cover our com
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 analys
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 c
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 wit

The concentration of our capital stock ownership with insiders upon the closing of this offering will limit your ability to influe

We anticipate that our executive officers, employees, directors, current 5% or greater stockholders, and their respective affiliates will together beneficiall
63.2% of the shares of our outstanding common stock, assuming no exercise of outstanding options or warrants following the closing of this offering (a
over-allotment option). As a result, these executive officers, directors and principal stockholders, acting together, will have substantial influence over
stockholders, including the election of directors, any merger, consolidation or sale of all or substantially all or of our assets or any other significant corpora
even if other stockholders, including those who purchase shares in this offering, oppose such action. These stockholders may delay or prevent a change
acquirer from attempting to obtain control of our company, even if such change of control would benefit our other stockholders. This concentration of st
perception of our corporate governance or delay, prevent or cause a change in control of our company, any of which could adversely affect the

We are an emerging growth company, and the reduced reporting requirements applicable to emerging growth companies may make our c

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 ad
requirements that are

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applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requiren of 2002, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previous company for up to five years from the date of our initial public offering on March 26, 2014, although circumstances could cause us to lose that status earlie 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 m 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 deb 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 allow us to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor att Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statemen 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 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 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 st emerging growth companies. As a result, changes in rules of U.S. generally accepted accounting principles or their interpretation, the adoption of new gui changes in our business could significantly affect our financial position and results of operations.

A significant portion of our total outstanding shares is restricted from immediate resale but may be sold into the market in the near future, which coul to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market th common stock intend to sell shares, could reduce the market price of our common stock. After this offering, we will have 16,077,942 shares of common st outstanding immediately following the closing of the underwriters over-allotment in our initial public offering on April 3, 2014 and the issuance of the exercise of the underwriters over-allotment option). Other than the 4,791,667 shares sold by us in our initial public offering, substantially all of the outsta a 180-day contractual lock-up with the underwriters for our initial public offering, which period began on March 26, 2014, and approximately 9.1 milli lock-up with the underwriters for this offering, which period will begin on the date of effectiveness of the registration statement of which this prospectus fo any applicable volume limitations under federal securities laws, after the earlier of the expiration of, or release from, the applicable lock-up period. The stock may be freely sold in the public market at any time to the extent permitted by Rules 144 and 701 under the Securities Act of 1933, as amended, whi holders of a substantial portion of our common stock have rights, subject to conditions, to require us to file registration statements covering their shares or that we may file for ourselves or other stockholders.

In addition, as of June 30, 2014, there were 1,043,748 shares subject to outstanding options under our equity incentive plans, all of which shares we pl registration statement on Form S-8. These shares, once vested and issued upon exercise, will be able to be freely sold in the

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subject to volume limits applicable to affiliates and the lock-up agreements described above, to the extent applicable. Furthermore, as of June 30, 2014, th warrants. These shares will become eligible for sale in the public market to the extent such warrants are exercised and to the extent permitted by the lock-u Act.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could resu 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, comm development activities, potential acquisitions, in-licenses, or collaborations and costs associated with operating a public company. To raise capital, we ma 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 securit transaction, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, and new 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 t

If you purchase shares of our common stock in this offering, you will experience immediate and substantial dilution, as the public offering price of our com net tangible book value per share of our common stock. If you purchase our common stock in this offering, you will suffer immediate and substantial dil underwriters exercise their over-allotment option, or if outstanding options and warrants to purchase our common stock are exercised, you will experience 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 pro 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 i 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 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 proceeds from this offering, their ultimate use may vary substantially from their currently intended use. While we have not allocated these estimated net p use the net proceeds from this offering to develop our product candidates and for general corporate purposes, including working capital. We may also us in-licenses of products and technologies that are complementary to our business. Although we have from time to time evaluated possible acquisitions and or agreements to make any material acquisition or in-license, and we may not make any acquisitions in the future. The failure by our management to a business. Pending their use, we may invest the net proceeds from this offering in short-term, investment-grade, interest-bearing securities. These investm 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 fina to decline.

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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 o 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 shares of our common stock in the foreseeable future. As a result, you may only receive a return on your investment in our common stock if the market pric 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 share

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 e companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion 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 Delawa change of control of our company or changes in our management and, therefore, depress the trading price of our co

Our certificate of incorporation, bylaws and Delaware law contain provisions that could have the effect of rendering more difficult or discouraging an ac directors, even if doing so would benefit our stockholders or remove our current management. Our corporate governance docume

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 sto

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

requiring advance notice of stockholder proposals for business to be conducted at meetings of our stockholders and for nominations of candi

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 boa

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 manag

As a Delaware corporation, we are also subject to provisions of Delaware law, including Section 203 of the Delaware General Corporation Law, which p from engaging in a business combination with an interested stockholder, generally a person which together with its affiliates owns, or within the last three period of three years after the date of the transaction in which the person became an interested stockholder, unless the business combination is

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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
potential acquirers of our company, thereby reducing the likelihood that you could receive a premium for your common stock

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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
these statements by forward-looking words such as may, could, should, would, intend, will, expect, anticipate, believe, estima
similar words. Any statements contained herein that are not statements of historical facts may be deemed to be forward-looking statements. You should
discuss our future expectations, contain projections of our future results of operations or of our financial condition or state other forward-looking inform
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, including wet AMD;
- our plans to conduct additional preclinical studies of our product candidate for treatment of AAT;
- our plans to pursue in-licensing, co-development, intellectual property acquisition or manufacturing agreements;
- our plans to expand our manufacturing capabilities and create a pilot manufacturing group;
- 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 a
risks and uncertainties. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should no
statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we ma
cautionary statements included in this prospectus, particularly in the Risk Factors section, that could cause actual results or events to differ materially fr
Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. Our forward-looking statements do

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
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
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 2,000,000 shares of our common stock in this offering will be approximately $27.8 millio commissions and estimated offering expenses payable by us. If the underwriters exercise their over-allotment option in full, we estimate that the net proc $32.0 million, after deducting underwriting discounts and commissions and estimated offering expenses payable

We plan to use the net proceeds from this offering as follows:

approximately $8 million to $12 million to fund our preclinical investigation and Phase 1/2 trials of potential product candidates for the treatr

approximately $3 million to $5 million to expand our manufacturing capabilities and, in particular, to develop a pilot program for in-house pr manufacturing at up to 100L scale, including expenditures for capital equipment of approximately $2.5 million; and

the balance to in-license, acquire or invest in complementary gene therapy technologies, products or assets and for working capital and other While we have and will continue to monitor the market for opportunities to in-license, acquire or invest in complementary gene therapy technologies, p agreement or commitment for any specific in-license, acquisition or investment and we have not allocated any portion of the estimated net proce

Our expected use of net proceeds from this offering represents our current intentions based upon our present plans and business condition. As of the da 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 u 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 regulatory submissions and evolving regulatory requirements, the amount and timing of our actual expenditures will depend upon such variables and we ca 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 ap addition, we might decide to postpone or not pursue certain clinical trials or preclinical activities if the net proceeds from this offering and the othe

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MARKET PRICE OF OUR COMMON STOCK

Our common stock has been listed on The NASDAQ Global Market under the symbol AGTC since March 27, 2014. Prior to that date, there was no pu
table sets forth, for the periods indicated, the high and low intraday sales prices of our common stock as reported by The NASD

Third fiscal quarter 2014 (beginning March 27, 2014)	
Fourth fiscal quarter 2014	
First fiscal quarter 2015 (through July 24, 2014)	

On July 24, 2014, the closing price of our common stock as reported on The NASDAQ Global Market was $15.61 per share. As of June 30, 2014, we ha

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
business. We do not expect to pay any cash dividends on our common stock in the foreseeable future. See Risk Factors Risks related to this offering
currently intend to pay dividends on our common stock and, consequently, your ability to achieve a return on your investment will depend on the appr

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CAPITALIZATION

The following table sets forth our cash and cash equivalents short-term investments, convertible preferred stock and capitalization a

An actual basis;

A pro forma basis giving effect to the completion of our initial public offering, including our issuance of an aggregate 4,791,667 shares of co $51.8 million, after deducting underwriting discounts and offering expenses, the conversion of all of our preferred stock into 9,120,081 share connection with the initial closing thereof and the conversion of all outstanding warrants exercisable for shares of Series A-1, Series A-1A ar exercisable for shares of common stock, resulting in our preferred stock warrant liability being reclassified to additional paid-in capital; and

A pro forma as adjusted basis, giving additional effect to the sale of 2,000,000 shares of our common stock offered in this offering at the offe underwriting discounts and commissions and estimated offering expenses payable by us.

You should read the following table in conjunction with our financial statements and related notes, Selected Financial Data and Management s Discus of Operations appearing elsewhere in this prospectus.

	Actu
Cash and cash equivalents	$ 8,(
Short-term investments	$ 16,5
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	$ 73,7
Common stock, $0.001 par value; 150,000,000 shares authorized; shares issued: 166,195 actual; 14,077,943 pro forma; 16,077,943 pro forma as adjusted	$
Additional paid-in capital	12,(
Accumulated deficit	(59,8
Total stockholders (deficit) equity	(47,1
Total capitalization	$ (47,1

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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 public offering price per share yo
adjusted net tangible book value (deficit) per share of our common stock after this offering.

We had a historical net tangible book value (deficit) of $(48.7) million, or $(293.32) per share of common stock, as of March 31, 2014. Historical net tan
tangible assets, less total liabilities and preferred stock, divided by the number of outstanding shares of our commo

Our pro forma historical net tangible book value (deficit) as of March 31, 2014 was $77.3 million, or $5.49 per share of common stock, taking into accou
including our issuance of an aggregate 4,791,667 shares of common stock for net proceeds of $51.8 million, after deducting underwriting discounts and
preferred stock into 9,120,081 shares of common stock on April 1, 2014 in connection with the initial closing thereof and the conversion of all outstandin
Series A-1A and Series B-1 preferred stock into warrants exercisable for shares of common stock, resulting in our preferred stock warrant liability bei

After giving effect to our issuance and sale of 2,000,000 shares of common stock in this offering, and after deducting underwriting discounts and commissi
us, the pro forma as adjusted net tangible book value (deficit) as of March 31, 2014 would have been $105.1 million, or $6.54 per share. This represents a
book value to existing stockholders of $1.05 per share. The public offering price per share will significantly exceed the pro forma as adjusted net tangi
investors who purchase shares of common stock in this offering will suffer an immediate dilution of their investment of $8.46 per share. The following ta
investors purchasing shares of common stock in this offering without giving effect to the over-allotment option granted to t

Public offering price	
Historical net tangible book value (deficit) per share as of March 31, 2014	
Increase per share attributable to the completion of our initial public offering	
Pro forma historical net tangible book value (deficit) per share as of March 31, 2014	
Increase per share attributable to sale of shares of common stock in this offering	
Pro forma as adjusted historical net tangible book value per share as of March 31, 2014	
Dilution per share to new investors	

If the underwriters exercise their over-allotment option in full, the pro forma as adjusted historical net tangible book value (deficit) will increase to $6.67 p
existing stockholders of $1.18 per share and an immediate dilution of $8.33 per share to new investors. If any shares are issued upon exercise of outstan
further dilution.

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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 Operations appearing elsewhere in this prospectus. Our selected statement of operations data for the fiscal years ended June 30, 2012 and 2013 and our se 2013 are derived from our audited financial statements included elsewhere in this prospectus. Our selected statement of operations data for the nine mon selected balance sheet data as of March 31, 2014 have been derived from our unaudited financial statements included elsewhere in this prospectus. Our hi 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. Th intended to replace our financial statements and the related notes.

	Fiscal Yea June 2012	
Statement of Operations Data:		
Revenue:		
Grant revenue	$ 718	
Sponsored research revenue	364	
Total revenue	1,082	
Operating expenses:		
Research and development	2,354	
General and administrative	787	
Total operating expenses	3,141	
Loss from operations	(2,059)	
Other income (expense):		
Interest income		
Interest expense	(69)	
Fair value adjustments to warrant liabilities (1)	204	
Fair value adjustments to Series B purchase rights (1)		
Total other income (expense), net	135	
Net loss	$ (1,924)	
Net loss per share, basic and diluted (2)	$ (17.65)	
Weighted-average shares outstanding, basic and diluted (2)	109	
Pro forma net loss per share, basic and diluted (unaudited) (2)		
Weighted-average pro forma shares outstanding, basic and diluted (unaudited) (2)		

	2012	
Balance Sheet Data:		
Cash and cash equivalents	$	7
Short-term investments	$	
Working capital	$	(3
Total assets	$	2,8
Current liabilities	$	1,4

Total stockholders (deficit) equity	$ (31,2

(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
(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 sha

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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 rela the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and st includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the Risk Facto could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. See 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 pa ophthalmology. Our lead product candidates, which are each in the preclinical stage, are treatments for X-linked retinoschisis, or XLRS, achromatopsia, o XLRP. These rare diseases of the eye are caused by mutations in single genes, significantly affect visual function and currently lack effective medical tre 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 p 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 characterized by progressive degeneration of the retina, leading to total blindness in adult men. In the longer term, we will seek opportunities to take adv platform to address a range of genetic diseases, both within and beyond our initial focus area of orphan ophthalm

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 opl or AAT deficiency, an inherited orphan lung disease, including activities to manufacture product in compliance with good manufacturing practices, prepar our product candidates, providing general and administrative support for these operations and protecting our intellectual property. We do not have any pro any revenue from product sales. We have funded our operations primarily through the private placement of preferred stock, common stock, convertible not our initial public offering, which closed in April 2014. We have also been awarded grant funding aggregating $10.6 million between our inception and Ma 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 Ey Health, or NIH, to support development of our ACHM product candidate. As a sub-awardee, as of March 31, 2014, we had received $0.4 million and we over the remaining three years of 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, for the nine months ended March 31, 2014. Substantially all our net losses resulted from costs incurred in connection with our research and development costs associated with our operations. We expect to continue to incur significant expenses and increasing operating losses for at least the next several y 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 th ophthalmology and in wet AMD;

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manufacture clinical trial materials and develop large-scale manufacturing capabilities;

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.

As of March 31, 2014, we had cash and cash equivalents and short-term investments of $24.5 million. Based on our preliminary analysis of our financial re and giving effect to our receipt of the net proceeds from our initial public offering, at June 30, 2014, we had cash and cash equivalents and short-term i preliminary, unaudited, subject to change upon completion of our year-end audit for our fiscal year ended June 30, 2014, and may differ from what will be of and for our fiscal year ended June 30, 2014.

We do not expect to generate revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one 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 ou 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 approval for our lead product candidates and to build the sales, marketing and distribution infrastructure that we believe will be necessary to commercializ will require substantial additional funding. Also, our current operating plan may change as a result of many factors currently unknown to us, and we ma planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other c arrangements or a combination of these approaches. However, we may be unable to raise additional funds or enter into such other arrangements when nee raise capital or enter into such other arrangements as and when needed would have a negative impact on our financial condition and our a

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 appro commercialize any approved products. Because of the numerous risks and uncertainties associated with product development, we are unable to predict the t 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, includin 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 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 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 gen In the two fiscal years ended June 30, 2012 and 2013, and the nine months ended March 31, 2014, all our revenues were derived from grants. Our grant re and development grant programs offered by federal, state, and local governments and agencies, including the United States Food and Drug Administration as the Foundation Fighting Blindness, or FFB, and the Alpha-1 Foundation. Grant revenue is recognized when there is reasonable assurance that the grant terms of the grant. Prior to fiscal year 2012, we also derived revenue from collaboration and license fees received under our agreement with Genzyme C expect to derive substantial additional revenue from our agreement with Genzyme.

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Research and development expenses

Research and development expenses consist primarily of costs incurred for the development of our product candidates, w

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

expenses incurred under agreements with academic research centers, contract research organizations, or CROs, and investigative sites that co

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

Research and development costs are expensed as incurred. Costs for certain development activities are recognized based on an evaluation of the progress to 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 w commercialization and sale of any of our product candidates that obtain regulatory approval. We may never succeed in achieving regulatory approval for costs, and timing of clinical trials and development of our product candidates will depend on a variety of factors, in

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 product candidate. For example, if the FDA, or another regulatory authority were to require us to conduct clinical trials beyond those that we currently ar 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 expen time on the completion of clinical development.

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

Product candidate or program	
XLRS	
ACHM	
XLRP	
LCA2	
Other orphan ophthalmology indications	
General research and process development	
AAT deficiency	
Total	

We plan to increase our research and development expenses for the foreseeable future as we continue the development of our XLRS, ACHM and XLR
applications of our gene therapy platform in other indications in orphan ophthalmology. Our current planned research and development ac

- 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 o
- 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
- we are currently designing preclinical studies to further evaluate the ability of an AAV vector to delay disease progression in animal models
will conduct additional preclinical studies required for submission of an IND to the FDA;
- we are currently reviewing possible targets for development of a treatment for wet AMD. If this review is successful, we will conduct preclin
to the FDA;
- we intend to devote substantial research and development resources to expansion of our manufacturing capabilities; 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 expense
finance and human resource functions. Other general and administrative expenses include facility-related costs and professional fees for directors, accoun
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 d
our product candidates. We also anticipate increased expenses related to audit, legal, regulatory, and tax-related services associated with maintaining comp
Exchange Commission requirements, director and officer insurance premiums, and investor relations costs associated with being a public company. Add
approval of the first product candidate appears likely, we anticipate an increase in payroll and related expenses as a result of our preparation for commercia
and marketing of our product candidates.

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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 a equipment and re-measurement gain or loss associated with the change in the fair value of our Series B purchase rights liability and our pr

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 pricing model, in part, on subjective assumptions, including stock price volatility, risk-free interest rate, dividend yield, and the fair value of the preferre 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 warran 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 prep accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to accru base our estimates on historical experience, known trends and events, and various other factors that are believed to be reasonable under the circumstances, judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimat

While our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this prospectus, we b 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 o research and development grant programs. We recognize revenue when amounts are realized or realizable and earned. Revenue is considered realizable a (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) assured.

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue in our balance sheets. Amounts expected to be rec following the balance sheet date are classified as current liabilities. We recognize revenue for reimbursements of research and development costs under 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 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 p straight-line basis. In situations where the performance of our obligations has been satisfied when the grant is received, revenue is recognized upon rece provisions. We review those refund provisions to determine the likelihood of repayment. If the likelihood of repayment of the g

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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 re
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
information and data provided to us by our vendors and our clinical sites. When outside contracts for research products or testing require advance payme
prepaid item and expensed when the service is provided or reaches a specific milestone outlined in the contra

Share-based compensation

We account for our share-based compensation in accordance with ASC 718, *Compensation Stock Compensation*. ASC 718 establishes accounting for
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 t
requisite service/vesting period. Determining the appropriate fair value model and calculating the fair value of share-based payment awards require the use
expected life of the share-based payment awards and stock price volatility.

We estimate the grant date fair value of stock options and the related compensation expense using the Black-Scholes option valuation model. This option
assumptions including: (1) estimated period of time outstanding, or expected term, of the options granted, (2) volatility, (3) risk-free interest rate and (4)
compensation expense is based on awards ultimately expected to vest, it is reduced for estimated forfeitures. ASC 718 requires forfeitures to be estimated
subsequent periods if actual forfeiture rates differ from those estimates. We have estimated expected forfeitures of stock options based on our historical tu
future forfeiture rate. If our actual forfeiture rate varies from our estimates, additional adjustments to compensation expense may be required in future perio
value of share-based payment awards represent management s best estimates, but the estimates involve inherent uncertainties and the application of mana
we use different assumptions, our share-based compensation expense could be materially different in the future. We will no longer be required to estimate
new equity awards after our initial public offering, now that our shares have begun trading.

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
stock that underlie the stock options we granted prior to our initial public offering in March 2014 were estimated by our board of directors based upon inf
further discussed below.

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Information pertaining to the Black-Scholes valuation of common stock options granted to employees during fiscal years 2012 and 2013 and the nine m follows:

	Fiscal Year Ended June 30,		
	2012	2013	
Options granted (number of shares)	3,934	193,066	19
Weighted-average exercise price	$ 3.50	$ 0.35	$
Weighted-average grant date fair value of common stock options	$ 1.75	$ 0.35	$
Assumptions:			
Expected volatility	65.02%	63.23%	
Expected term in years	6.25	6.25	
Risk-free interest rate	1.39%	1.37% to 1.40%	
Expected dividend yield	0.00%	0.00%	

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 u option expiration data to estimate the expected term assumption for the Black-Scholes grant-date valuation. We have therefore utilized the simplified m Bulletin No. 107, Share-Based Payment, to estimate on a formula basis the expected term of our stock options considered to have plain vanilla charact 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 pe 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 b competition or having been presented by independent parties as a comparable company based upon market sector. In determining a comparable, we l estimated at the time of the grant and revised, if necessary, in subsequent periods if actual forfeitures differ from those estimates. Share-based compens operations for the years ended June 30, 2012 and 2013 and the nine months ended March 31, 2013 and 2014 does not record tax related effects on stock- anticipated operating losses and offsetting changes in its valuation allowance that fully reserves against potential deferre

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 Ju fair value per share utilized to calculate share-based compensation expense for each grant:

Date of grant	Number of shares	Exer price pe
August 25, 2011	3,999	$
November 2, 2011	3,934	
January 6, 2013	192,067	
April 19, 2013	999	
September 18, 2013	371,718	
March 26, 2014	156,770	

Share-based compensation totaled $24,445 and $25,237 for fiscal years 2012 and 2013, and $17,781 and $199,314 for the nine months ended March 31 amount of our share-based compensation expense for stock options granted to employees and non-employees to increase in future periods due to increases value of our common stock.

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The intrinsic value of all vested and unvested options outstanding at March 31, 2014 was $8.9 million, representing the difference between the aggregat assuming a value per share equal to $15.00, which was the closing price of our common stock on The Nasdaq Global Market on that date, and the aggregat a weighted average exercise price equal to $4.79 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 ba of grant. The board of directors considered numerous objective and subjective factors in the assessment of fair value, including reviews of our business industry in which we operate and the markets that we serve and general economic, market and United States and global capital market conditions, the la likelihood of achieving a liquidity event for the shares of common stock underlying these stock options, the preferences and privileges of the preferred s status of the clinical trials and preclinical studies relating to our product candidates and third-party valuations of our common stock. Prior to the comple generally considered the most persuasive evidence of fair value to be the prices at which our securities were sold in actual arms

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 stoc 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 con 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 p February 2010, which our board determined to be not less than the fair value of our common stock. These were our last option grants

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 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 candid 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 th 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

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 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 approac 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. our preferred stock, the valuation firm determined the equity value attributable to our common stock and, after applying a marketability discount of 40% an 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 $3.5 than the fair value of our common stock.

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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 preferenc 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 common stock underlying the stock options in the near term. We also considered developments in the preclinical and clinical trials of our product candidat 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 no pending clinical trial of our most advanced proof-of-concept product candidate for the treatment of AAT deficie

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 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 approac 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 pref After subtracting the liquidation preferences of our preferred stock, the valuation firm determined the equity value attributable to our common stock and, and a control discount of 20%, concluded that the fair value of our common stock as of June 30, 2010 was $3.50 pe

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 th

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 no

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 tr which was our most advanced program. The data were encouraging, in that they provided evidence of safety, dose response and sustained expression. Hov not reach therapeutic levels. As a result, we concluded that additional development and clinical trials would be necessary for this product, and that it wo whom to share the cost of this effort.

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 th

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 no

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 entere $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 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

We believe that the primary factors that influenced this lower valuation were the failure of our AAT deficiency product candidate to achieve serum AAT e trials; the

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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 produc generating encouraging clinical data, addressed a disease with a small population estimated at only 600 patients in the United States and Europe; the fac licensed our wet AMD product candidate to Genzyme were such that we no longer expected to receive substantial revenue from the wet AMD program; an 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 N previously performed annual valuations of our common stock. In conducting its valuation, the valuation firm again determined that the market approach given the then-current stage of our development and the nature of our company. In applying the market approach, the valuation firm estimated our enterpr 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 our preferred stock, the valuation firm determined the equity value attributable to our common stock was zer

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 l (other than an initial public offering that resulted in mandatory conversion to common stock of our outstanding preferred stock), would result in the receip In January 2013, conditions in the United States economy remained uncertain and the investment climate for biotechnology companies in general was not f in the United States, Europe and Canada declining in 2012 compared to 2011. We also considered that our business development efforts had not identifie programs other than the wet AMD program licensed to Genzyme. The initial public offering market in the United States was weak, no gene therapy compa 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 n 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 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 t 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 we with the discount we applied in determining the fair value of our common stock in relation to our previous preferred sto

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 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 fundi Foundation to support our clinical trials of our product candidate addressing AAT deficiency. In March 2013, we also obtained primate data demonstrati 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 as-converted to common stock basis). However, after taking into account the uncertain investment climate for gene therapy companies and what we still c near term exit scenario at a valuation that would result in receipt of consideration by our

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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 previo
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,
value of our common stock on that date. Share-based compensation expense attributable to these awards is accounted for in our statement of operations

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 Septe
third-party valuation by an independent valuation firm other than that which had previously performed valuations of our common stock. This valuation, da
accordance with the framework of the American Institute of Certified Public Accountants Technical Practice Aid, *Valuation of Privately-Held-Company*
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 var
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

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

Scenario
IPO by first calendar quarter of 2014
IPO by second calendar quarter 2014
Remain private through late 2015

In assigning probabilities to the two IPO scenarios and to the remain private scenario, we considered the uncertainties affecting the public securities
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 g
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
these factors made it less likely that we would complete a public offering, particularly if market conditions were unfavorable, and more likely that we wo

We used the market approach, in addition to considering the preliminary valuation indications that we received from various investment banking groups,
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
considered the current stage of development of our various product candidates, analysis of pre-money valuations in recent IPOs by other companies
development, the strength of the current market for initial public offerings in the biotechnology industry and the preliminary valuations provided to us by
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 correspond
price per share of common stock issuable upon the conversion of Series B-2 preferred stock sold in April 2013. Th

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pricing method treats preferred and common stock as call options on the enterprise s value, with exercise prices based on the liquidation and conversion pr common equity per-share values equal to outstanding option and warrant exercise prices. The option pricing method relies on a number of inputs, includin free rate, volatility and expected dividend yield.

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

Assumption
Expected time to liquidity
Risk-free interest rate
Volatility
Expected dividend yield

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 arr 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 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 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 declared a dividend on our common stock and do not expect to do so in the foreseeable future, we utilized an assumed 0.0%

After applying the probability weightings described above, we determined the probability-weighted marketable value of the common stock based on the 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 th estimate the DLOM. Based upon these methods, we considered an appropriate DLOM to be 20%. Taking this into account, we determined the fair value o 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 Ap 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 co A growing body of clinical data providing evidence of efficacy and safety of gene therapy in a variety of diseases, improvements in vector de others and the establishment of regulatory guidelines for the development and approval of gene therapy products had led to increased investm

In November 2012, the first gene therapy treatment to be approved by any regulatory authority in the Western world had been approved by th 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 importa 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 c relevant to us, beginning in the second calendar quarter of 2013 and particularly in the third and fourth calendar quarters of 2013, the

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volume of initial public offerings by biotechnology companies accelerated significantly. Even more importantly, for the first time, these inclu of developing treatments in various disease areas, including one based on gene therapy. As a result of these developments, we believed that i 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 o receive approximately $4.0 million over the next five years. During the summer of 2013, we continued to make progress to complete the desi 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 began to consider conducting an underwritten public offering of our common stock. We began interviewing investment banks, and by early S for a proposed initial public offering. The organizational meeting for the offering contemplated by this prospectus occurred on September 12,

Option awards since September 18, 2013

We did not grant any stock options or other form of equity-based compensation between September 18, 2013 and March 26, 2014, the effective date of t public offering. Since that time, the exercise price per share of all options that we have granted has been set at the closing price of our common stock on T date of grant, which our board of directors believes represents the fair value of our common stock.

Warrant liability

As of June 30, 2012 and 2013 and March 31, 2014, we had warrants outstanding to purchase shares of our Series A-1, Series A-1A and Series B-1 preferr and Series B-1 preferred stock are subject to redemption under circumstances outside of our control, the outstanding shares of these series of preferre 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 fai fair value of the warrants classified as liabilities is estimated using the Black-Scholes option pricing model. The estimates in the Black-Scholes option p assumptions, including stock price volatility, term of the warrants, risk free interest rate, dividend yield, and fair value of the preferred stock underlying 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 recogni net.

Upon the closing of our initial public offering, these warrants were converted into warrants exercisable for commo

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 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 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, 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 $ 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 an such amount was to be determined separately for each tranche by reference to which, if any, of three milestones specified in the agreem

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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 ma decision to complete the second and third tranche was deemed to be outside our control. We therefore recorded, at the time of entry into the Series B Pu 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 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 appli milestones were met, with each scenario being assigned an estimated probability as of the valuation date. The aggregate of these probability-weighted valu 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 millio to the Series B purchase rights reduced the amount allocated to the carrying value of the Series B-1 preferred stock on our

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 under 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 th 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 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 pur 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 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 th 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 a 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 connecti 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 Purch 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 This right was extinguished upon the conversion to common stock of all outstanding shares of our preferred stock upon the closing of

During the nine months ended March 31, 2014, we recorded a change in value of the Series B purchase right liability of $2.8 million to other expense, purchase right immediately prior to the closing of the third tranche was reallocated to the carrying value of the Series B-3

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

Assumption	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%

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Income taxes

We recognize deferred taxes for temporary differences between the basis of assets and liabilities for financial statement and income tax purposes. At Ju
approximately $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
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 loss
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 o
June 30, 2013, computed based on statutory federal and state rates, are completely offset by valuation allowanc

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 car
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 5
limitation may significantly reduce the utilization of our net operating loss carryforwards before they expire. We have not completed a study to assess w
whether there have been multiple ownership changes since our inception, due to the significant costs and complexities associated with such study. Howev
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 ownershi
limit the amount of net operating loss carryforwards that could be utilized annually in the future to offset our taxable inc

For all years through June 30, 2013, we generated research credits but we have not conducted a study to document the qualified activities. When complete
research and development credit carry forwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an u
valuation allowance has been provided against our research and development credits and, if an adjustment is required, this adjustment would be offse
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 March 31,
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 ov
reasonable assurance regarding the reliability of our financial reporting and the preparation of financial statements for external purposes in accordance with
United States. Internal control over financial reporting includes maintaining records that in reasonable detail accurately and fairly reflect our transactions; p
are recorded as necessary for preparation of our financial statements; providing reasonable assurance that receipts and expenditures of our assets are made
and providing reasonable assurance that unauthorized acquisition, use or disposition of our assets that could have a material effect on the financial stateme
basis. Because of its inherent limitations, internal control over financial reporting is not intended to provide absolute assurance that a misstatement of ou
detected. Furthermore, our controls and procedures can be circumvented by the individual acts of some persons, by collusion of two or more people or
misstatements due to error or fraud may occur and not be detected on a timely basis.

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Our management has determined that at June 30, 2013, we had material weaknesses in our internal control over financial reporting which relate to the des reporting processes and our accounting for debt, equity and convertible instruments. We have concluded that these material weaknesses in our internal cor that we do not have the appropriate resources with the appropriate level of experience and technical expertise to oversee our closing and financial reportin financial reporting requirements related to our issuances of convertible notes, preferred stock warrants, stock options, preferred stock and preferred stock p not been remediated at March 31, 2014.

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 continue to seek additional accounting and finance staff members, including a manager of financial reporting, to augment our current staf closing and financial reporting processes; and

we continue to formalize our accounting policies and internal controls documentation and strengthen supervisory reviews by our managemen Notwithstanding the material weaknesses that existed as of June 30, 2012 and 2013, our management has concluded that the consolidated financial state present fairly, in all material respects, our financial position, results of operation and cash flows in conformity with U.S. generally acce

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 misstatemen 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. Ou has not assessed the effectiveness of our internal control over financial reporting and, under the Jumpstart our Business Startups Act of 2012, or the JO attestation report on the effectiveness of our internal control over financial reporting so long as we qualify as an emerging growth company, which may inc 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 acco We have chosen to opt out of this provision and, as a result, we will comply with new or revised accounting standards as required when they are ad 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 (do
Grant revenue	$ 718	$ 4
Sponsored research revenue	364	5
Total revenue	$ 1,082	$ 9

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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
FDA orphan grants relating to our LCA2 and AAT deficiency product candidates. Sponsored research revenue increased by $0.1 million from $0.4 millio
year 2013. The increase was primarily the result of increased activity under our sponsored research arrangement with FFB related to the develop

Research and development expense

	Fiscal Year Ended June 30, 2012	2013
		(dolla
Research and development expense	$ 2,354	$ 3,133

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
ACHM product candidates, including increased facilities costs relating to new laboratory expansion, increased personnel costs relating to new hires and

General and administrative expense

	Fiscal Year Ended June 30, 2012	2013
		(dollar
General and administrative expense	$ 787	$ 1,403

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
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 followi
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 2
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 m
purchase rights and our warrant liabilities that are described in note 11 to our financial statements appearing elsewhere in t

Comparison of the nine months ended March 31, 2013 and 2014

Revenue

	Nine Months Ended March 31, 2013	2014
		(dollars
Grant revenue	$ 326	$ 648
Sponsored research revenue	$ 239	$ 357
Total Revenue	$ 565	$ 1,005

Grant revenue for the nine months ended March 31, 2014 increased by $0.3 million to $0.6 million from $0.3 million for the nine months ended March 31
the inception of

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new grant-funded projects related to our ACHM product candidate, which was partly offset by decreased activity of grant-funded projects relating to our A
Sponsored research revenue increased by $0.1 million from $0.2 million to $0.4 million from the nine months ended March 31, 2013 to the nine month
primarily the result of increased activity under our sponsored research agreement with The Alpha-1 Project related to the development of our AAT deficie
by decreased activity under our sponsored research arrangement with the Foundation Fighting Blindness related to the development of o

Research and development expense

	Nine Months Ended March 31,	
	2013	2014
		(dollars in
Research and development expense	$ 1,900	$ 5,801

Research and development expense increased by $3.9 million from $1.9 million for the nine months ended March 31, 2013 to $5.8 million for the nine mo
the result of increased activity relating to our XLRS and ACHM product candidates, including increased facilities costs relating to new laboratory expans
hires and the acquisition of related laboratory supplies.

General and administrative expense

	Nine Months Ended March 31,	
	2013	2014
		(dollars i
General and administrative expense	$ 972	$ 3,335

General and administrative expense increased by $2.4 million from $1.0 million for the nine months ended March 31, 2013 to $3.3 million for the nine mo
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 $(1.3) million for the nine months ended March 31, 2013 to expense of $(3.3) million for the nine
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 exp
adjustments to our Series B purchase rights and our warrant liabilities that are described in the footnotes to our financial statements for the nine months end
prospectus.

Liquidity and capital resources

We have incurred cumulative losses and negative cash flows from operations since our inception in 1999, and as of March 31, 2014, we had an accumula
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
development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, whic
offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliance

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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 bo
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 th
to be repaid through 24 equal monthly installments of principal and accrued interest. In April 2013, we repaid all outstanding principal and accrued

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 exercis
be exercised at any time until the seventh anniversary of their date of issuance.

As of March 31, 2014, we had cash and cash equivalents and short-term investments of $24.5 million. This amount does not include the net proceeds of
public offering, which closed on April 1, 2014. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily w
Currently, our cash and cash equivalents are held in bank accounts. Our short-term investments consist of certificates of deposits with maturity within

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,	
	2012	**2013**
		(in th
Net cash provided by (used in):		
Operating activities	$ (1,372)	$ (2,777)
Investing activities	(108)	(14,481)
Financing activities	427	25,377
Net (decrease) increase in cash and cash equivalents	$ (1,053)	$ 8,119

Operating activities. For the nine months ended March 31, 2014 and 2013, net cash used in operating activities was $9.0 million and $2.6 million, respect
$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 c

Investing activities. Net cash used in investing activities for the nine months ended March 31, 2014 was $2.7 million and consisted primarily of $24.5 mil
investments, $0.1 million of costs related to the acquisition and maintenance of intellectual property and $0.1 million related to the purchase of property
$22.0 million of proceeds received upon the maturity of short-term investments. Net cash used in investing activities for the nine months ended March 31
million related to the purchase of property and equipment and $0.1 million of costs related to the acquisition and maintenance of o

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 mai
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
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
costs related to the acquisition and maintenance of our intellectual property.

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Financing activities. Net cash provided by financing activities for the nine months ended March 31, 2014 was $10.9 million and consisted primarily of $1 of Series B-3 preferred stock and $0.2 million of proceeds from the exercise of options to purchase shares of our common stock. Net cash provided by f March 31, 2013 was $7.8 million and consisted primarily of $7.5 million of proceeds from the sale of shares of Series B-1 and Series B-2 preferred stock a our bank credit facility, partially offset by $0.2 million in expenses related to the repayment of our bank credit facility and paymen

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 repayment 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 mi million.

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 no product sales unless and until we obtain regulatory approval of and commercialize one of our current or future product candidates. We anticipate that foreseeable future, and we expect the losses to increase as we continue the development of, and seek regulatory approvals for, our product candidates, and We are subject to all of the risks incident in the development of new gene therapy products, and we may encounter unforeseen expenses, difficulties, comp may adversely affect our business. Since the closing of our initial public offering, we have incurred additional costs associated with operating as a publi substantial additional funding in connection with our continuing operations.

We expect that the net proceeds from our initial public offering and this offering together with our existing cash and cash equivalents at March 31, 2014 wi 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 a build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize our lead product candidates, if approved, w

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 capita the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical products, we are unable to estima 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 i AMD;
- our success in scaling our HAVE manufacturing method and expanding our manufacturing capabilities;
- the number and characteristics of product candidates that we pursue;
- the outcome, timing and costs of seeking regulatory approvals;
- subject to receipt of marketing approval, revenue received from commercial sales of our product candidates;
- the terms and timing of any future collaborations, licensing, consulting or other arrangements that we may establish;

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the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecu other intellectual property rights;

the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending ag

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

Contractual obligations and commitments

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

	Total	L tha Y
Operating lease obligations (1)	$ 104	$

(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 development, regulatory and commercial milestones (such as the start of a clinical trial, filing of a Biologics License Application, or BLA, approval by the these obligations on our balance sheet or in the table above because the achievement and timing of these milestones is not fixed and determi

Under each of our various licenses with the University of Florida Research Foundation, or UFRF, covering the AAV construct containing the deficiency using this construct, a small cone cell specific promoter, and the use of engineered capsids and under our joint license with UFRF particular HSV construct and various compositions thereof, we will be required to make payments based upon development, regulatory and c 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 intellectu subject to a minimum floor, for any third-party payments required to be made. We have the right to sublicense our rights under this agreemen of such license income. We are required to make annual maintenance payments under these licenses, which payments are creditable against r

Under our license agreement with the UAB Research Foundation pursuant to which we license a patent covering the use of HSV helpers to p make payments based upon development and regulatory milestones for any products covered by the in-licensed intellectual property. We wil products covered by the in-licensed intellectual property. The royalty is subject to reduction, subject to a minimum floor, for any third-party 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 m license, which payments are creditable against royalty payments on a year-by-year basis.

Under the terms of our license agreement with the Trustees of the University of Pennsylvania, pursuant to which we license intellectual prop RPGR X-linked retinal degeneration, we will be required to make payments ranging from the low-five figures to the mid-six figures based up and commercial milestones for any products covered by the in-licensed intellectual property. Prior to commercialization, we are required to s commercialization expenses a minimum
diligence expenditure ranging from the low- to mid- six figures. We will also be required to pay

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royalties on the net sale of products covered by the in-licensed intellectual property in the low single digits. The royalty is subject to reductio made, with a minimum floor ranging from the low-single digits or less, depending on the amount of annual net sales. The license is sublicens would be required to pay a percentage in the mid-single digits of the sublicense income that we receive. We are required to make annual mai the low four figures to the low five figures. Following our commercialization of a product covered by the in-licensed intellectual property, we royalty payments, which extend into five figures and are creditable against royalty payments on a year-to-year basis.

If any of our product candidates that utilize technology licensed under these agreements reached commercialization, we will be obligated to make royalty p 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 fiscal years 2012 and 2013, we paid annual royalty and license maintenance payments in the aggregate amount of $41,000 and $

Based on the anticipated development timeline for our current product candidates described elsewhere in this prospectus, see Our Business Overview, milestone payments that we will be required to make pursuant to these license agreements during fiscal years 2014, 2015, 2016, and 20

Fiscal Year	Aggrega Pa
2014	$
2015	$
2016	$
2017 and beyond	$

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

(2) Consists of payments to UFRF, the UAB Research Foundation and Johns Hopkins University in connection with the achievement of regulatory mil product candidates.

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

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

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 March 31, 2014, we had cash and cash equivalents $22.9 million and $24.5 million, respectively, primarily held in bank accounts and certificates of deposit. Our primary exposure to market risk is interest r 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 in 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 i 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 orpha proprietary gene therapy platform and our expertise in viral vector selection and design, delivery and manufacturing will facilitate the rapid clinical advan 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. T mutations in single genes, significantly affect visual function and currently lack effective medical treatments. XLRS is characterized by abnormal splittin 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 Ne States Food and Drug Administration, or FDA, in late 2014, and thereafter to initiate Phase 1/2 clinical trials in the United States, with initial clinical data by the absence of cone photoreceptor function, resulting in extremely poor visual acuity, light sensitivity, day blindness and complete loss of color discrim expect to file an IND in early 2015, and thereafter to initiate Phase 1/2 clinical trials in the United States, with clinical data expected in late 2015. We have candidate addressing XLRP, a disease characterized by progressive degeneration of the retina, which can lead to total blindness in adult men. For our XLR late 2016, and thereafter to initiate Phase 1/2 clinical trials in the United States, with clinical data expected in mid

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 func through a variety of delivery methods, and we have obtained preliminary indications of safety and efficacy in clinical trials. These vectors deliver the funct providing safe, sustained expression of the therapeutic protein to treat the disease without modifying the existing DNA o

We have developed extensive internal expertise in viral vector selection and design, delivery and manufacturing that is supported by a broad intellectual manufacturing process is both reproducible and scalable. We have assembled an experienced management team and a world-class group of scientific 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 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 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 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 diseases in ophthalmology where there is significant unmet medica underserved orphan indications that are small enough to allow for clinical trials on a manageable scale but prevalent by orphan disease standards and that using a small, targeted commercial infrastructure. The eye diseases we are targeting are well-understood with highly predictive animal models and clearly will facilitate clinical development and regulatory approval of our product candidates. We believe our initial focus on these orphan eye diseases will provid position us to drive the advancement of gene therapy technology. We plan to leverage our experience in orphan ophthalmology to develop new treatments such as wet AMD. We will also evaluate opportunities to extend the commercial application of our gene therapy platform in other underserved

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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
sufficient to accommodate more than 90% of the individual genes in the human genome. We have developed a proprietary manufacturing process tha
manufactured reliably on a commercial scale. Our gene therapy platform therefore has the potential to provide treatments for many other diseases outside
including those with large dosing requirements or in larger markets. We have already conducted preclinical proof-of-concept studies and Phase 1 and Ph
antitrypsin deficiency, or AAT deficiency, an inherited orphan lung disease. We expect to explore other therapeutic areas selectively, eithe

The chart below summarizes our current gene therapy programs:

Our initial 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 la
orphan diseases can provide us with an attractive business opportunity. We are concentrating initially on several underserved diseases that are prevalent b
allow for clinical trials on a manageable scale and to provide markets that we believe we can serve using a small, targeted comm

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We have focused on orphan ophthalmology because we believe there is a significant unmet medical need in eye diseases. The diseases we are targeting are that, in combination, have enabled us to screen and more accurately predict the potential safety and efficacy of products at an early

Well-understood disease mechanisms. Because sight is the most important sense to humans many people fear blindness more than premature 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 ge 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 also highly predictive animal models in which the disease is caused by the outcomes that are similar to those in humans.

Local delivery of therapeutic agent. Direct delivery to the eye of a therapeutic agent, via methods already widely used in ophthalmology, allo unintended effects.

Short time to clinical data. In XLRS and ACHM, we expect to obtain meaningful clinical data within three to six months after a one-time adr patient, which we believe will facilitate the 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 e are accepted by the United States Food and Drug Administration, or FDA. Other orphan drug companies have spent considerable time and resources work endpoints and develop measurement tools in sometimes ill-defined diseases. In ophthalmology the four accepted endpoints visual acuity, visual fiel well-understood, routinely measured by clinicians, and the FDA consistently applies them and provides guidance on how much improvement is required f defined endpoints will help accelerate the process of clinical study and regulatory approval for our ophthalmic pro

Finally, through our internal research work and in collaboration with partners, we have obtained preliminary safety data in clinical trials with the two m intravitreal and subretinal injection. In clinical trials conducted by our licensee Genzyme, up to 34 patients with wet AMD were treated by intravitreal in 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 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 eye diseases, and to thereby provide a better li some cases there are no currently available treatments. Our strategy to accomplish this goal is:

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

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XLRP. Given the severity of these diseases and the current lack of treatment options, a one-time-treatment alternative that corrects the underl long-term value for patients, their families and the healthcare system more broadly.

Expand our position in ophthalmology.

Continue our leadership position in orphan ophthalmology. We have developed significant experience in the orphan ophthalmolo XLRP and LCA2. We have strong relationships with key opinion leaders in the field and with leading patient advocacy groups. We h from the Foundation Fighting Blindness, or FFB, the National Institutes of Health, or NIH, the National Eye Institute, or NEI, and the comprised of leaders in the fields of ophthalmology and genetics, such as William W. Hauswirth, Ph.D., the Rybaczki-Bullard Profes at the University of Florida College of Medicine who is also one of our scientific founders.

Expand our product offerings to wet AMD. We plan to develop new treatments for wet AMD by leveraging our experience develo our work with Genzyme on a first generation product for wet AMD. Advances have been made in understanding of the disease etiolo has increased since the first anti-VEGF gene therapy programs were designed. We plan to use our resources and access to experts in rapidly move a product candidate into the clinic.

Seek opportunities for strategic partnerships and acquisitions in ophthalmology gene therapy. We believe that with additional partner with newly commercial companies and academic groups. We expect that our breadth of experience in research, manufacturin identify and execute in- licensing, co-development arrangements, intellectual property acquisitions or manufacturing agreements that ophthalmology gene therapy.

Extend our expertise in AAV vector design, delivery and manufacturing. We believe that our understanding of our target indications and design, physical vector delivery, vector manufacturing, clinical trial design and clinical trial conduct are significant competitive advantages. resources to developing the science underlying successful AAV vector design and delivery, as well as to expanding the capabilities of our rep also intend to enhance our discovery capabilities and reduce our reliance on external research at academic organizations by expanding our ba vector design and candidate therapeutic screening.

Expand our manufacturing capabilities and create a pilot manufacturing group. We will seek to decrease our dependence on contract m and staffing a facility capable of process development and non-cGMP manufacturing at a scale of up to 100 liter, or 100 L, batches, for indica facility would enable us to complete process development at a final manufacturing scale appropriate for many indications prior to transfer of better control of our future manufacturing requirements. We believe these investments will facilitate the more rapid advancement of our prod the therapeutic and commercial potential of our gene therapy platform.

Pursue orphan indications with high unmet medical need and greater probability of clinical, regulatory and commercial success. We underlying genetic defect is well-characterized and can be addressed by correcting or inserting a single gene, for which predictive animal mo objective and have been validated by the FDA. We believe that focusing on these types of indications will enable us to obtain data more rapid and regulatory approval of our products.

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Given the relatively low prevalence of orphan diseases and the strong key opinion leader communities and patient advocacy groups around th these markets independently with a small, targeted commercial infrastructure.

Evaluate opportunities to leverage our gene therapy platform to address indications outside of ophthalmology. We intend to develop a our pipeline and the utilization of our gene therapy platform. The adaptability of our platform also presents an opportunity for us to selectivel capabilities and product offerings into a range of genetically defined diseases and potentially to accelerate the development and commercializ One such alliance led to the preclinical development and eventual license to Genzyme of a treatment for wet AMD. We are also continuing cl orphan lung disease AAT deficiency. We continue to evaluate similar opportunities to extend the commercial application of our gene therapy

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 mu generation. Mutated genes can cause production of abnormal proteins, which can cause disease.

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 vira 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 offeri believe that by correcting the underlying genetic defect, gene therapy can provide transformative disease modifying effects potentially with life-long cl 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 efficac improvements in vector design and manufacturing processes by us and others and the establishment of regulatory guidelines for the development and appr have led to increased investment from the biopharmaceutical industry and supported the emergence of gene therapy as an important therapeutic modalit 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 necessa vector using our proprietary production methods, and then deliver the product directly to the appropriate cells in a patient by a suitable physical delivery m simple, the process of developing and manufacturing AAV vectors capable of delivering the genetic material safely into a patient s own cells is highly 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 o 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 d
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 fro

Stability AAV is extremely stable: it is resistant to degradation by shear, solvents and enzymes, facilitating purification and final formulation. AAV
freeze-dried formulation, should this become necessary for larger markets where shipping and distribution of the current frozen formul

Sustained expression Unlike vectors based on other viruses, our AAV vectors are capable of inserting the functional gene into the patient s cells as ar
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
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 ce
the life of the cell, which, in the cell types we are currently focused on treating, may approximate the duration of the pati

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
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 h

AAV vectors, while they provide sustained expression, do not alter the patient s existing DNA, and safety is therefore improved ove
early versions of lentiviral vectors, which insert genes directly into, and thereby alter, the patients DNA, resulted in several well-pu
of leukemia.

AAV has never been linked to human disease, unlike most other viruses used as gene delivery vectors such as adenovirus, herpes vir

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 gen
human clinical trials for LCA2, AAT deficiency and wet AMD, over 100 patients were treated using AAV vectors, with no serious adverse events attribu
deficiency product candidate, patients were treated with doses more than 1,000-fold higher than those planned for use in any of our ophthalmic indicati

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 tha
than 3,000 base pairs in length, we expect to be able to pursue a wide variety of indications with our AAV vect

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

therapeutic gene,

promoter and related gene regulatory elements,

AAV sequences needed to signal replication and packaging, and

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AAV capsid (the protein shell) in which these elements are packaged.

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 th protein requires a promoter, which is a genetic element to drive expression. Certain promoters function well only in certain cell types, whereas other prom 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 phy 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 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 specifi 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 tre the design of the gene construct and the physical administration method. We have spent years conducting research on the best combinations of these el 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 r 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 manufacturing method addresses problems of low productivity and low efficacy that have historically plagued efforts to manufacture AAV vectors and potency, efficiency and safety over processes previously used by us and others. It also enables us to produce a more purified and concentrated end produ 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 p United States and Europe. Our manufacturing process is also reproducible and scalable. It has been transferred successfully to Genzyme and to SAFC Pha where it is used in manufacturing clinical materials pursuant to the FDA s current good manufacturing practices, or GMP

We and SAFC Pharma have successfully produced the necessary material for the clinical trials we have conducted to date, and have more than enough mar our planned

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future trials. We are currently investing in the development of mid- to large-scale manufacturing processes with a view towards supporting our product ca
are developing a pilot manufacturing group to decrease our dependence on contract manufacturers by securing capital equipment and staffing a facility ca
manufacturing at up to 100 L scale.

We hold or have licensed 26 issued and 6 pending patents covering our manufacturing technology. We believe that our core competency and intellectu
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 targ
cells we are targeting for treatment.

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

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
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 t
and other cells in the lateral portions of the eye. This routine procedure can be carried out in an ophthalmologist

Subretinal injection between the photoreceptors in the outer retina and the retinal pigment epithelium just beyond the retina are best for delivering the pro
the lens, where the AAV vector can readily enter photoreceptor cells and retinal pigment epithelium cells. This is a short, outpatient surgical procedure th

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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 intr
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
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

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
oncology and anesthesiology. In preclinical animal studies of our product candidate for AAT deficiency, using a vascular delivery method wa
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. AA
methods to a wide array of other cells, such as heart muscle cells in certain cardiac diseases or directly into the brain in certain

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 candid
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,
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 p
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 visu
effective medical treatments. We are also pursuing early stage preclinical research in treating other orphan eye diseases, s

We initially developed our gene therapy platform and obtained evidence of its safety and efficacy in proof-of-concept programs involving two other eye
licensed our wet AMD technology to Genzyme. Genzyme recently informed us that it no longer intends to use our manufacturing technology to produce
product and will develop the product independently of us. As a result of this decision by Genzyme, we were released from non-competition covenants wi
within the field of ocular neovascularization, and we are currently investigating opportunities to leverage our gene therapy infrastructure and expertise for t
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 pr
clinical trials. We believe our AAT deficiency program provides proof of concept for the use of our gene therapy platform in indications outside o

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 sa
approach in

preclinical studies and clinical trials. These programs enabled us to develop significant experience working with clinical trial design and conduct, clinica
vector design, delivery and manufacturing. They also demonstrate that our manufacturing platform has been successfully vetted by regulatory agencies an
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 retin
early childhood or adolescence. Studies by Dr. Edward Stone published in the *American Journal of Ophthalmology* (2007) indicate the overall prevalence
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 phototransdu
converted to an electrical signal transmitted to the brain. A review paper by den Hollander, published in *Progress in Retinal and Eye Research* (2008), r
responsible for about 6% of all cases of LCA, from which we estimate that there are approximately 600 LCA2 patients in the United St

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.
was demonstrated by behavioral testing and electroretinogram, or ERG, testing, which measures electrical signaling in differen

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 anima
treatment and at three months and one, two and three years after a one-time therapeutic subretinal injection of our LCA2 product candidate. After treatmen
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 tes
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 injectio
consistent with the expected effects of subretinal surgery, were not vector dose-dependent and resolved during the three-

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;

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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 recom
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 cli
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
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 vis
and there has been no evidence of safety issues.

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 produ
sensitivity of cone photoreceptors to light stimulation, from black (minimal sensitivity) to white (moderate sensitivity). Before treatment, both eyes had a
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 su
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 cand
illustrated by the dark spot in the middle of the eye, that was present before treatment disappeared after treatment of th

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

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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 t
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,
planned clinical trials conducted by multiple academic research centers in the United States and several European c

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 fie
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 th
endothelial growth factor, or VEGF. The abnormal blood vessel growth, or neovascularization, causes vision loss due to blood and prote

If left untreated, bleeding, leaking and scarring from these blood vessels eventually cause irreversible damage to the photoreceptors and rapid vision loss
drugs that inhibit VEGF can cause regression of the abnormal blood vessels and improve vision when injected directly into the vitreous humor of the ey
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 retina
engineered version of the receptor to which VEGF binds, and these cells then provide sustained production of the VEGF-inhibit

In preclinical studies, the wet AMD product candidate was evaluated in animal models of retinal neovascular diseases, used for testing products that inhib
primates. After intravitreal injection of the wet AMD product candidate, long-term expression of the engineered sFLT01 protein was demonstrated in bo
model, the wet AMD product candidate resolved the neovascularization, with efficacy results similar to those shown for currently ma

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 rec
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 leak
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)

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

we had a type B pre-IND meeting with the FDA during which meeting the FDA provided guidance on the manufacturing, nonclinical and cli
candidate;

the NIH RAC reviewed draft protocols for the Phase 1 clinical trial and its recommendations were incorporated into the final protocol and inf

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 schedu
evaluations for the last patient in July 2014.

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 w
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
vascular method for delivering our AAT deficiency product candidate to muscle cells, and expect to submit an amendment to our existing IND to allow
2015. For more information about this program, see Proof-of-concept programs beyond ophthalmology; our Alpha-1 antitrypsin d

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, p
primarily from photoreceptor cells and binds strongly and specifically to the surface of photoreceptor and bipolar cells in the retina. Mutated forms of retin
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 b
initial diagnosis. Complications such as retinal hemorrhage or retinal detachment occur in up to 40% of patients, especially in older patients. According to
(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 popul
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

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
macula when viewed by an ophthalmoscope, which is the instrument commonly used by ophthalmologists and optometrists to view the retina. Images obta
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 schoo
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 E
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
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, preferen
of handouts with high contrast. Surgery may be required to address complications of vitreous hemorrhage or full-thickness retinal detachment. Anecdotal
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
interpretation of these reports difficult. In addition, treatment with carbonic anhydrase inhibitors does not address the fundamental genetic defect in persons
inhibitors nor any other medicinal products have been approved by regulatory agencies for treatment of XLR

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 t
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
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 DN
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 photorecep
together and eliminating any splitting between the layers of the cells. Upon light stimulation of the photoreceptor cells, the presence of the retinoschisin a
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
episome persists in the cell, which may be for many years or even life-long, thereby providing long-term potential benefit after a one-tim

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 XL
knocking out, the RS1 gene in mice. These knockout mice have clinical features similar to humans with XLRS, including reduced visual acuity, sch
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 i
knockout mouse treated with an AAV-RS1 vector (right). The knockout mouse retina has no expression of retinoschisin and has splitting and disorganizat
arrowheads in the middle panel of the nuclear staining. After treatment, RS1 staining is present in a normal fashion and the nuclear staining shows restora
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

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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 resp
The figure shows a progressive decrease in the ERG response in the untreated mice but a slower decrease and eventual increase in the ER

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
packaged in several different AAV capsids in monkeys and demonstrated that a vector packaged in an engineered capsid was able to target expression to
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
believe that intravitreal injection of a vector containing the RS1 gene in the same engineered capsid would show expression of retino

Based on AGTC animal study data

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We are currently conducting additional preclinical studies of our XLRS product candidate that are required for submission of an IND to the FDA. These st
mice and nonhuman primates, the design of which is based on specific guidance from the FDA s Office of Cellular, Tissue and Gene Therapy received in
and distribution of the AAV-RS1 vector in animals after the product candidate is delivered by intravitreal injection. Dosing of mice and nonhuman prima
2014 and we expect that data for submission as part of an IND will be available by December 2014.

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 absen
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
safety and efficacy. The study is being conducted at three clinical sites that specialize in inherited retinal diseases: the Casey Eye Institute in Portland, Ore
Dallas, Texas, and the Kellogg Eye Center in Ann Arbor, Michigan.

We plan to submit an IND in late 2014 and initiate a Phase 1/2 clinical trial in early 2015 for our XLRS product candidate in up to 15 patients affected by
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
function over a 12-month period. If successful, we believe the results of this second trial could support submission of a Biologics License Application, o
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
throughout life. Individuals with this condition have no cone photoreceptor function, markedly reduced visual acuity, photophobia, or light sensitivity, an
only functioning photoreceptors are rod photoreceptors, which respond to low intensity light conditions and mediate night vision but cannot achieve fine
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 e
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 m
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
phototransduction, the process whereby a light signal is converted to an electrical signal that is then transmitted to the brain. According to *Retinal Dystrop*
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
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

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 vi
reading. Children with ACHM are provided preferential seating in the front of classrooms to benefit maximally from their ma

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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 ph
contains the CNGB3 gene and a promoter, the PR1.7 promoter, that has been shown in preclinical studies to drive efficient gene expression in prima
photoreceptor function in dog and mouse models of achromatopsia. We have identified an AAV capsid that works well for subretinal delivery and are eval
that work well for intravitreal delivery that could be used in follow-on products.

After our ACHM product candidate containing the functional CNGB3 gene enters a photoreceptor cell, the gene is processed by normal biochemical proc
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
photoreceptor membrane that is required for phototransduction. Restoration of phototransduction enables cone photoreceptors to convert light entering the
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,
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

ACHM occurs in two breeds of dogs, Alaskan malamutes and German shorthaired pointers, due to mutations in the CNGB3 gene that either produce
production of the protein. Both breeds have clinical characteristics similar to human ACHM patients, with day blindness and absence of retinal cone fu
subretinal injection of an AAV vector expressing human CNGB3 restored cone function in dogs with either mutation. Cone-specific ERG responses were
were clearly detected after treatment. Day blindness was demonstrated before treatment by testing the ability of the dogs to navigate a maze under progres
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
treatment, the day blindness was substantially eliminated, and the treated ACHM dogs were able to navigate the maze under bright light conditions

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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 ACHM that were treated with our ACHM product candidate. The figure shows that under low light conductions (0.2 lux, equivalent to the light condition mediated only by rod photoreceptors, all three groups navigated the maze rapidly. As the light intensity was progressively increased (to 646 lux, equivalent vision became mediated by cone photoreceptors, the untreated ACHM dogs took progressively longer to navigate the maze, as they bumped into walls in th contrast, as the light intensity was progressively increased, the time taken to navigate the maze did not change for normal dogs and increased onl

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 treatment there was a dramatic improvement in this important clinical manifestation of ACHM. The restored function persisted for more than 2.

In addition, a mouse model of ACHM was developed by knocking out the CNGB3 gene in mice. These knockout mice have markedly impaired cone pho visual acuity testing. Treatment by subretinal injection of an AAV vector expressing human CNGB3 in the knockout mice improved cone-specific ERG r 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 ar 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 st 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 impo measuring visual function in these patients and will guide us in the design of subsequent clinical trials in which our

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

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
candidate in up to 15 persons affected by ACHM caused by mutations in the CNGB3 gene. We will test the safety and efficacy of the ACHM product ca
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
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 pivot
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
would be additional potential product candidates for treatment of ACHM caused by mutations in these genes, and we believe they would have the potenti
candidate for ACHM caused by CNGB3 mutations were already approved. We have initiated studies with a product candidate for ACHM caused by CN
capsid used in our CNGB3 product candidate, and will evaluate the CNGA3 product candidate for safety and efficacy in mouse and sh

X-linked retinitis pigmentosa

Retinitis pigmentosa is an inherited retinal dystrophy with progressive loss of vision. It is commonly first observed in boys and young men who notice prob
night blindness, followed by a restriction of peripheral visual fields, or tunnel vision, leading to poor central vision and eventu

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 7
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 pub
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 XLR
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 re
expressing RPGR. We have inserted a stable form of the RPGR cDNA into an HSV helper to produce our XLRP product candidate and are currently desig
ability of this product candidate to delay disease progression in animal models of XLRP. If these studies are successful, we will conduct additional preclini
the FDA. These studies will include single-dose toxicology studies in animals that will evaluate the safety and distribution within the animals after our X
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 di
early research

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work. In this way, we anticipate being able to move products rapidly through preclinical studies and into clinical development. We also believe that there a AAV vectors may provide benefit, such as wet AMD.

Other autosomal recessive retinal diseases

As of June 30, 2014, 220 genes causing inherited retinal disease have been identified, of which 146 are autosomal recessive and therefore most amenable most common autosomal recessive forms of retinitis pigmentosa, LCA and cone or cone-rod dystrophy, 38 have gene coding regions of less than 3,76 accommodated within our AAV vectors. We are continuing to evaluate indications having these characteristics to select those most appropriate for addi pipeline.

Wet AMD

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 fie 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 th endothelial growth factor, or VEGF. The abnormal blood vessel growth, or neovascularization, causes vision loss due to blood and protein leakage below t 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 estim AMD in the United States and Europe combined.

Wet AMD is currently treated with intravitreal injections of anti-VEGF agents delivered every one to two months, for an indefinite period. While these VE for many patients, there is an urgent medical need to improve on the approximately 35% success rate for existing therapies by targeting other critical fac frequency for patients and physicians.

Based on our proof-of-concept studies, we believe that gene therapy offers a potential long-term solution to treat wet AMD with one injection. Additionally in oncology, there is a strong rationale for combination therapy to become the standard of care in wet AMD. For instance, we are aware that others are con growth factor, or PDGF, agent in combination with anti-VEGF agents for wet AMD. We believe that, while the predictability of targeting VEGF itself compelling gene therapy approach would offer not only sustained expression but also pathway synergy with existing anti-VEGF options. We have defined with a comprehensive review of possible targets.

The development pathway for wet AMD therapies has been well-established. Preclinical CROs offer highly predictive animal models that reproduce the ne and yield results within a few months. In the clinic, physicians can readily detect therapeutic effects by measuring visual function with an eye chart and a imaging devices. We intend to test several lead targets head-to-head in animal models. If sufficient rationale exists for more than one target, we will inv multiple targets. Given our already-established manufacturing infrastructure and our planned regulatory path, we expect to be able to file an IND for a 24 months.

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) wa types of cone photoreceptors provides the ability to perceive the full range of colors in the visual spectrum.

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Blue cone monochromacy, or BCM, is an inherited retinal disease characterized by lack of functional L and M cone photoreceptors but generally norma
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. The clinic
persons affected by ACHM. BCM is a rare disease; we estimate that there are about 1,500 persons in the United States and about 2,500 p

Color vision deficiency, commonly called color blindness, is the inability or decreased ability to perceive the full spectrum of color differences. The condit
L or M opsin gene, resulting in either a missing or abnormal L or M opsin protein; According to a review article by Dr. Matthew Simunovic published in t
affects a large number of individuals, as many as 8% of men and 0.5% of women,. Individuals with color vision deficiency are not blind; their best-corr

We are currently designing preclinical studies to evaluate the ability of our gene therapy approach to correct the visual abnormalities in animal models of
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 w
color vision deficiency treated if an AAV gene therapy product were available. Results of these studies will help us to determine whether to conduct cl
conditions.

Optogenetics

There are a variety of progressive retinal diseases that ultimately result in advanced retinal degeneration and blindness, including retinitis pigmentosa, AM
developing products to treat these diseases before they progress to blindness, but many patients will have advanced retinal degeneration despite

One approach to treatment of advanced retinal degeneration, in which photoreceptors are no longer functional and able to process new genetic informati
photoreceptors and deliver a light-sensitive protein to neurons in the retina. One such light-sensitive protein is channelrhodopsin 2, or ChR2, a protein tha
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 t
combination of techniques from optics and genetics to control individual neuron activity in living tissue. We are currently evaluating partnerships with
develop an AAV vector for treatment of advanced retinal degeneration.

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

We also plan to pursue gene therapy programs that target muscle cells via direct intramuscular injections or vascular delivery, to leverage the unique prop
our first proof-of-concept programs, we have developed a product candidate for the treatment of AAT deficiency, which is characterized by reduced seru
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 emphys
AAT-deficient patients, accounting for about 72% of cases. According to the National Institutes of Health Genetics Home Reference, the incidence rate fo
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 th
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 Un

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 indi augmentation therapy, consisting of intravenous infusions of AAT protein purified from plasma obtained from healthy human donors, can achieve effecti cost of augmentation therapy, administered by weekly intravenous infusions over the lifetime of the patient, can be more than

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. I candidate was evaluated in single-dose toxicology studies in mice and rabbits. These studies demonstrated that vector administration was not associated w adverse effects on hematology or serum chemistry parameters or gross pathology findings. We plan to perform an additional toxicology study in monk deficiency product candidate to muscle cells by a vascular route of delivery that in animals was able to achieve much higher serum levels compa

We have had extensive dialogue with the FDA, the EMA and other regulatory authorities and advisory bodies concerning the clinical advancement of ou 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 defici
- 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 cli 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 fina
- 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 s
- we received Scientific Advice from the EMA s Committee for Medicinal Products for Human Use, or CHMP, in 2010 related to the manufa 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 deficien 2013.

Our AAT deficiency product candidate has been evaluated in two clinical trials in 18 patients with AAT deficiency. Both trials were designed to evaluat achieve sustained expression of normal AAT protein in the serum. In these trials, there were no serious adverse events attributed to administration of ou bacterial epididymitis and one patient developed diverticulitis, each of which events was considered unrelated to our product candidate. In a Phase 2a tr linearly in direct proportion to the dose and these AAT levels were sustained for more than two years.

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

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 AGT

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 r
However, we have established that in animals, delivering AAV vectors to muscle cells using a vascular method can achieve much higher serum levels than
into muscles. We are currently conducting additional nonclinical studies of this new method for delivering our AAT deficiency product candidate to
biodistribution study for which animals completed dosing in June 2014 and a study comparing direct intramuscular injection with the vascular delivery met
will submit results of these studies in an amendment to our existing IND to allow us to initiate a Phase 2b trial in early 2015 in which our AAT deficiency
six patients with AAT deficiency using the vascular delivery method.

Other non-ophthalmology product opportunities

As we further develop the AAT program, we will investigate the opportunity to expand to other indications where high levels of circula

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
challenges for gene therapy manufacturing relate to the difficulty of developing constructs that provide the necessary helper functions, and in having syste
potency. We have made significant investments in developing improved manufacturing processes, which include the

We have developed proprietary AAV vector manufacturing processes and techniques that produce a more purified and concentrated product
25- to 30-fold reduction in non-infectious viral contaminants as compared to vectors used in many previous clinical trials.

We do not need a specially cloned and isolated cell line for each of our disease targets; we instead use specially engineered replication-incom
which are stable and straightforward to clone.

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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 c
We believe these improvements and our continued investment in our manufacturing platform will enable us to develop best-in-class, next g

Our viral vector production platform for AAV-based gene therapeutics, which we call the herpes-assisted vector expansion, or HAVE method, offers signi
used by others to manufacture AAV vectors, as summarized in the following table.

AAV production method	Straightforward cloning	High efficiency	Hig
Transfection	Yes	No	
Baculovirus	No	No	
Adenovirus	No	Yes	
Our HAVE method	**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
V27. The complementing cell line is required to provide critical functions that allow the replication-incompetent HSV helpers to grow; the sa
all disease targets. This step occurs in disposable culture vessels of increasing size, up to and including disposable stirred tank bioreactors. Th
processed and concentrated to prepare them for use in producing our AAV vectors. These HSV helpers can be stored frozen for years before

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 c
sBHK cell line does not provide the critical functions that would allow for growth of the HSV helpers, which provides an added layer of safe
AAV vectors for all disease targets. This step occurs in disposable culture vessels of increasing size depending on the amount of AAV vector
by using a detergent solution to break open the sBHK cells and release the AAV vectors. This step also destroys any residual HSV helpers th

The third step is to purify the harvested AAV vector using two chromatography columns. The exact method used to column-purify our AAV
used in the product candidate; we have developed purification methods for multiple AAV capsids. We have shown in formal clearance studie
and two chromatography columns can remove up to 10^{14} (100 trillion) units of HSV. This step also helps to eliminate any remaining parts, su
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 vec
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 us
We have already demonstrated our manufacturing knowledge through multiple successful production batches of both HSV helpers and AAV vectors at
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 deficien
purification step to accommodate new AAV capsids, complete removal of animal-derived products from the V27 cell growth step, and formulations that

We are also in the process of acquiring capital equipment and staffing a facility capable of process development and non-cGMP manufacturing at 100 L sc
all process development at final manufacturing scale appropriate for many indications prior to transfer of manufacturing to a cGMP facility, giving us
requirements.

Strategic collaborations and acquisitions

We have formed strategic alliances where both parties contribute expertise to enable the discovery and development of potential gene therapy product can
other resources required to develop and commercialize gene therapy products, we intend to seek other opportunities to form strategic alliances with collab
gene therapy expertise.

As an example we entered into an agreement with SAFC Pharma, which also is our current contract manufacturing organization, for cGMP manufacture o
arrangement allows us to approach other gene therapy companies that might benefit from our manufacturing and vector design capabilities. Under su
manufacturing technology and receive upfront payments, milestones and royalties. SAFC Pharma would do the manufacturing of co

Our plan to bring in-house a pilot manufacturing facility will further support these efforts. Such a facility will allow us to manufacture small amounts of no
companies as they perform their pre-clinical experiments. It will also enable us to develop additional expertise in viral vector design as we look to forge pa
space.

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We also plan to continue to in-license additional intellectual property to support our current programs, to establish new development programs and to supp we will seek to partner with both new commercial gene therapy companies and academic institutions to leverage our expertise in vector design, research, goal of these collaborations would be to forge strategic partnerships around technologies and programs that would fit with our current development pipeli property, development programs in rare diseases, pipeline products where the regulatory pathway is understood, partners with strong scientific, clinical and synergy with our current knowledge base and product pipeline that would add to our industry leadership. We would also be looking at programs where the population that there would be adequate financial returns for the investment of resources.

We will also evaluate opportunities to add products, technology and talent in areas consistent with our strategy through selec

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 pro for planning, budgeting, workload, decision-making, costs and future revenues. The parties had joint ownership of any intellectual property that arose partnership. In collaboration with Genzyme, early product development work, production of materials for animal studies, development of several manu IND-enabling toxicology and biodistribution studies, technology transfer of our HSV-based manufacturing process to Genzyme, production of the AAV ve trial, and drafting of the IND were conducted.

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 o technology and interest in the wet AMD program to Genzyme. The license provides for modest late-stage milestone payments to us and royalties on s development costs from mid-2006 to the date the license was signed. Genzyme is responsible for all further development and commercialization of the non-exclusive rights to jointly developed technology. Genzyme also has options, expiring in 2015 and 2017, to license our manufacturing technology, as i genes associated with diseases outside our current area of focus. Genzyme recently informed us that it no longer intends to use our HSV-based manufact being used for the wet AMD product. Our license agreement with Genzyme was further amended in December 2013 to reflect this fact and, among othe Genzyme for use of our HSV-based manufacturing technology in wet AMD except as to specified pending research activities, and to eliminate restriction 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 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 funded research at multiple labs at UF. Pursuant to four agreements, we have licensed three U.S. patents and multiple pending applications covering invent genetic cloning, gene therapy manufacturing, animal model development and facilities for both small and large animal testing, and in certain instances v important research at UF without having to expand in-house facilities and personnel. We interact frequently with all members of the Powell Gene Therap relationship with the UF Office of Technology Licensing.

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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
founders and Professor and holder of the Rybaczki-Bullard Chair in the Department of Ophthalmology at UF, as principal investigator. As a sub-awardee,
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
the Foundation Fighting Blindness, or FFB, and the Alpha-1 Foundation. Both organizations are well known for their advocacy of patients interests in
providing for reimbursement. Both actively support research into treatment, and we have been awarded three research grants totaling $1.6 million from th
Alpha-1 Foundation. More importantly, both organizations have been instrumental in assisting us in forming ties with disease experts, recruiting patients
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 ophth
conduct sponsored research, act as advisors and collaborate with us on grant proposals. We have been awarded grant funding aggregating $10.6 million b
either independently or with our collaborators. This funding provides peer-reviewed scientific validation of our programs and has facilitated critical e
candidates.

Intellectual property

We strive to protect and enhance the proprietary technology, inventions, and improvements that are commercially important to the development of our
defending patent rights, whether developed internally or licensed from third parties. We also rely on trade secrets relating to our proprietary technolo
technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position in the field of gene therapy that may be
We additionally rely on regulatory protection afforded through orphan drug designations, data exclusivity, market exclusivity, and patent te

Our commercial success may depend in part on our ability to obtain and maintain patent and other proprietary protection for commercially important techn
business; defend and enforce our patents; preserve the confidentiality of our trade secrets; and operate without infringing the valid enforceable patents and
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
these activities. With respect to both licensed and company-owned intellectual property, we cannot be sure that patents will be granted with respect to a
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
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 develop
products. Our proprietary intellectual property, including patent and non-patent intellectual property, is generally directed to, for example, certain genes,
cells, processes to manufacture our AAV-based product candidates and other proprietary technologies and processes related to our

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As of July 10, 2014, our patent portfolio included approximately 48 patents and patent applications that we own and approximately 64 patents and pate
specifically, we own five U.S. patents, four pending U.S. applications, 25 foreign patents and 14 foreign patent applications. We have licensed 22 U.S. pat
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 m
patent portfolio includes patents and patent applications directed to our AAT deficiency, XLRS and ACHM programs, as well as our foundational AA

In addition to the above, we have established expertise and development capabilities focused in the areas of preclinical research and development, manuf
quality control, quality assurance, regulatory affairs and clinical trial design and implementation. We believe that our focus and expertise will help us
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, th
the non-provisional application. In the United States, a patent s term may be lengthened by patent term adjustment, which compensates a patentee for adm
Trademark Office in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed

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. pa
during the FDA regulatory review process. The Hatch-Waxman Act permits a patent term extension of up to five years beyond the expiration of the pate
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 1
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 p
product. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug. When possi
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 pro

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 p
entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and c
maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in
agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise be
competitors. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may
know-how and inventions.

Our patents and patent applications.

Manufacturing. We own or in-license 32 patents and patent applications that cover methods to manufacture AAV vectors. More specifically, we have 6 U.S
patents and patent applications covering manufacturing methods. There are still patents pending in this group. The longest lived and most significant manu

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Small Cone Promoters. We own 10 pending patent applications directed to small cone promoters and uses thereof. Of these 10 patent applications, two ar
patent applications. As these patent applications have been filed recently, no issued patents exist covering small cone promoters. A patent issuing from thi

The following table summarizes our material owned and in-licensed patents and patent applications that are practiced in the manufacture an

Description of Patent or Patent Application	Product C
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
Pseudotypes and Other AAV Compositions (7)	All of our product candidates
CNGB3 Gene (8)	Our ACHM product candidate
Expression Cassettes for Achromatopsia (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
AAV Mediated Gene Therapy for RPGR X-linked Retinal Degeneration (12)	Our XLRP 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
Switzerland, Germany, Spain, France, the United Kingdom, Ireland, Italy, Luxembourg, Monaco, and The Netherlands, and pending patent applica
(2) Includes issued patents, each of which is expected to expire in 2018, in Canada, France, Germany, Israel, Italy, the United Kingdom, and the Unite
(3) Includes pending patent applications in the United States, Australia, Canada and the European Patent Office.
(4) Includes two issued patents in New Zealand and one issued patent in each of Australia and the United States, all of which are expected to expire in
(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 four issued United States patents, three of which are expected to expire in 2019 and one of which is expected to expire in 2021.
(8) 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
expected to expire in 2021: Australia, Canada, France, Germany and the United Kingdom. Also includes two pending patent applications in Japan.
(9) Includes two pending patent applications in the United States and one pending patent application in each of Australia, Canada, China, India, Japan,
(10) Includes issued patents, each of which is expected to expire in 2019, in Belgium, Ireland, Monaco, Greece, Cyprus, Switzerland, Germany, Denma
Zealand, Portugal, Sweden, Netherland, Hong Kong, the United States, Canada, Great Britain, Austria and France.
(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 applica
(12) Includes one pending multijurisdictional patent application filed pursuant to the Patent Cooperation Treaty.

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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 lice
royalty payments, and their provisions regarding diligence, insurance, indemnification and other similar matters, to be reasonable and

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
of which is included in our historical results of operations.

University of Florida. We currently have four license agreements with the University of Florida Research Foundation, or UFRI

A license from UFRF signed in September 2001 relates to the AAV construct containing the AAT gene and the method to treat AAT deficier
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-fi
development, regulatory and commercial milestones for any products covered by the in-licensed intellectual property. Assuming that we meet each of
commercial milestones not more than once for each product, which we expect will be the case, the maximum aggregate milestone payments payable und
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
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 ou
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 fig
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 bre
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
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
upon development, regulatory and commercial milestones for any products covered by the in-licensed intellectual property. Assuming that we meet each
commercial milestones not more than once for each product, which we expect will be the case, the maximum aggregate milestone payments payable und
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
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 ou
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 fig
creditable against royalty payments on a year-by-year basis.

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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, 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 notice to UFRF.

The longest-lived patent covered by this license is expected to expire in 2018.

Two licenses from UFRF, signed in September and November 2012, respectively, relate to the use of engineered AAV capsids. We have an e November 2012 license in the fields of ACHM, XLRS and XLRP and a semi-exclusive license in all other fields of orphan ophthalmology. W use with respect to the patents covered by the September 2012 license. Currently these patents are most useful for ACHM, XLRS and XLRP 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 th regulatory and commercial milestones for any products covered by the in-licensed intellectual property. Assuming that we meet each of the specified devel not more than once for each product, which we expect will be the case, the maximum aggregate milestone payments payable under these licenses with 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 th 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 unde 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 t 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 breaches by us of the terms of the licenses and we may terminate the licenses at any time by submitting written notice

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

University of Alabama at Birmingham. A license agreement from the UAB Research Foundation affiliated with The University of Alabama at Birmingham claims covering the use of HSV helpers to produce AAV vectors. The patent is expected to expire in 2025. Effective in March 2014, we modified the licen then existing licensees.

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 fro based upon development and regulatory milestones for any products covered by the in-licensed intellectual property. Assuming that we meet each of thes 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 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 digi 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 agreeme 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 unde against royalty payments on a year-by-year basis.

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

University of Pennsylvania. In April 2014, we signed an exclusive license agreement with the Trustees of the University of Pennsylvania for intellectual p for X-linked retinal degeneration associated with mutations in the RPGR gene. The patent application was filed in 2013 and upon issue i

Under the terms of the agreement, we made a cash upfront payment to the University of Pennsylvania and will be required to make payments ranging fr based upon development, regulatory and commercial milestones for any products covered by the in-licensed intellectual property. Assuming that we met ea commercial milestones not more than once for each product, the maximum aggregate milestone payments payable under this license with respect to any in $1.3 million. Prior to commercialization, we are required to spend annually on research, development, regulatory and commercialization expenses a minim to mid-six figures. Upon commercialization, we will be required to pay royalties on the net sale of products covered by the in-licensed intellectual propert to reduction for any third-party payments required to be made, with a minimum floor ranging from the low single digits or less, depending on the amount o and should we choose to sublicense we would be required to pay a percentage in the mid-single digits of the sublicense income that we receive. There is ar from the low four figures to the low five figures. There are also minimum royalties post-commercialization which extend into five figures, which paymen year-to-year basis.

This license continues until the expiration of all the patents subject to the licenses or if later, a specified number of years following the first sale of the first property. Additionally, we or the University of Pennsylvania may terminate this license upon certain breaches by the other party of the terms of the license by submitting written notice to the University of Pennsylvania.

Competition

The biotechnology and pharmaceutical industries are characterized by intense and rapidly changing competition to develop new technologies and proprieta successfully develop and commercialize will have to compete with existing therapies and new therapies that may become available in the future. While w and scientific expertise in the gene therapy field provide us with competitive advantages, we face potential competition from many different sources, inclu specialty pharmaceutical and biotechnology companies, as well as from academic institutions and governmental agencies and public and private resear competitive products or technologies. To the extent that we develop product candidates for indications with larger patient populations, such as wet AMD competition from larger and better funded pharmaceutical companies. Any product candidate for wet AMD that we may develop will compete with estab Avastin and Regeneron s Eylea and new drug candidates being developed by others and currently in clinical trials, as well as other treatment mo

Currently there are no approved products for any of our lead orphan ophthalmology indications of XLRS, ACHM and XLRP. We believe the key compet product candidates, if approved, are likely to be their efficacy, safety, convenience of administration and delivery, price, the level of generic competitio government and other third-party payors.

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Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and human resources than we do discovery and development of product candidates, obtaining FDA and other regulatory approvals of treatments and the commercialization of those tr biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. Our c eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, wh 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 Pu federal, state, local and foreign statutes and regulations. Both the FD&C Act and the PHS Act and their corresponding regulations govern, among other thi labeling, packaging, storage, record keeping, distribution, reporting, advertising and other promotional practices involving biological products. Before clini 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 DNA Advisory Committee, or RAC. FDA approval of a BLA also must be obtained before marketing of biological products. The process of obtainin compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources a 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 NIH on gene therapy issues and engages in a public discussion of scientific, safety, ethical and societal issues related to proposed and ongoing gene the published guidance documents with respect to the development and submission of gene therapy protocols. The FDA also has published guidance documen products in general, their preclinical assessment, observing subjects involved in gene therapy studies for delayed adverse events, potency testing, and chem 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 In could result in additional regulations restricting or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign regulating biotechnology. More restrictive regulations or claims that our products are unsafe or pose a hazard could prevent us from commercializing any p established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative ch guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, m

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 produc Office of Cellular, Tissue and Gene Therapies, or OCTGT, within CBER, to consolidate the review of gene therapy and related products, and the Cell Committee, or CTGTAC, to advise CBER on its reviews. In addition, the FDA has issued a growing body of clinical guidelines, chemical, manufacturin guidelines, all of which are intended to facilitate industry s development of gene therapy products.

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In 2012, the EMA approved a gene therapy product called Glybera, which is the first gene therapy product approved by regulatory authoritie

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 invol

completion of nonclinical laboratory tests and animal studies according to good laboratory practices, or GLP, requirements and applicable re 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 additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the intended use;

submission to the FDA of a Biologics License Application, or BLA, for marketing approval that includes substantive evidence of safety, puri testing and clinical trials;

satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biological product candidate is produced to assure that the facilities, methods and controls are adequate to preserve the biological product candidate s identity, strength, quality and puri

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 test nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety an of the preclinical tests must comply with federal regulations and requirements including GLP requirements.

Where a gene therapy trial is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, prior to the submission documentation is submitted to and the trial is registered with the NIH Office of Biotechnology Activities, or OBA, pursuant to the NIH Guidelines for R Nucleic Acid Molecules, or NIH Guidelines. Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for r many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. The NIH is responsible for convening the discusses protocols that raise novel or particularly important scientific, safety or ethical considerations at one of its quarterly public meetings. The OB regarding the necessity for full public review of a gene therapy protocol. RAC proceedings and reports are posted to the OBA web site and

The clinical trial sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical da 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 a 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 out

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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 protoco 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 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 FI recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND w 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 investiga under the trial sponsor s control. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing pro 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 c 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 requirem subjects provide informed consent. Further, each clinical trial must be reviewed and approved by an independent institutional review board, or IRB, at or 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 ind 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 representative and must monitor the clinical trial until completed. Clinical trials also must be reviewed by an institutional biosafety committee, or IBC, a oversees basic and clinical research conducted at that institution. The IBC assesses the safety of the research and identifies any potential risk t

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 f when the product candidate may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is oft

Phase 2. The biological product candidate is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily 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 geographica trials are intended to establish the overall risk/benefit ratio of the product candidate and provide an adequate basis for pro

Post-approval clinical trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These clinical trials are treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up. The FDA recommends that sponsors observe subj 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 per

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinic 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 adverse events, any findings from other trials,

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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 s protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information 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 in 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 including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate ap clinical trial is not being conducted in accordance with the IRB s requirements or if the biological product candidate has been associated with

Human gene therapy products are a new category of therapeutics. Because this is a relatively new and expanding area of novel therapeutic interventions, t 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 g 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 Modif 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. helpful guidance on development of gene therapy products and shown a willingness to work closely with developers, especially with those

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

United States review and approval processes

After the completion of clinical trials of a biological product candidate, FDA approval of a BLA must be obtained before commercial marketing of the include results of product development, laboratory and animal studies, human trials, information on the manufacture and composition of the product can 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 effe the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the pr may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any biological produ designation has been granted. The testing and approval processes require substantial time and effort and there can be no assurance that the FDA will accep 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 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 als ($104,060) and an annual establishment fee ($526,500) on facilities used to manufacture prescription biologics. Fee waivers or red

certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed
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 a
any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BL
information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FD
BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product candidate is safe and potent, or effective, for its intende
whether the product candidate is being manufactured in accordance with GMP regulations to assure and preserve the product candidate s identity, safety,
may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, ty
experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bo
committee, but it considers such recommendations carefully when making decisions. During the biological product approval process, the FDA also wi
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 th
physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools
sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if requ

Before approving a BLA, the FDA will inspect the facilities at which the product candidate is manufactured. The FDA will not approve the product candid
processes and facilities are in compliance with GMP requirements and adequate to assure consistent production of the product candidate within required s
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 G
compliance, an applicant must incur significant expenditure of time, money and effort in the areas of training, record keeping, produc

Notwithstanding the submission of relevant data and information, the FDA may ultimately decide that the BLA does not satisfy its regulatory criteria for a
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 th
complete response letter that usually describes all of the specific deficiencies in the BLA identified by the FDA. The deficiencies identified may be minc
major, for example, requiring additional clinical trials. Additionally, the complete response letter may include recommended actions that the applicant migh
approval. If a complete response letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the

If a product candidate receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use ma
commercial value of the product candidate. Further, the FDA may require that certain contraindications, warnings or precautions be included in the produ
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 F
sometimes referred to as Phase 4 clinical trials, designed to further assess a biological product s safety and effectiveness, and testing and surveillance prog
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
made. The FDA

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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 proc
by three months if the FDA requests or the BLA sponsor otherwise provides additional information or clarification regarding information already provide
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
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 exp
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. Orpha
submitting an NDA or BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disc
designation does not convey any advantage in or shorten the duration of the regulatory review and approval pro

If a product candidate that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designa
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
such as a showing of clinical superiority to the product with orphan exclusivity. Competitors, however, may receive approval of different products for th
exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan product exclusivit
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
the same indication or disease. If a drug or biological product designated as an orphan product receives marketing approval for an indication broader tha
orphan product exclusivity. Orphan drug status in the European Union has similar, but not identical, benefits

Expedited development and review programs

The FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing new drugs and biological products that meet certain
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
or condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. The spon
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
consider for review sections of the marketing application on a rolling basis before the complete application is submitted, if the sponsor provides a sched
application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable, and the sponsor pays any required user f
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 intend
priority review and accelerated approval. Any product candidate is eligible for priority review if it has the potential to provide safe and effective therapy wi
a significant improvement in the treatment, diagnosis or prevention of a disease compared to marketed products. The FDA will attempt to direct additional
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 si
review. Additionally, a product candidate may be eligible for accelerated

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approval. Drug or biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful t
receive accelerated approval, which means that they may be approved on the basis of adequate and well-controlled clinical trials establishing that the pr
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 morbidi
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 m
product candidate receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. In addition, the FDA currently req
pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the p

Lastly, under the provisions of the new Food and Drug Administration Safety and Innovation Act, or FDASIA, enacted in 2012, a sponsor can reque
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 se
preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant
observed early in clinical development. Drugs designated as breakthrough therapies are also eligible for accelerated approval and receive the same benefits
must take certain actions, such as holding timely meetings and providing advice, intended to expedite the development and review of an application

Fast Track designation, priority review, accelerated approval and breakthrough therapy designation do not change the standards for approval but may ex

Post-approval requirements

Maintaining compliance with applicable federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resourc
biological products continues after approval, particularly with respect to GMP requirements. We will rely, and expect to continue to rely, on third parties
quantities of any products that we may commercialize. Manufacturers of our products are required to comply with applicable requirements in the GMP re
assurance and maintenance of records and documentation. Other post-approval requirements applicable to biological products include reporting of GMP d
purity and overall safety of a distributed product, record-keeping requirements, reporting of adverse effects, reporting updated safety and efficacy informa
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,
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 samp
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.
tests on lots of some products, such as viral vaccines, before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laborator
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
populations that are not described in the product s approved labeling (known as off-label use), industry-sponsored scientific and educational activities
Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing
the market as well as possible civil or criminal

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sanctions. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approva
administrative or judicial civil or criminal sanctions and adverse publicity. FDA sanctions could include refusal to approve pending applications, withdra
untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government co
communications with doctors, debarment, restitution, disgorgement of profits, or civil or criminal penalties. Any agency or judicial enforcement action

Biological product manufacturers and other entities involved in the manufacture and distribution of approved biological products are required to register
state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with GMPs and other laws. Acco
time, money, and effort in the area of production and quality control to maintain GMP compliance. Discovery of problems with a product after appro
manufacturer, or holder of an approved BLA, including withdrawal of the product from the market. In addition, changes to the manufacturing process or
before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also s

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 fo
Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendmen
years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot e
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
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
must be submitted prior to the expiration of the patent. The United States Patent and Trademark Office, in consultation with the FDA, reviews and approv
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 beyo
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 exclusivi
exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric stud
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 la
the Biologics Price Competition and Innovation Act of 2009 which created an abbreviated approval pathway for biological products shown to be similar
reference biological product. This amendment to the PHS Act attempts to minimize duplicative testing. Biosimilarity, which requires that there be no cl
biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical
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
times, the biologic and the reference biologic may be switched after one has been

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previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. However, comple
complex, structure of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation

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 re
and proposed to cut this twelve-year period of exclusivity down to seven years. He also proposed to prohibit additional periods of exclusivity for branc
formulations, a practice often referred to as evergreening. The first biologic product submitted under the abbreviated approval pathway that is determine
has exclusivity against other biologics submitting under the abbreviated approval pathway for the lesser of (i) one year after the first commercial marketi
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 subr
been approved if a lawsuit is ongoing within the 42-month period.

Pharmaceutical Coverage, Pricing and Reimbursement

Sales of our products, when and if approved for marketing, will depend, in part, on the extent to which our products will be covered by third-party payors,
health care programs, commercial insurance and managed healthcare organizations. These third-party payors are increasingly reducing reimbursements
addition, the U.S. government, state legislatures and foreign governments have continued implementing cost containment programs, including price contro
and requirements for substitution of generic products. Adoption of price controls and cost containment measures, and adoption of more restrictive polici
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
reduce physician usage of our products once approved and have a material adverse effect on our sales, results of operations and

Other Healthcare Laws

Although we currently do not have any products on the market, we may be subject to additional healthcare regulation and enforcement by the federal go
foreign jurisdictions in which we conduct our business. Such laws include, without limitation, state and federal anti-kickback, fraud and abuse, false claim
laws and regulations, many of which may become more applicable to us if our product candidates are approved and we begin commercialization. If our o
such laws or any other governmental regulations that apply to us, we may be subject to penalties, including, without limitation, civil and criminal penalties,
of our operations, exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability t

Facilities

Our corporate headquarters are located in Alachua, Florida. Our current leased facility encompasses 5,532 square feet of office and laboratory space. Th
December 31, 2014, subject to our option to renew for up to two additional one-year terms. The lease for the office facility expires on December 31, 201
additional three-year term. We are currently reviewing options to re-locate the office space within the same corporate campus as the existing laboratory sp
concurrent building starting in January 2015, and believe that suitable space will be available on commercially reason

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Employees

As of July 10, 2014, we had 18 full-time employees, 14 of whom have Ph.D., M.D. or other post-graduate degrees. Of these full-time employees, ten are e 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 agr our personnel, and is responsible for administering all payroll functions, including tax withholding, and providing health insurance and other benefits for th costs and pay TriNet an administrative fee for its services. We are responsible for, and control, all aspects of the hiring, retention, compensation, manag 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 o administrative burden on us.

We have no collective bargaining agreements with our employees and we have not experienced any work stoppages. We consider our relatio

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 Although the results of litigation and claims cannot be predicted with certainty, any such future litigation could have an adverse impact on us because o management resources and other factors.

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MANAGEMENT

Executive Officers, Directors and Key Employees

Our directors, executive officers, their current positions and their current ages as of July 10, 2014 are set forth be

Name	Age	Position(s)
Susan B. Washer	52	President, chief executive officer and directo
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	40	Director, process development
Scott Koenig, M.D., Ph.D. (1)	61	Chairman of the board of directors
Jill Carroll	39	Director
David R. Guyer, M.D. (2)	54	Director
Ed Hurwitz (1) (2) (3)	50	Director
Ivana Magovcevic-Liebisch, Ph.D.	46	Director
Arnold L. Oronsky, Ph.D. (3)	74	Director
James Rosen (1) (3)	44	Director
Sam Wu, M.D., Ph.D. (2)	47	Director

(1) Member of the compensation committee.
(2) Member of the nominating and corporate governance committee.
(3) Member of the audit 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 20 executive officer, Ms. Washer served as our chief operating officer from October 2001 to March 2002. From August 1996 to October 2001, Ms. Washer Scenic Productions Inc., a specialty construction firm providing sculpting, painting and construction services to the entertainment industry. From June 19 Founding Executive Director and then Business Advisor for the North Florida Technology Innovation Center, a public-private organization financing and p licensing technology from Florida universities. From October 1983 to June 1994, Ms. Washer served in various research and pharmaceutical management p 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 t background in science and business management, her years of experience in the pharmaceutical and biotechnology industries, her service as a senior exe extensive knowledge of our company and its business qualify her to serve as a member of our board of directo

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 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 2 as principal clinical program head of HIV and opportunistic infections clinical development for GlaxoWellcome Inc. from 1994 to 2001, and in various Research Institute of Infectious Diseases, including chief of the virology division from 1992 to 1994, chief of the department of pathogenesis and imm intracellular pathogens from 1991 to 1992 and research investigator in the virology division from 1989 to 1991. Dr. Chulay earned a medical degree from diploma in tropical medicine and hygiene from the London School of Hygiene and Tropical Medicine. Dr. Chulay served his residency at Cleveland Metr Infectious Disease at the Walter Reed Army Institute of Research. He is the author of more than 100 peer-reviewed pu

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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 t biotherapeutics company. From October 2007 to September 2011, Mr. Menichella served as the senior vice president for corporate business development a producer of plasma-derived protein therapies. Prior to joining Talecris, Mr. Menichella served in various corporate business development and allianc pharmaceutical company Merck KGaA, as the president and senior vice president of global corporate development for MorphoSys AG, a biotechnology co vice president, business development and national accounts for Novartis Animal Health, US Inc. Mr. Menichella received a B.A. from Harvard Universit 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 chie Inc., a biotechnology company specializing in the development and commercialization of products to address musculoskeletal conditions. Prior to his ten served as the chief financial officer of Ribozyme Pharmaceuticals Inc. from 1996 to 2003 and La Jolla Pharmaceutical Company from 1990 to 1996. 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 Universi engineering. Since that time, Dr. Knop has served in a number of positions with us relating to our HAVE manufacturing method, including as our associat 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 office biopharmaceutical company, a role which he has held since September 2001. Prior to joining MacroGenics, Dr. Koenig served as senior vice presi biopharmaceuticals company. From 1984 to 1990, he worked in the Laboratory of Immunoregulation at the National Institute of Allergy and Infectious D Health (NIH), where he investigated the immune response to retroviruses and studied the pathogenesis of AIDS. Dr. Koenig currently serves as a membe Organization (BIO), the Children s National Medical Center, the Scientific Management Review Board of the NIH, and the Children s Research Institute 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 Heal Dr. Koenig s education and professional background in science and medicine, his experience as chief executive officer of MacroGenics and as a scien companies and research organizations and his service as a director of other biopharmaceutical companies, medical institutions and industry groups quali 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 corp since September 2011. Prior to her tenure at S.R. One, Limited, she was Senior Director, Corporate Development at Dynavax Technologies Corporation, a August 2010 and the Vice President of Corporate Development at Limerick Biopharma Inc., a pharmaceuticals company, from August 2010 to September 2 the consulting firms Clearview Projects, Inc. from October 2001 to May 2004 and Mercer Management Consulting from March 1999 to July 2001. She University and her M.S. in Biochemistry, Cellular and Molecular Biology from Johns Hopkins University. We believe that Ms. Carroll s educational bac consultant and as an executive and venture capitalist focused on the biotechnology and pharmaceutical industries and her experience with biotechnology an serve as a member of our board of directors.

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David R. Guyer, M.D. has served as a member of our board of directors since June 2014. Dr. Guyer has served as Chairman of the board of directors of Op
Chief Executive Officer of Ophthotech since April 2013. Dr. Guyer, served as a Partner at SV Life Sciences, a venture capital firm, from December 2009
Life Sciences from May 2006 to December 2009. In April 2013, Dr. Guyer resumed his role as Venture Partner at SV Life Sciences Advisers, LLC. Dr. G
and served as Chief Executive Officer and as a member of its board of directors from 2000 to 2006. Prior to co-founding Eyetech Pharmaceuticals, Dr. Gu
the Department of Ophthalmology at New York University School of Medicine. Dr. Guyer received a B.S. from Yale College and an M.D. from Johns Ho
ophthalmology residency at Wilmer Ophthalmological Institute, Johns Hopkins Hospital and a retinal fellowship at the Massachusetts Eye and Ear Infirma
Dr. Guyer s extensive experience in developing and commercializing ophthalmologic therapies qualify him to serve as a member

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
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 fro
to serve as a consultant to that firm. He also serves on the boards of directors of Cara Therapeutics Inc., a biotechnology company and of MacroGenics, In
senior vice president and chief financial officer of Affymetrix, Inc., a manufacturer of DNA microarrays, from 1997 to 2002. From 1994 to 1997, Mr. Hu
the investment bank Robertson Stephens & Company, and from 1992 to 1994, was a biotechnology research analyst for the investment bank Smith Barn
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, respectiv
from Cornell University. We believe that Mr. Hurwitz s education and professional background in science, business management and law, his work as a
the biotechnology industry and his experience as a director of other public and private biotechnology companies qualify him to serve as a m

Ivana Magovcevic-Liebisch has served as a member of our board of directors since June 2014. Dr. Magovcevic-Liebisch has served as Senior Vice Presid
Teva Pharmaceutical Industries Ltd., or Teva, since April 2013. Prior to joining Teva, Dr. Magovcevic-Liebisch held several senior positions within Dyax
2013, most recently serving as Executive Vice President and Chief Operating Officer. Prior to joining Dyax, Dr. Magovcevic-Liebisch was Director of
Transkaryotic Therapies, Inc. from November 1999 until March 2001. Dr. Magovcevic-Liebisch received her J.D. from Suffolk University Law School and
We believe that Dr. Magovcevic-Liebisch s extensive experience in biopharmaceutical business development and operations qualify her to serve

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 Pa
Prior to joining InterWest, Dr. Oronsky was vice president for discovery research at Lederle Laboratories, a division of American Cyanamid Company focu
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 Depart
School. Dr. Oronsky serves as the chairman of the board of directors of Dynavax Technologies Corporation, a biopharmaceutical company, as well as
oncology-focused biopharmaceutical company. Dr. Oronsky also served on the boards of directors of the biopharmaceutical companies, Macrogenics, Inc
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
District of California in January 2010. We believe that Dr. Oronsky s education and professional experience in science a

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experience building and operating research and development operations and his experience in the venture capital industry, particularly with biotech and ph
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, w
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 y
in the health care and biotechnology sectors, including serving as an equity research analyst at Brean Murray & Co., from 2000 to 2003, covering biophar
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-Fla
University of North Carolina School of Public Health. We believe that Mr. Rosen s education and professional background in science, business managem
a scientist and executive in the healthcare and biotechnology industries and as a venture capitalist concentrating on those industries, qualify him to se

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 Ve
AstraZeneca Group, since September 2010. Before joining MedImmune, Dr. Wu held various roles, including principal, at SV Life Sciences Advisers, LL
at SV Life Sciences, Dr. Wu was an engagement manager with McKinsey and Company s Pharmaceuticals and Medical Products practice. Dr. Wu holds
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 educat
and internal medicine and his experience in management consulting and as a venture capitalist concentrating on the biotechnology and pharmaceutical ind
board of directors.

There are no family relationships among any of our directors or executive officers.

Board Composition

Our board of directors currently consists of nine members, of whom seven were elected as directors pursuant to a stockholders agreement that we entered i
stockholders agreement terminated upon the closing of our initial public offering and there are no other contractual obligations regarding the election of ou
successors have been elected and qualified or until the earlier of their resignation or removal. Ms. Carroll and Dr. Wu have notified us that each intends to
of July 31, 2014, in accordance with policies of the respective venture investment firms with which they are affiliated. In addition, Dr. Oronsky has informe
directors no later than the first anniversary of the closing of our initial public offering.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the authorized number of directors may be changed o
amended and restated certificate of incorporation and amended and restated bylaws also provide that our directors may be removed only for cause by the a
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
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, our board of directors is divided into three classes, class I, class II and class III
three-year terms, divided as follows:

the class I directors are Dr. Guyer, Dr. Oronsky and Dr. Wu, and their initial term will expire at the annual meeting of stockholders to be held

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the class II directors are Ms. Carroll, Dr. Koenig and Dr. Magovcevic-Liebisch, and their initial term will expire at the annual meeting of stoc

the class III directors are Ms. Washer, Mr. Hurwitz and Mr. Rosen, and their initial term will expire at the annual meeting of stockholders to
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
expires.

We have no formal policy regarding board diversity. Our priority in selection of board members is identification of members who will further the interes
record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, knowledge of our business a

Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including fami
determined that each of our directors, with the exception of Ms. Washer, is an independent director as defined under Rule 5605(a)(2)

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 stand
administers this oversight function directly through the board of directors as a whole, as well as through various standing committees of our board of direct
areas of oversight. In particular, our board of directors is responsible for monitoring and assessing strategic risk exposure and our audit committee has the
financial risk exposures and the steps our management has taken to monitor and control these exposures, including guidelines and policies to govern
management is undertaken. The audit committee also monitors compliance with legal and regulatory requirements. Our nominating and corporate governar
corporate governance practices, including whether they are successful in preventing illegal or improper liability-creating conduct. Our compensation comm
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 governa
prospectus. Each of these committees, which are the only standing committees of our board of directors, operate under a charter that has been

Audit committee. The current members of our audit committee are Mr. Hurwitz, Dr. Oronsky and Mr. Rosen. Our board of directors has determined that M
the NASDAQ Stock Market independence standards and the independence standards of Rule 10A-3(b)(1) of the Securities Exchange Act. Each of the
requirements for financial literacy under applicable rules and regulations of the SEC and the NASDAQ Stock Market. The board of directors has also det
committee financial expert, as defined by applicable rules of the NASDAQ Stock Market and the SEC. The audit committee assists our bo

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.

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The audit committee has direct responsibility for the appointment, compensation, retention and oversight of the work of our independent registered pu
establishes and implements policies and procedures for the pre-approval of all audit services and all permissible non-audit services provided by our indep
reviews and approves any related party transactions entered into by us.

Compensation committee. The current members of our compensation committee are Mr. Hurwitz, Dr. Koenig and Mr. Rosen, each of whom is an indepe

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. The members of our nominating and corporate governance committee are Dr. Guyer, Mr. Hurwitz a
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

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 servi
one or more executive officers who serve as members of our board of directors or our compensation committee. None of the members of our compensati
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. A copy of the code is poste
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
nature of such amendment or waiver on our website.

Director Compensation

Prior to our initial public offering, we did not have a formal policy regarding compensation of our non-employee directors, other than our chairman. We
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, tha
non-employee directors has historically received any compensation. We do not pay any compensation to our president and chief executive officer in conne

Following the closing of our initial public offering, our non-employee directors other than Dr. Guyer receive equity-based compensatio

each non-employee director receives an annual cash fee in the amount of $35,000;

our chairman receives an additional cash fee in the amount of $27,500;

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the chairperson of each of our board committees receives an additional annual cash fee as follows: audit committee chair, $15,000; compensa
and corporate governance committee chair, $7,000; and

each other member of a board committee receives an additional annual cash fee as follows: audit committee, $7,500; compensation committe
governance committee, $3,500.

The cash fees described above are paid quarterly in arrears. Non-employee directors are also reimbursed upon request for travel and other out-of-pocke
attendance at meetings of the board and of committees on which they serve.

Upon his or her initial election to our board of directors, our non-employee directors other than Dr. Guyer are entitled to receive a non-qualified stock opti
first three anniversaries of the date of grant, to purchase 9,375 shares of our common stock. In addition, each non-employee director other than Dr. Gu
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 annua
equal to the fair value of our common stock on the date of grant.

We have agreed to pay to Dr. Guyer an annual cash fee in the amount of $70,000 for his service on our board of directors in lieu of the cash paym

The following table sets forth information regarding compensation awarded to, earned by or paid to our non-employee directors who served during fiscal
discussion of the compensation of Ms. Washer.

Name	Fees earned or paid in cash (1)	
Scott Koenig, M.D., Ph.D.	$ 31,875	$
Jill Carroll	$ 8,750	$
David R. Guyer, M.D. (3)	$	$
Ed Hurwitz	$ 14,625	$
Ivana Magovcevic-Liebisch, Ph.D. (4)	$	$
Arnold L. Oronsky, Ph.D.	$ 10,625	$
James Rosen	$ 13,125	$
Sam Wu, M.D., Ph.D.	$ 10,500	$

(1) Represents amount earned paid during fiscal year 2014.
(2) Represents the grant date fair value of option awards granted in fiscal year 2014 in accordance with ASC Subtopic 505-50. The assumptions we use
note 5 to notes to financial statements appearing elsewhere in this prospectus.
(3) Dr. Guyer served as a consultant to us from January 20, 2014 until his election as a director on June 27, 2014 for which he earned aggregate fees of
(4) Dr. Magovcevic-Liebisch was elected as a director on June 27, 2014, as a result of which she received in July 2014 a stock option for 9,375 shares

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The table below shows the aggregate numbers of option awards held as of June 30, 2014 by each non-employee director who was

Name	Options Fiscal
Scott Koenig, M.D., Ph.D.	
Jill Carroll	
David R. Guyer, M.D.	
Ed Hurwitz	
Ivana Magovcevic-Liebisch, Ph.D.	
Arnold L. Oronsky, Ph.D.	
James Rosen	
Sam Wu, M.D., Ph.D.	

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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, our Chief Financial Officer and o who served during fiscal year 2014. We refer to these individuals as our named executive officers.

Name	Year	Salary ($)	Option awards ($)(1)	Bonus ($)
Susan B. Washer	2014	313,913	1,601,509	
President and chief executive officer	2013	285,000	23,655	
Lawrence E. Bullock (5)	2014	125,000	908,819	50,000(6)
Chief financial officer				
Jeffrey D. Chulay, M.D.	2014	336,189	366,259	
Vice president and chief medical officer	2013	326,398	6,960	
Daniel Menichella (5)	2014	241,667	603,145	14,000(6)
Vice president and chief business officer				

(1) Represents the grant date fair value of option awards granted in fiscal years 2013 and 2014 in accordance with ASC 718. The assumptions we used granted in fiscal year 2013 are discussed in note 5 to notes to financial statements appearing elsewhere in this prospectus. The assumptions we used granted in fiscal year 2014 are as follows:

Expected volatility	60.
Expected term in years	
Risk-free interest rate	
Expected dividend yield	

(2) Consists of 401(k) matching contributions.

(3) Amounts earned in fiscal year 2014 have not been determined as of the date of this prospectus. The amount earned during fiscal year 2014 will be b goals and other factors deemed relevant by our compensation committee and board of directors. Ms. Washer, Mr. Bullock, Dr. Chulay and Mr. Mer compensation for performance during fiscal year 2014 in an amount equal to up to 30%, 30%, 25% and 35% of their respective base salaries.

(4) Amounts represent cash bonuses earned in fiscal year 2013, and paid during fiscal year 2014, based on achievement of individual performance goal compensation committee and board of directors.

(5) Messrs. Bullock and Menichella were hired by us during fiscal year 2014 and neither was employed by us during fiscal year 2013.

(6) Amount consists of signing bonus paid to the individual in connection with his initial employment by us.

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 in comparable positions in the market, the historical compensation levels of our executives, individual performance as compared to our expectations and obje 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 spe compensation among base salary, bonus or long-term incentives.

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Our board of directors has historically determined our executives compensation. Our compensation committee typically has reviewed and discussed mar executive officer for all executives other than our chief executive officer. Based on those discussions and its discretion, the compensation committee then executive officer. Our board of directors, without members of management present, has discussed the compensation committee s recommendations and executive officers. Effective upon the closing of the closing of our initial public offering, our compensation committee approves the compensation

In preparing to become a public company, we began a thorough review of all elements of our executive compensation program, including the function and fiscal year 2014, our compensation committee engaged Aon Consulting s Radford Surveys + Consulting to assist us with the identification of an appro benchmarking the competitiveness of our executive compensation. Our compensation committee will evaluate the need for revisions to our executive comp competitive with the companies with which we compete for executive talent and that it is appropriate for a public c

Outstanding Equity Awards at Year End

The following table sets forth information regarding outstanding stock options held by our named executive officers as of

			Option Awards	
Name	**Number of Securities Underlying Unexercised Options (#) exercisable**	**Number of Securities Underlying Unexercised Options (#) unexercisable**	**Option Exercise Price ($)**	
Susan B. Washer	5,328		$	3.50
	19,541		$	3.50
	3,877		$	3.50
	42,742(1)	77,944	$	0.35
	26,250(1)	113,750	$	4.90
	4,132(1)	95,024	$	14.08
Lawrence E. Bullock	(2)	100,520	$	12.00
	(2)	2,502	$	14.08
Jeffrey D. Chulay, M.D.	8,028		$	3.50
	2,277		$	3.50
	2,142		$	3.50
	12,576(1)	22,935	$	0.35
	7,614(1)	32,997	$	4.90
	801(1)	18,429	$	14.08
Daniel Menichella	(3)	126,968	$	4.90
	(4)	7,567	$	14.08

(1) This option becomes exercisable in equal monthly installments over four years from the date of grant.

(2) This option becomes exercisable for 25% of the underlying shares on February 3, 2015, and thereafter becomes exercisable in equal monthly instal being exercisable for 100% of the underlying shares on February 3, 2018.

(3) This option becomes exercisable for 25% of the underlying shares on the first anniversary of the grant date, and thereafter becomes exercisable for monthly installments over three years, resulting in the option being exercisable for 100% of the underlying shares on the fourth anniversary of the g

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(4) This option becomes exercisable for 10-5/12% of the underlying shares on September 17, 2014, and thereafter becomes exercisable for the remaini installments over 43 months, resulting in the option being exercisable for 100% of the underlying shares on April 17, 2018.

Employment Agreements, Severance and Change in Control Arrangements

We entered into offer letters with each of Messrs. Bullock and Menichella in connection with their employment by us. Pursuant to the terms of the offer l required to make severance payments to Messrs. Bullock and Menichella following a termination of their respective employment by us. If, at any time foll start of his employment with us, and in the case of Mr. Menichella, prior to August 1, 2018, either Mr. Bullock s or Mr. Menichella s employment by u either Mr. Bullock or Mr. Menichella, as applicable, following a sale of all or substantially all of our stock or assets, whether by merger, acquisition or othe the successor entity with substantially equivalent responsibilities and with total compensation, benefits and severance rights at least equivalent to those h event, which we refer to as a change of control termination, the affected individual will receive:

in the case of Mr. Bullock,

- an amount equal to six-months of his then-current base salary and earned bonus, if the termination occurs prior to the first annive or
- an amount equal to nine-months of his then-current base salary and earned bonus, if the termination occurs on or after the first an employment, or
- an amount equal to twelve months of base salary and bonus, if the termination is a result of a change in control of AGTC and Mr financial officer of the acquiring company; or

in the case of Mr. Menichella, an amount equal to six-months of his then-current base salary.

The following table provides information regarding the estimated amounts payable to the Messrs. Bullock and Menichella upon the occurrence of the tri assuming that the trigger event occurred on June 30, 2014, the last day of our most recently completed fiscal year. The amounts shown as payable upon include amounts earned by the individual and accrued before the occurrence of the triggering event but payable after the triggering event, such as accrued unused vacation days.

Name and Trigger Event
Lawrence Bullock
Termination of employment by the Company without cause
Change of control termination
Termination of employment by Mr. Bullock following a change of control of AGTC in which is he not offered the position of chief financial officer of the acquiring company
Daniel Menichella
Termination of employment by the Company without cause
Change of control termination

(1) Because Mr. Bullock had been employed by us for less than six months on June 30, 2014, under the terms of his offer letter he would not have been connection with a termination of his employment occurring on that date.

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Except as described above, we do not have formal employment agreements with any of our named executive officers and none of our named executive of
connection with the termination of his or her employment. Each of our named executive officers is an employee-at-will of

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,
broadly to our employees, including our named executive officers. The material terms and conditions of our stock option and other equity comp

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) 2
Equity and Incentive Plan and 2013 Employee Stock Purchase Plan are the only effective equity compensation plans pursuant to whi

2001 Stock Option Plan

The 2001 Stock Option Plan, as amended, provided for the grant of incentive and nonqualified stock options. Stock options may no longer be granted ur

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 a
this prospectus forms a part.

General. As of June 30, 2014, the total number of shares of common stock reserved for issuance upon exercise of options outstanding under the 2001 Stoc
be issued under our 2001 Stock Option Plan to any person pursuant to an award are counted against this limit as one share for eve

Purpose. The purpose of our 2001 Stock Option Plan is to promote the company s financial success by creating an additional incentive for key employe
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 appointe

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 c
under the 2001 Stock Option Plan that were terminated, unexercised, or repurchased without having been fully exercised could be granted u

Eligibility. Options under the 2001 Stock Option Plan could be granted to employees (including officers) and directors of the company, any successor co
parent and/or subsidiary corporations of such corporation, or collectively, the Company Group. Options could also be granted to individuals renderin
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 option
amended, or the

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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 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 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 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

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 a 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

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

No option granted pursuant to the 2001 Stock Option plan may be assigned, except by will or by the laws of decent and

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 agreement, any unvested portion of an outstanding option that would otherwise become vested within twelve months following the effective time of a trans 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 su corporation, or parent corporation thereof, may either assume the company s rights and obligations or substitute for outstanding options substantially eq stock. Any options not assumed prior to the transfer of control shall be deemed canceled effective as of the closing of a tra

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 stock options, restricted stock awards, restricted stock units, stock appreciation rights and other share-based awards. No restricted stock awards, restricted share-based awards have been granted under the 2011 Stock Incentive Plan. Following the closing of our initial public offering, awards are no longer g

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 exhib prospectus forms a part.

General. As of June 30, 2014, the total number of shares of common stock reserved for issuance under the 2011 Stock Incentive Plan was 653,960. Any s Incentive Plan to any person pursuant to an award are counted against this limit as one share for every one share g

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 motiva contributions to the company and by providing such persons with equity ownership opportunities and performance-based incentives that are intended to ali

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Administration. Our 2011 Stock Incentive Plan is administered by our board of directors. The board of directors may, to the extent permitted by law, delega 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 committ awards and the terms and conditions of such awards, interpret provisions of our 2011 Stock Incentive Plan and select participan

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 sha stock underlying any awards issued under the 2011 Stock Incentive Plan that are terminated, surrendered, or cancelled without having been fully exercised 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 be added back to the shares of common stock with respect to which awards may be granted under the 2011 Stock Ince

Eligibility. Awards may be granted under the 2011 Stock Incentive Plan to our employees, officers, directors, and individual con

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 impair the rights of participants with respect to outstanding awards without the affected participant s consent to such amendment. In addition, an amen stockholders to the extent required by law. Unless terminated earlier, our 2011 Stock Incentive Plan will terminate in 2021, but will contin

Options. Our 2011 stock incentive plan permits the granting of options to purchase shares of common stock intended to qualify as incentive stock option as incentive stock options, which are referred to as nonstatutory stock options. We may grant nonstatutory stock options to our employees, directors, offic our board of directors. Incentive stock options will only be granted to our employees and employees of other entities which are eligible to receive i

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 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 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 sh

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 op any, after retirement, death, disability or termination of employment during which options may be exercised. Options may be made exercisable in installm 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 t 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 under shares of our common stock, by a cashless exercise through a broker supported by an irrevocable and unconditional undertaking by such broker to deli price, by delivery of shares of common stock having a fair market value equal to the aggregate exercise price of the options

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delivery of a promissory note or such other lawful consideration as approved by the board of directors, or by any combination of th

Except as the board of directors may otherwise expressly determine or provide in an option grant, options granted under our 2011 stock incentive plan ma 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

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 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 governing the restricted stock award, all shares subject to the restricted stock award shall be entitled to vote and shall receive dividends du

Restricted stock units. Restricted stock units entitle the recipient to acquire shares of common stock pursuant to certain terms and conditions. The boar 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 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 com combination of shares and cash, having an aggregate fair market value equal to the product of (a) the excess of the fair market value (as of the exercise dat stock specified in the stock appreciation right by (b) the number of shares of common stock subject to the stock appreciation rights. Stock appreciation righ 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 iss the individual limitations on awards, to reflect any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification capitalization or event, or any dividend or distribution to holders of common stock other than an ordinary cash div

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 ma

provide that the participant s awards will be assumed, or substantially equivalent awards will be substituted, by the acquiring or succeeding 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 immedia 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 whc 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 considerati receive upon consummation of the change of control and (b) the aggregate exercise price of all outstanding options, in exchange for the termi

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provide that, in connection with a liquidation or dissolution of the company, awards shall convert into the right to receive liquidation proceed

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, id

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 outsta
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 instrum
restrictions and conditions on such awards then outstanding shall automatically be deemed terminated and satis

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 s
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 iss
awards, denominated in shares of our common stock, including incentive stock options, nonstatutory stock options, stock appreciation rights, restricted st
stock awards, performance share awards and dividend equivalent rights.

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
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
retaining persons with the requisite experience and ability, and (iii) to more closely align the interests of such employees, officers, directors and other ke
stockholders.

Administration. Our 2013 Equity and Incentive Plan will be administered by the compensation committee of our board of directors. The compensation co
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 a
compensation committee shall be binding on all persons subject to the plan including the company and plan gran

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 share
stock underlying any award issued under the 2013 Equity and Incentive Plan that are forfeited, canceled, satisfied without issuance of stock, otherwise term
any unvested full value award, reacquired by the company shall be added back to the shares of common stock with respect to which awards

Eligibility. Incentive stock options may only be granted to our employees. All other awards may be granted to our employees, officers, directors and key p
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 re
directors may, at any time, amend or discontinue the plan and the compensation committee may, at any time, amend or

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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 o

Options. Our 2013 Equity and Incentive Plan permits the granting of options to purchase common stock that are intended to qualify as incentive stock o qualify as incentive stock options, which are referred to as nonstatutory stock options. We may grant non-qualified stock options to our employees, dire 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 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 comm 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 o

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 law the foregoing, the compensation committee, in its sole discretion, may provide in the award agreement regarding a given option, or may agree in writing optionee may transfer their nonstatutory stock options to members of their immediate family, to trusts for the benefit of such family members, or to partn only partners, provided that the transferee agrees in writing with the company to be bound by all of the terms and conditions of this pla

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 co broker supported by an irrevocable instruction to such broker to deliver sufficient funds to pay the applicable exercise price, by reducing the number of s 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 o 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 rec 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 th (ii) multiplied by the number of shares of stock with respect to which the stock appreciation right shall have been ex

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 term 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 committee, shares of common stock subject to such restrictions and conditions as the compensation committee may determine at the time of grant. Conditio achievement of pre-established performance goals and objectives. A holder of a restricted stock award may exercise voting rights upon (i) execution of a (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 rig determined in the award agreement. The compensation committee shall determine the restrictions and conditions applicable to each restricted stock unit a stock unit shall only have exercisable rights

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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 t 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 restric of unrestricted stock awards on a deferred basis may not be sold, assigned, transferred, pledged or otherwise encumbered, other than by will or

Performance share awards. Pursuant to the 2013 Equity and Incentive Plan, we may grant performance share awards entitling the recipient to acquire sh specified performance goals; provided, however, that the compensation committee, in its discretion, may provide either at the time of grant or at the time o 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 performa 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 credit 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 share installment or installments. A dividend equivalent right granted as a component of another award may provide that such dividend equivalent right shall be s or lapse of restrictions on, such other award, and that such dividend equivalent right shall expire or be forfeited or annulled under the same

Cash awards. The compensation committee, in its discretion, may provide for cash payments to be made under the 2013 Equity and Incentive Plan. Such c conditions and restrictions as the compensation committee considers necessary or advisable.

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 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 t 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 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 va 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 manner determined by the compensation committee to reflect the change in control; (v) cause the award to be assumed, or new rights substituted therefo provision as the compensation committee may consider equitable to the holders of awards and in our best intere

2013 Employee Stock Purchase Plan

Concurrently with our initial public offering, our board of directors established and our stockholders approved our 2013 Employee Stock Purchase Plan, or other employees are allowed to participate in our ESPP. A total of 128,571 shares of our common stock have been reserved for issuance under our ESPP, s below. Our compensation committee has full and exclusive authority to interpret the terms of the ESPP and determine

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Our employees are eligible to participate at the beginning of the first offering period that begins following their commencement of employment with us. H
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

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 st

Our ESPP is intended to qualify under Code Section 423, and provides for consecutive 6-month offering periods. The offering periods generally start on
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 a
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 ea
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 lo
aggregate payroll deductions that have been withheld for the account of the participant during the offering period divided by the purchase price for the s
(c) such other lesser maximum number of shares as the administrator may determine prior to the commencement of the o

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, whicheve
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. Pa
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

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
assume or substitute for the outstanding purchase rights, the offering period then in progress will be shortened, and a new exercise date will be set which w
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 exercis
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
ESPP, except that, subject to certain exceptions described in the ESPP, no such action may adversely affect any outstanding rights to pu

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 unde
our employees are eligible to participate, beginning on the first day of the month following commencement of their employment. The 401(k) plan includes
participants may elect to reduce their current compensation by up to the statutorily prescribed limit and have the amount of the re

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the 401(k) plan. We match participant contributions to the 401(k) plan up to 4% of a participant s annual compensation, subje

Limitation of Liability and Indemnification

Our amended and restated certificate of incorporation includes provisions that limit or eliminate the personal liability of our directors to the maximum ex provides that directors of a corporation will not be personally liable for monetary damages for breaches of their fiduciary duties as dir

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 injun 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

As permitted by Delaware law, our certificate of incorporation also provides 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 de

we will advance expenses to our directors and officers in connection with legal proceedings in connection with a legal proceeding to the fulle

The indemnification provisions contained in our certificate of incorporation are not exclusive.

We believe that these provisions are necessary to attract and retain qualified persons as directors and officers. Insofar as indemnification for liabilities arisi directors, officers or persons controlling our company pursuant to the foregoing provisions, we understand that in the opinion of the SEC such indemnifica 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 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 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 be 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 obt as applicable, in connection with the transactions described below were comparable to terms available or the amounts that would be paid or received,

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 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 preferr 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, sh 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 principal amount of the note purchased by the applicable purchaser, divided by (b) the applicable exercise price of th

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 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 th executive officers and holders of more than 5% of our voting securities, and their affiliates or immediate family me

Purchaser	Principal amount of convertible promissory notes
Intersouth Partners VI, L.P.	$ 202,480
Entities affiliated with InterWest Partners	$ 270,010
MedImmune Ventures, Inc.	$ 270,010

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 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 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 sh aggregate of $10.7 million. The Series B holders exercised this right and we completed the sale of these Series B-3 shares on

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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 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
Alta Partners VIII, L.P.	26,473,934	$ 3,429,000	49,060,606	$ 7,285,500
S.R. One, Limited	17,625,289	$ 2,286,000	32,707,070	$ 4,857,000
Entities affiliated with InterWest Partners	6,409,436	$ 831,304(1)	11,893,926	$ 1,766,248
Intersouth Partners VI, L.P.	4,806,416	$ 623,392(2)	8,919,218	$ 1,324,504
MedImmune Ventures, Inc.	6,409,436	$ 831,304(1)	11,893,926	$ 1,766,248
Osage University Partners I, L.P.	4,406,322	$ 571,500	8,176,767	$ 1,214,250

(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.

Upon the closing of our initial public offering in April 2014, each share of Series B-1, B-2 and B-3 preferred stock was automatically converted

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 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 request that their shares be covered by a registration statement that we may otherwise file. See Description of Capital Stock Registration

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 a 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 fi of securities by certain holders of our capital stock. The amended and restated voting agreement contains provisions with respect to the election of our amended and restated right of first refusal and co-sale agreement and the amended and restated voting agreement will each terminate upo

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 righ the amended and restated investor rights agreement to require inclusion of our securities held by them in the registration statement for

As of July 10, 2014, holders of the requisite number of shares of our common stock, on behalf of all of the parties to the amended and restated investor righ the amended and restated investor rights agreement to require inclusion of our securities held by them in the registration statem

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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 g fund affiliated with Alta Partners, and continues to serve as a consultant to that firm and as a board representative on its portfolio companies.

Indemnification of Officers and Directors

Our certificate of incorporation provides that we will indemnify our directors and officers to the fullest extent permitted by Delaware law. In addition, we with each of our directors that are broader in scope than the specific indemnification provisions contained in the Delaware General Corporation Law. S 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 stockh directors to review all interested party transactions and not to authorize any such transaction unless the board of directors, excluding any interested direct transaction are as favorable or more favorable to our company than would be available from an unrelated party in an arms length negotiation. Pursuant committee that we expect to become effective upon the closing of this offering, our audit committee will be responsible for reviewing and approving in a 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 (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 th 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, whet less favorable than terms generally available to an unaffiliated third-person under the same or similar circumstances and the extent of the relate

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PRINCIPAL STOCKHOLDERS

The following table sets forth certain information with respect to beneficial ownership of our common stock, as of June

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 p common stock subject to options or warrants held by that person that are currently exercisable or exercisable within 60 days of June 30, 2014 are deemed o 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 com 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 o 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 14,082 of June 30, 2014. Each stockholder s percentage ownership after the offering assumes the issuance of the 2,000,000 shares of our common stock off underwriters over-allotment option. Except as otherwise set forth under the heading Right to Acquire, the table below assumes no exercise of stock o purchase an aggregate of 1,093,559 shares of our common stock. Amounts under the heading Right to Acquire represent shares that may be acquired warrants exercisable within 60 days of June 30, 2014.

Name of Beneficial Owner	Shares Outstanding	Right to Acquire	
Alta Partners VIII, L.P. (1)	2,948,400		2
S.R. One, Limited (2)	1,989,598		1
Entities affiliated with InterWest Partners (3)	1,452,216	11,895	1
MedImmune Ventures, Inc. (4)	1,452,196	11,896	1
Intersouth Partners VI, L.P. (5)	1,205,537	8,920	1
Ridgeback Capital Investments L.P. (6)	1,082,240		1
Susan B. Washer (7)	15,000	116,863	
Lawrence E. Bullock (8)			
Jeffrey D. Chulay, M.D. (9)		37,412	
Daniel Menichella (10)			
Scott Koenig, M.D., Ph.D. (11)	2,228	27,373	
Jill Carroll (2) (12)			
David Guyer, M.D.			
Edward Hurwitz (12)			

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Name of Beneficial Owner	Shares Outstanding	Right to Acquire	
Ivana Magovcevic-Liebisch, Ph.D.			
Arnold L. Oronsky, Ph.D. (3) (12)			
James Rosen (5) (12)			
Samuel Wu, M.D., Ph.D. (4) (12)			
All current executive officers and directors (13 persons) (13)	17,628	194,372	2

* Less than 1.0%

(1) The address of Alta Partners VIII, L.P. is One Embarcadero Center, 37th Floor, San Francisco, California 94111. Alta Partners Management VIII, L
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,
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.
Hurwitz, a member of our board of directors, was a director of Alta Partners Management VIII, LLC.

(2) The address of S.R. One, Limited is 161 Washington Street, Suite 500, Conshohocken, Pennsylvania 19428. Jill Carroll, a member of our board of
Limited.

(3) Includes 11,895 shares of common stock issuable exercise of stock purchase warrants exercisable within 60 days of the date of this table. InterWest
L.P., and InterWest Investors Q VIII, L.P. are collectively referred to as the entities affiliated with InterWest Partners. InterWest Management Partn
entities affiliated with InterWest Partners and has sole voting and investment control over the shares held by the entities affiliated with InterWest P
Scott Hedrick, W. Stephen Holmes, Gilbert H. Kliman and Arnold L. Oronsky, a member of our board of directors, are the managing directors of I
Each of the managing directors share voting and investment control with respect to the shares held by the entities affiliated with InterWest Partners
Partners, 2710 Sand Hill Road, Second Floor, Menlo Park, California 94025.

(4) Includes 11,896 shares of common stock issuable exercise of stock purchase warrants exercisable within 60 days of the date of this table. The addre
MedImmune Way, Gaithersburg, Maryland 20878. Sam Wu, a member of our board of directors, is a managing director of MedImmune Ventures,

(5) Includes 8,920 shares of common stock issuable exercise of stock purchase warrants exercisable within 60 days of the date of this table. The addres
Hall Plaza, Suite 200, Durham, North Carolina 27701. Mitchell Mumma and Dennis Dougherty are the managing members of Intersouth Associate
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
James Rosen, a member of our board of directors, is a Partner at Intersouth Associates VI, LLC.

(6) This information is based on information contained in a Schedule 13G filed with the SEC on April 10, 2014 by Ridgeback Capital Investments L.P
Ridgeback Capital Management LP, which reported that they shared voting and dispositive power with respect 1,082,240 shares of our common sto
reporting persons is 75 Ninth Avenue, 5th Floor, New York, NY 10011.

(7) Excludes 271,725 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(8) Excludes 103,022 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

(9) Excludes 70,388 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.

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(10) Excludes 134,535 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.
(11) Excludes 39,732 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table.
(12) Excludes 9,375 shares subject to outstanding stock options that are not exercisable within 60 days of the date of the table
(13) Excludes 688,097 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 sto stock, $0.001 par value per share, and 5,000,000 shares of preferred stock, $0.001 par value per share. As of June 30, 2014, we had 14,082,091 shares shares of preferred stock issued and outstanding, 1,043,748 shares of common stock potentially issuable pursuant to outstanding stock options, and 49,81 pursuant to outstanding warrants. As of June 30, 2014, there were 52 holders of record of our common stoc

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 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 deci having a majority in voting power of the votes cast by the stockholders present or represented and voting on such matter. Our common stock do

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 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 common stock will be entitled to receive pro rata all of our remaining assets available for distribution to our stockh

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 sink provide for the granting of preemptive rights to any stockholder. All outstanding shares are fully paid and nonasse

Preferred Stock

Our board of directors is authorized, without stockholder approval, from time to time to issue up to 5,000,000 shares of preferred stock in one or more se preferences, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, as the board of directors may d 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 pref in connection with possible acquisitions and other corporate purposes, could have the effect of making it more difficult for others to acquire, or of disco majority of our outstanding voting stock. We have no current plans to issue any shares of preferred stock.

Options

As of June 30, 2014, options to purchase 1,043,748 shares of our common stock were outstanding under our equity compensation plans, at a weighted

Warrants

As of June 30, 2014, we had outstanding warrants to purchase 49,811 shares of our common stock at a weighted average exercise p

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Registration Rights

Under the terms of an investor rights agreement between us and certain of our investors, the holders of approximately 9.2 million shares of common stock
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 inclu
we file.

Demand registration rights. The holders who in the aggregate hold more than 50% of the shares having registration rights have the right to demand that w
These registration rights are subject to specified conditions and limitations, including the right of the underwriters to limit the number of shares inclu
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
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
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
any underwritten offering will have the right to limit the number of shares included in such offering for the account of stockholders

Expenses of registration. We will pay all expenses, other than underwriting discounts and commissions, relating to all demand registrations, Form S-3

Expiration of registration rights. The registration rights described above will terminate upon the earlier of April 1, 2019 and, as to a given holder of registr
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 affil
90-day period 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 m
were to initiate a registration and include registrable securities because of the exercise of registration rights, the inclusion of registrable securities could

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 prox
incumbent directors and otherwise. These provisions, summarized below, are expected to discourage types of coercive takeover practices and inadequate t
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 propon
acquire or restructure us outweigh the disadvantages of discouraging takeover or acquisition proposals because negotiation of these proposals could

We must comply with Section 203 of the Delaware General Corporation Law, an anti-takeover law. In general, Section 203 prohibits a publicly held Dela
combination with an interested stockholder for a period of three years following the date the person became an interested stockholder, unless the bu
person became an interested

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stockholder is approved in a prescribed manner. Generally, a business combination includes a merger, asset or stock sale, or other transaction resulting An interested stockholder includes a person who, together with affiliates and associates, owns, or did own within three years before the determination o corporation s voting stock. The existence of this provision generally will have an anti-takeover effect for transactions not approved in advance by the boa that might result in a premium over the market price for the shares of common stock held by stockholders.

Our certificate of incorporation and by-laws require that any action required or permitted to be taken by our stockholders must be effected at a duly called and may not be effected by a consent in writing. In addition, special meetings of our stockholders may be called only by the board of directors and some of notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of candidates for elec incorporation and by-laws also provide for our board of directors to be divided into three classes, with each class serving staggered three-year terms. The hostile takeovers or delaying changes in our control or management.

Listing on the NASDAQ Global Market

Our common stock is listed on the NASDAQ Global Market under the symbol AGTC.

Authorized but Unissued Shares

The authorized but unissued shares of common stock and preferred stock are available for future issuance without stockholder approval, subject to any l Rules. These additional shares may be used for a variety of corporate finance transactions, acquisitions and employee benefit plans. The existence of auth stock and preferred stock could make it more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tend

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

Future sales of significant amounts of our common stock, including shares issued upon exercise of outstanding options or warrants or in the public market 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 NASDAQ Global Market under the symbol AGTC.

Upon the closing of this offering, and after giving effect to the issuance of the 2,000,000 shares of our common stock offered in this offering, we will have of common stock, assuming no exercise of outstanding options or warrants after June 30, 2014. Of these shares, the 2,000,000 shares sold by us (assumi option) in this offering, and the 4,791,667 shares sold by us in our initial public offering, will be freely tradable without restriction or further registration u agreements described below and except in each case for any shares purchased by our affiliates, as that term is defined in Rule 144 under the Securities resale restrictions described below, other than the holding period requirement.

The remaining shares of common stock are restricted securities, as that term is defined in Rule 144 under the Securities Act and are subject to either res described below or restrictions on transfer for a period through September 22, 2014 under stock option agreements entered into between us and the holde 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 Securities Act, which are summarized below.

In addition, of the 1,043,748 shares of common stock that were issuable pursuant to stock options outstanding under our equity incentive plans, options to vested and were exercisable as of June 30, 2014. Upon exercise, these shares will be eligible for sale, subject to the lock-up agreements and securities law common stock that were issuable pursuant to warrants outstanding as of June 30, 2014, were exercisable as of June 30, 2014 and upon issuance these share agreements and securities laws described below.

Rule 144

Affiliate Resales of Restricted Securities

In general, under Rule 144 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 ow 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

1% of the number of shares of our common stock then outstanding, which will equal approximately 160,000 shares immediately after this off

the average weekly trading volume in our common stock on the NASDAQ Global Market during the four calendar weeks preceding the filing 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 sol three-month period exceeds 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 t either the placing of a sale order with the broker or the execution directly with a market maker.

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Non-Affiliate Resales of Restricted Securities

In general, under Rule 144 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 pre 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 info shares for at least one year, such person can resell under Rule 144(b)(1) without regard to any Rule 144 restrictions, including the current p

Non-affiliate resales are not subject to the manner of sale, volume limitation or notice filing provisions of Rule

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 compe agreement entered into before the effective date of our initial public offering is entitled to sell such shares without further restriction

Lock-up Agreements

Our executive officers and directors and the holders of substantially all of our outstanding stock agreed with the underwriters for our initial public offering or hedge any of their common stock or securities convertible into or exchangeable for shares of common stock for a period through September 22, 2014 representatives of the underwriters.

In addition, our executive officers and directors and certain holders of our outstanding common stock, who, together with our executive officers and direc million shares of our outstanding common stock, have agreed with the underwriters, subject to certain exceptions, not to dispose of or hedge any of their exchangeable for shares of common stock for a period through the date 90 days after the date of this prospectus, except with the prior written consent

The representatives of the underwriters currently do not anticipate shortening or waiving any of the lock-up agreements and do not have any pre-establishe 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 o

Registration Rights

Subject to the lock-up agreements described above, the holders of approximately 9.2 million shares of common stock and warrants to purchase up to appro the right to require us to register these shares under the Securities Act under specified circumstances. After registration pursuant to these rights, these share under the Securities Act. See Description of Capital Stock Registration Rights for additional information regarding thes

Stock Options and Warrants

As of June 30, 2014, we had outstanding options to purchase 1,043,748 shares of common stock, of which options to purchase 199,610 shares of commor this offering, we

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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 the 2013 Equity and Incentive Plan and the 2013 Employee Stock Purchase Plan.

As of June 30, 2014, we also had outstanding and exercisable warrants to purchase 49,811 shares of common stock. Any shares purchased by our non-affi of our warrants will be freely tradable under Rule 144(b)(1), subject in certain cases to the lock-up periods described above. Any shares purchased through 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 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 c with respect to the U.S. federal, state, local and non-U.S. tax consequences of the purchase, ownership and disposition of ou

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 prop 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 di 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 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. 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 circu 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 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 t acquiring, holding and disposing of our common stock through a partnership or other pass-through entity, as appl

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 a 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 challe

NON-U.S. HOLDERS ARE URGED TO CONSULT THEIR TAX ADVISORS WITH RESPECT TO THE APPLICATION OF THE U.S. FEDERA OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK IN LIGHT OF THEIR PARTICULAR SITUATION

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AS ANY TAX CONSEQUENCES ARISING UNDER THE LAWS OF ANY STATE, LOCAL, FOREIGN OR OTHER TAXING JURISDICTION OR

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 individu 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 Stat 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 author 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 fo distributions of cash or property on our common stock, those payments generally will constitute dividends for U.S. federal income tax purposes to the e earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, 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 t 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. 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 properly executed IRS Form W-8BEN (or other appropriate version of IRS Form W-8 or successor form) and satisfy applicable certification and other requ 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 applica 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% applicable certification and disclosure requirements by providing a properly executed IRS Form W-8ECI (or successor form). However, such U.S. effe 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 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 spe

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 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 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 pro 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 ta 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 3 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 dispos 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 th 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) 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 ex 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 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 proper 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 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 endin 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 o 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, w 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 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 Cor 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 foreign, unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally 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. 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 m U.S. office of a broker. Non-U.S. holders should consult their tax advisors regarding the application of the information reporting and bac

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 unde 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 refund
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
sale or other disposition of, our common stock paid to certain foreign entities (including foreign financial institutions and foreign intermediaries), unle
registration, certification information reporting and due diligence requirements (generally relating to ownership by U.S. persons of interests in or a

The FATCA withholding tax rules generally will be applicable to dividends on our common stock that are paid after December 31, 2014, and to gross pr
common stock that occurs after December 31, 2016. Prospective investors should consult their tax advisors regarding the possible implications of FATC

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UNDERWRITING

BMO Capital Markets Corp., Stifel, Nicolaus & Company, Incorporated and Wedbush Securities Inc. are acting as representatives of the underwriters and
the terms of an underwriting agreement, which will be filed as an exhibit to the registration statement, each of the underwriters named below has severa
number of shares of common stock shown opposite its name below:

Underwriters
BMO Capital Markets Corp.
Stifel, Nicolaus & Company, Incorporated
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 conditi
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 n
option to purchase additional shares. The underwriting fee is the difference between the initial price to the public and the amount the under

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 t
dealers, which may include the underwriters, at such offering price less a selling concession not in excess of $0.54 per share. After the offering, the represe
selling terms.

The expenses of the offering that are payable by us are estimated to be approximately $440,000 (excluding underwriting discounts and commissions). We
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, u
the public offering price less underwriting discounts and commissions. This option may be exercised to the extent the underwriters sell more than 2,000,00
extent that this option is exercised, each underwriter will be obligated, subject to certain conditions, to purchase its pro rata portion of these additional s
underwriting commitment in the offering as indicated in the table at the beginning of this Underwriting sectio

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Lock-Up Agreements

We, all of our directors and executive officers, and certain holders of shares of our outstanding stock, who, together with our directors and executive offi million shares of our common stock, have agreed that, for a period of 90 days, or the lock-up period, after the date of this prospectus subject to certain lim 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 (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 l 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 securities convertible into or exercisable or exchangeable for common stock, (2) enter into any swap or other derivatives transaction that transfers to ano 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 o 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 th securities convertible into or exercisable or exchangeable for common stock or any of our other securities, or (4) publicly disclose the inten

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 dat other dispositions made exclusively by the holder to the holder s family, partners, members, stockholders or affiliates (as applicable), and transfers or 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 an 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 exercise of warrants or stock options granted pursuant to the Company s stock option/incentive plans or otherwise, or the conversion of securities, i prospectus, *provided* that the restrictions shall apply to the shares of common stock issued upon such exercise or conversion; (4) the establishment of any tr 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 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 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 t 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 release common stock and other securities from lock-up agreements, BMO Capital Markets Corp. will consider, among other factors, the holder s reasons 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. h without the prior written consent of Stifel Nicolaus & Company, Incorporated and Wedbush Securities Inc. BMO Capital Markets Corp., Stifel Nicola 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 imposed in connection with our ini director of the Company, BMO Capital Markets Corp., Stifel Nicolaus & Company, Incorporated and Wedbush Securities Inc. will notify us of the impen announce the impending release or waiver by press release through a major news service at least two business days before the effective

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Indemnification

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act, and to contribute to payments that 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 purc 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 position. This short position may be either a covered short position or a naked short position. In a covered short position, the number of share 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 additional shares. In a naked short position, the number of shares involved is greater than the number of shares in their option to purchase add any short position by either exercising their option to purchase additional shares and/or purchasing shares in the open market. In determining position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the through their option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that th 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

Penalty bids permit the representatives to reclaim a selling concession from a syndicate member when the common stock originally sold by th 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 comm 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 mar NASDAQ Global Market or otherwise and, if commenced, may be discontinued at any time.

Neither we nor any of the underwriters make any representation or prediction as to the direction or magnitude of any effect that the transactions describe stock. In addition, neither we nor any of the underwriters make any representation that the representatives will engage in these stabilizing transactions or th discontinued without notice.

Listing on The NASDAQ Global Market

Our common stock is listed 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 practice offering price listed on the cover page of this prospectus.

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Other Relationships

The underwriters and certain of their affiliates are full service financial institutions engaged in various activities, which may include securities trading, c advisory, investment management, investment research, principal investment, hedging, financing and brokerage activities. The underwriters and certai performed, and may in the future perform, various commercial and investment banking and financial advisory services for the issuer and its affiliates, for v 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 a related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers, and such inv 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 under certain other of those underwriters or their affiliates may hedge, their credit exposure to us consistent with their customary risk management policies. Typi 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 secu potentially the shares of common stock offered hereby. Any such credit default swaps or short positions could adversely affect future trading prices of th underwriters and certain of their affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish o of such securities or instruments and may at any time hold, or recommend to clients that they acquire, long and/or short positions in suc

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 of 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 actio 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 jurisdiction (other than the United States) where any such action for that purpose is required. Accordingly, each underwriter has undertaken that it will not, common stock or have in its possession, distribute or publish any prospectus, form of application, advertisement or other document or information i 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 sh same terms.

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 off 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 time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant M

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 D than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior co for any such offer; or

in any other circumstances falling within Article 3(2) of the Prospectus Directive,

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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 Pr
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 contemplate
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)
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 Mer
term is defined in the Prospectus Directive, or in the circumstances in which the prior consent of the representatives of the underwriters has b
common stock have been acquired by it on behalf of persons in any Relevant Member State other than qualified investors, the offer of such c
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 s
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 in
common stock, as the same may be varied in that Relevant Member State by any measure implementing the Prospectus Directive in that Relevant Memb
means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member S
measure in each Relevant Member State and the expression 2010 PD Amending Directive means Directive 201

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 in
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
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 relati
involving the United Kingdom.

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
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
not claim to comply with the disclosure standards of the listing rules of SIX Swiss Exchange and corresponding prospectus schemes annexed to the listing r
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 inves
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 othe
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 co
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
be copied and/or distributed to the public in (or from) Switzerland.

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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 m
Laws of Hong Kong), or (ii) to professional investors within the meaning of the Securities and Futures Ordinance (Cap.571, Laws of Hong Kong) a
circumstances which do not result in the document being a prospectus within the meaning of the Companies Ordinance (Cap.32, Laws of Hong Kong
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), w
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 whi
persons outside Hong Kong or only to professional investors within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Ho

Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document 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 o
whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapte
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
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 sc
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 acc
investments and each beneficiary is an accredited investor, shares, debentures and units of shares and debentures of that corporation or the beneficiarie
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
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 f

Japan

The securities have not been and will not be registered under the Financial Instruments and Exchange Law of Japan (the Financial Instruments and Exchan
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 a
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 Jap
registration requirements of, and otherwise in compliance with, the Financial Instruments and Exchange Law and any other applicable laws, regulat

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 underwrite
this offering, or by their affiliates. In those cases, prospective investors may view offering terms online and

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depending upon the particular underwriter or selling group member, prospective investors may be allowed to place orders online. The underwriters may
shares for sale to online brokerage account holders. Any such allocation for online distributions will be made by the representatives on the

Other than the prospectus in electronic format, the information on any underwriter s or selling group member s web site and any information contained i
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 end
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 connectio underwriters by Cooley LLP, New York, New York.

EXPERTS

The balance sheets as of June 30, 2012 and 2013, and the related statements of operations, convertible preferred stock and stockholders (deficit) equity ar in this prospectus have been audited by McGladrey LLP, an independent registered public accounting firm, as stated in their report appearing elsewhere 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 so constitutes part of the registration statement, does not include all of the information contained in the registration statement and the exhibits, schedules and a 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 and schedules to the registration statement filed as part of the registration statement. Statements contained in this prospectus about the contents of any cor 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 sta 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 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 mo public reference room. In addition, the SEC maintains an Internet website, which is located at www.sec.gov, that contains reports, proxy and informatio issuers that file electronically with the SEC. You may access the registration statement of which this prospectus is a part at the S

We are subject to the informational and periodic reporting requirements of the Exchange Act. We file periodic reports and other information with the SE annual reports containing financial statements certified by an independent registered public accounting firm. We also maintain a website at *www.agtc.com*, charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. Our website is not a pa

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APPLIED GENETIC TECHNOLOGIES CORPORATION

INDEX TO FINANCIAL STATEMENTS

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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 Jun statements of operations, convertible preferred stock and stockholders (deficit) equity and cash flows for the years then enc responsibility of the Company s management. Our responsibility is to express an opinion on these financial state

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement audit of the Company s internal control over financial reporting. Our audit included consideration of internal control over fin audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supp the financial statements, assessing the accounting principles used and significant estimates made by management, and evalu 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 Appli 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 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
ASSETS	
Current assets:	
Cash and cash equivalents	$ 774
Restricted cash	50
Short-term investments	
Grants receivable	184
Other current assets	87
Total current assets	1,095
Property and equipment, net	53
Intangible assets, net	1,672
Other assets	4
Total assets	$ 2,824
LIABILITIES, CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS (DEFICIT) EQUITY	
Current liabilities:	
Accounts payable	$ 118
Accrued expenses	369
Deferred revenue	
Current portion of debt and capital lease	1,007
Series B purchase rights	
Total current liabilities	1,494
Long-term liabilities:	
Debt and capital lease, net of current portion	16
Warrant liabilities	80
Total liabilities	1,590
Commitments and contingencies (Note 8)	
	21,526

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)	
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
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)	
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)	
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)	
Additional paid-in capital	12,146
Accumulated deficit	(43,436)
Total stockholders (deficit) equity	(31,290)
Total liabilities, convertible preferred stock and stockholders (deficit) equity	$ 2,824

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)

Revenue:	
Grant revenue	
Sponsored research revenue	
Total revenue	
Operating expenses:	
Research and development	
General and administrative	
Total operating expenses	
Loss from operations	
Other income (expense):	
Interest income	
Interest expense	
Fair value adjustments to warrant liabilities	
Fair value adjustments to Series B purchase rights	
Total other income (expense), net	
Net loss	
Net loss per share, basic and diluted	
Weighted-average shares outstanding, basic and diluted	
Pro forma net loss per share, basic and diluted (unaudited) (Note 2)	
Weighted-average pro forma shares outstanding, basic and diluted (unaudited) (Note 2)	

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 (DEFI

FOR THE FISCAL YEARS ENDED JUNE 30, 2012 AND 2013

(in thousands)

	Convertible Preferred Stock									
	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
Balance, June 30, 2011	22,466	$ 21,526	11,479	$ 10,998		$		$		$
Share-based compensation										
Net loss										
Balance, June 30, 2012	22,466	$ 21,526	11,479	$ 10,998		$		$		$
Beneficial conversion of notes payable to preferred stock										
Conversion of notes payable					5,970	741				
Issuance of preferred stock and Series B purchase rights, net of issuance costs					60,177	5,798	122,750	19,040		
Share-based compensation										
Net loss										
Balance, June 30, 2013	22,466	$ 21,526	11,479	$ 10,998	66,147	$ 6,539	122,750	$ 19,040		$
Issuance of Series B-3 convertible preferred stock (unaudited)									58,817	10,722
Conversion of convertible preferred stock	(22,466)	(21,526)	(11,479)	(10,998)	(66,147)	(6,539)	(122,750)	(19,040)	(58,817)	(10,722)

o common stock (unaudited)					
Reclassification of Series B purchase rights (unaudited)					
Reclassification of warrants to purchase stock o additional paid-in capital (unaudited)					
Pro Forma, June 30, 2013 (unaudited)	$	$	$	$	$

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)

Cash flows from operating activities	
Net loss	
Adjustments to reconcile net loss to net cash used in operating activities:	
Share-based compensation	
Depreciation and amortization	
Non-cash interest expense	
Fair value adjustments to warrant liabilities	
Fair value adjustments to Series B purchase rights	
Change in operating assets and liabilities	
Decrease in grant receivable	
Increase in other current assets	
(Decrease) increase in accounts payable	
Increase in deferred revenues	
Increase (decrease) in accrued expenses	
Net cash used in operating activities	
Cash flows from investing activities	
Purchase of property and equipment	
Purchase of and costs related to intangible assets	
Release of restricted cash	
Purchase of short-term investments	
Net cash used in investing activities	
Cash flows from financing activities	
Proceeds from issuance of preferred stock and Series B purchase rights, net of issuance costs of $306	
Proceeds from issuance of convertible notes with detachable warrants	
Proceeds from issuance of bank term note and warrants	
Payment of bank term notes and capital lease	
Net cash provided by financing activities	
Net (decrease) increase in cash and cash equivalents	
Cash and cash equivalents, beginning of year	

Cash and cash equivalents, end of year

Supplemental disclosure of cash flow information

Cash paid for interest

Supplemental disclosure of non-cash financing activities

Capital lease of property and equipment

Conversion of Series B purchase rights to Series B-2 convertible preferred stock

Conversion of notes payable and accrued interest to Series B-1 convertible preferred stock

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 Janua corporation on October 24, 2003. The Company is a clinical-stage biotechnology company developing gene therapy produc 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 any products. The Company has generated revenue from collaboration agreements, sponsored research payments and grants, bu date and is subject to a number of risks similar to those of other early stage companies, including dependence on key indivi development of commercially viable products, the need to obtain additional capital necessary to fund the development of its companies. As of June 30, 2013, the Company had an accumulated deficit of $48,426. The Company has financed its operati placements of its convertible preferred stock, collaborations, bank debt, convertible debt financings, grant funding and pay Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, development technological innovations, risks of failure of clinical studies, dependence on key personnel, protection of proprietary techno regulations and ability to transition to large-scale production of products. The Company expects to continue to incur losses f 2013, the Company had capital resources consisting of cash, cash equivalents and short-term investments of $22,893 and b sufficient to allow the Company to fund its current operating plan for at least the next 12 mo

(2) Summary of Significant Accounting Policies:

(a) **Basis of Presentation** The accompanying financial statements have been prepared in conformity with accountin United States (GAAP).

(b) **Segment Reporting** The Company operates in only one segment. The chief operating decision-maker and mana 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: Company s Series B-3 preferred, which the Company expects to occur on November 5, 2013 (note 14), for cash p all the convertible preferred stock, including the Series B-3, into shares of common stock upon the consummation reclassification of the Series B purchase rights liability to additional paid-in capital; and the conversion of all outs of Series A-1, Series A-1A and Series B-1 preferred stock into warrants exercisable for shares of common stock, r liability being reclassified to additional paid-in capital. Unaudited pro forma net loss per share is computed using common stock equivalents outstanding after giving effect to the conversion of all the convertible preferred stock i 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 ma
the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the fi
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
equivalents. Cash and cash equivalents include cash held in banks and money market accounts. Cash equivalents a
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
current unless a related liability is classified as long-term. The balance sheet at June 30, 2012 includes $50 in cash
Company s credit card with a credit limit of the same amount. The collateral money market account paid interest
maintained the credit card on an unsecured basis as of June 30, 2013. The balance sheets at both June 30, 2012 an
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
investments. Short-term investments include certificates of deposit with maturity within 91 to 360 days of date of
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 liabiliti
assessment of the inputs used in determining the reported fair values. The Financial Accounting Standards Board
Codification (ASC) Topic 820, *Fair Value Measurements and Disclosures* (ASC 820), establishes a hierarc
inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained
Company. Unobservable inputs are inputs that reflect the Company s assumptions about the inputs that market pa
or liability, and are developed based on the best information available in the circumstances. The fair value hierarc
used in determining the reported fair value of the investments and is not a measure of the investment credit quality
hierarchy are described below:

Level 1 Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Comp
measurement date.

Level 2 Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all sig
directly or indirectly.

Level 3 Valuations that require inputs that reflect the Company s own assumptions that are both significant to the fair

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determ
judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instrumen
instrument s level within the fair value hierarchy is based on the lowest level of any input that is significant to th

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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 wa

(i) **Property and equipment** Property and equipment are recorded at cost. Depreciation is computed using the stra useful lives of the assets, which are generally three to seven years. The Company incurs maintenance costs on son 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 fron related to exclusive licenses that have alternative future use in multiple potential programs. The Company also cap issuance, and prosecution of patents. The Company reviews its capitalized costs periodically to determine that cos applications that have future value. The Company evaluates costs related to patents that it is not actively pursuing Amortization expense is computed using the straight-line method over the estimated useful lives of the assets, whi The Company amortizes in-licensed patents and patent application from the date of the applicable license and inte applications from the date of the initial application. Licenses and patents converted to research use only are expen

(k) **Impairment of long-lived assets** The Company reviews its long-lived assets for impairment when impairment indicators exist, management determines whether impairment in value has occurred by comparing the estimated u operations with the carrying values of the assets. Management considers several indicators in assessing impairmen well as the effects of obsolescence, demand, competition and other economic factors. For the fiscal years ended Ju did not identify any indicators of impairment for its long-lived assets. The Company has not yet generated positive not materialize for a significant period in the future. As a result, future evaluations of long-lived assets may result been impaired.

(l) **Warrants to purchase convertible preferred stock** In conjunction with various financing transactions, the Co shares of the Company s Series A-1, Series A-1A and Series B-1 preferred stock. The Company s Series A-1, Se are subject to redemption under circumstances outside of the Company s control. Therefore, the associated shares Consequently, the warrants to purchase shares of Series A-1, Series A-1A and Series B-1 preferred stock are acco fair value at the end of each reporting period. The fair value of the warrants classified as liabilities is estimated usi model. The estimates in Black-Scholes option pricing model are based, in part, on subjective assumptions, includi warrants, risk free interest rate, dividend yield, and fair value of the preferred stock underlying the warrants. Such the future. The gain or loss associated with the change in the fair value of the preferred stock warrant liability fron

component of other (expense) income, net.

(m) **Revenue recognition** The Company has primarily generated revenue through collaboration agreements, sponsc 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. Th
amounts are realized or realizable and earned. Revenue is considered realizable and earned when the following cri
of an arrangement exists; (2) delivery has occurred or services have been rendered; (3) the price is fixed or determ
amounts due are reasonably assured.

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue in the Company s ba
recognized as revenue within the 12 months following the balance sheet date are classified as current liabilities. The Company
of research and development costs under collaboration agreements as the services are performed. The Company records these r
reduction of research and development expenses, as the Company has the risks and rewards as the principal in the resea

The Company evaluates the terms of sponsored research agreement grants and federal grants to assess the Company s obligati
satisfied by the passage of time, revenue is recognized on a straight-line basis. In situations where the performance of the Co
when the grant is received, revenue is recognized upon receipt of the grant. Certain grants contain refund provisions. The Con
to determine the likelihood of repayment. If the likelihood of repayment of the grant is determined to be remote, the grant is re
of repayment is determined to be more than remote, the Company records the grant as a deferred revenue liability, until such
been satisfied.

(n) **Income taxes** The Company uses the asset and liability method for accounting for income taxes. Under this me
are recognized for the estimated future tax consequences attributable to differences between the financial statemer
and liabilities and their respective income tax bases. Deferred tax assets and liabilities are measured using enacted
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 t
likely than not sustain the position following an audit. For tax positions meeting the more-likely-than-not threshold, the amoun
is the largest benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the releva
income tax returns in the U.S. federal jurisdiction and the state of Florida. As of June 30, 2012 and 2013, the Company does
positions.

(o) **Research and development** Research and development costs include costs incurred in identifying, developing
consist primarily of payroll expenses for research related employees, laboratory costs, animal and lab maintenanc
and pre-clinical expenses, as well as payments for sponsored research, scientific and regulatory consulting fees an
as incurred. Costs for certain development activities are recognized based on an evaluation of the progress to com
information and data provided to us by our vendors and our clinical sites. When outside contracts for research pro

payments, they are recorded on the balance sheet as a prepaid item and expensed when the service is provided or r
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, current assets on the balance sheets.

(p) **Inventory** The Company expenses costs for clinical materials stored for master and working viral banks that re future use at those sites. Since the Company can use each of the raw materials in only a single product, each raw 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 the grant date fair value of the award. That cost is recognized on a straight-line basis over the period during which service in exchange for the award. The fair value of options on the date of grant is calculated using the Black-Sch assumptions such as stock price, expected volatility and expected term. The Company s estimates of these assum valuations, historical data, peer company data and judgment regarding future trends and factors. The Company ac non-employees in accordance with the provisions of ASC Subtopic 505-50, *Equity-Based Payments to Non-emplo* options using the Black-Scholes option pricing model and measuring such stock options to their current fair value

(r) **Net loss per share and unaudited pro forma net loss per share** Basic net loss per share is calculated by divid shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per sh average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determi For purposes of the diluted net loss per share calculation, preferred stock, stock options, and warrants are consider have been excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive for all diluted net loss per share was the same for all periods presented. The calculations for the unaudited pro forma bas the conversion of all outstanding shares of preferred stock into shares of common stock as if the conversions had or the date of issuance, if later.

(3) Property and Equipment, Net:

Property and equipment consists of the following:

Lab equipment	
Office equipment	
Leasehold improvements	
Software	
Property and equipment, gross	
Less: Accumulated depreciation and amortization	
Property and equipment, net	

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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. Dep
$10 and $14 was included in general and administrative expenses for the years ended June 30, 2012 and 2013, respectively. De
$44 and $50 was included in research and development expenses for the fiscal years ended June 30, 2012 and 2013, respecti
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:

Licenses	
Patents	
Other	
Intangible assets, gross	
Less: Accumulated amortization	
Intangible assets, net	

Amortization expense related to intangible assets for the years ended June 30, 2012 and 2013 was $208 and $221, respectivel
intangible assets is included in research and development expenses on the statements of opera

Estimated amortization expense for the next five years and thereafter is as follows:

Fiscal Year Ending June 30,
2014
2015
2016
2017

2018
Thereafter

(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 incentive and/or nonqualified stock options to certain employees and non-employees. On September 18, 2009, the board reso 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 of common stock as incentive and/or nonqualified stock options to certain employees and non-employees. On April 6, 2013, t 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 direc 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 d 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 valuatio and judgment regarding future trends and factors, management has determined the per share price equals or exceeds fair mar market for the Company s stock. The employee options generally vest ratably over four years, with 25% vesting one full yea month thereafter, until vested in full. The options expire ten years from the date of the awa

A summary of the employee option activity is as follows:

	Fiscal 2012	
	Shares	Weigh Avera Exerc Pric
Outstanding, beginning of year	69	$
Granted	4	
Exercised		
Terminated	(4)	12
Outstanding, end of year	69	$

Exercisable, end of year	58
Weighted average fair value of options granted during the year	$ 1.75

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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		June 30, 2013
Exercise Price	**Number**	**Weighted Average Contractual Life Remaining**	**Number**
$0.35			193
$3.50	69	5.49	69
	69		262

The following table summarizes information about incentive stock options exercisable:

	June 30, 2012		June 30, 2013
Exercise Price	**Number**	**Weighted Average Contractual Life Remaining**	**Number**
$0.35			20
$3.50	58	5.10	65
	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 o
available to be granted. As of June 30, 2012 and 2013, there was $16 and $30, respectively, of total unrecognized compensatio
stock options.

Share-based compensation cost related to employee incentive stock options included in expense amounted to $18 and $19 fo
2013, respectively. The expense was allocated as follows:

Research and development	
General and administrative	

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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
estimating fair value:

Assumption	**2012**	**Fi**
Dividend yield		
Expected term		
Risk-free interest rate		
Expected volatility		

The dividend yield is based upon the assumption that the Company will not declare a dividend over the life of the options. Sinc
been unable to use historical employee exercise and option expiration data to estimate the expected term assumption for the B
Company therefore has utilized the simplified method, as prescribed by the SEC s Staff Accounting Bulletin No. 107, *Shar*
basis the expected term of our stock options considered to have plain vanilla characteristics. The risk-free interest rate is bas
date of the grant. The Company computes volatility under the calculated value method of ASC 718 by utilizing the av
publicly-traded companies and expect to continue to do so until the Company has adequate historical data regarding the volatil
The peer group was determined based upon companies considered to be direct competition or having been presented by indepe
based upon market sector. In determining a comparable, the Company has excluded large-cap entities. Forfeitures are estima
necessary, in subsequent periods if actual forfeitures differ from those estimates. Share-based compensation expense recognize
years ended June 30, 2012 and 2013 does not record tax related effects on stock-based compensation given the Company s hi
and offsetting changes in its valuation allowance that fully reserves against potential deferred ta

The fair value of the shares of common stock that underlie the stock options the Company has granted has historically been d
directors based upon information available to it at the time of grant. The Company s board of directors considered numerous
assessment of fair value, including reviews of the Company s business and financial condition, the conditions of the industry
markets that the Company serves and general economic, market and United States and global capital market conditions, the
stock, the likelihood of achieving a liquidity event for the shares of common stock underlying these stock options, the prefer
stock over the rights of the common stock, the status of the clinical trials and preclinical studies relating to its product candid
common stock. The Company s board has generally considered the most persuasive evidence of fair value to be the prices at v
in actual arms length transactions.

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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)

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 t
market value. There is currently no active market for the Company s stock. The non-employee options vest variably over thre
the date of the award. A summary of non-employee option activity follows:

	Fiscal 2012	
	Shares	Weigh Avera Exerc Pric
Outstanding, beginning of year	64	$
Granted	1	
Terminated	(1)	
Outstanding, end of year	64	$
Exercisable, end of year	59	
Weighted average fair value of options granted during the year	$ 1.75	

In accounting for stock options to non-employees, the value of goods and services related to the options granted are recognized
consistent with receipt of services. Therefore, vested portions vary based upon services and terms of each option. The Compa
options each reporting period using the estimated fair value of the Company s common stock as of the last day of each report
amounted to $6 for the years ended June 30, 2012 and 2013 and was allocated to general and administ

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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)

(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 basis:

Description	Total	Quoted prices in active markets (Level 1)	Significan observabl (Leve
Assets:			
June 30, 2013			
Short-term investments	$ 14,000	$	$
Liabilities:			
June 30, 2012			
Warrant liabilities	$ 80	$	$
June 30, 2013			
Series B purchase rights	$ 2,096	$	$
Warrant liabilities	110		
Total	$ 2,206	$	$

Short-term investments Short-term investments consist of certificates of deposit placed through an account registry servi which the fair market value is measured based on level 2 inputs (quoted prices for identical assets in markets

Warrant liabilities In connection with various financing transactions that were consummated in periods prior to June 30, 20 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 and lenders. Each warrant was immediately exercisable and generally expires approximately 5 or 10 years from the origina purchase shares of the Company s convertible preferred stock have an exercise price equal to the estimated fair value of the u such shares were issued. Each warrant is exercisable on either a physical settlement or net share settl

There were no exercises, cancellations, or expirations of warrants during the fiscal years ended June 30, 2012 and 2013. A exercisable as of June 30, 2012 and 2013.

The terms and accounting treatment for the warrants outstanding are summarized below

		June 30, 2012		
Warrants to purchase:	**Shares**	**Exercise Price**	**Expiration**	**Share**
Series A-1 Convertible Preferred Stock	384	$ 0.9658	October 3, 2013 - July 5, 2017	38
Series A-1A Convertible Preferred Stock	94	$ 0.9658	October 3, 2013 - July 5, 2017	9
Series B-1 Convertible Preferred Stock	1,145	$ 0.1297	May 2, 2017	1,42
	1,623			1,90

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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)

(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 Black-Scholes option pricing model. The significant assumptions used in preparing the option pricing model for valuing

Assumption	Fiscal Year 2012
Exercise price	$ 0.1297 to $0.9658
Fair value of preferred shares	$0.1297
Expected life (in years)	1.26 to 7.17
Risk-free interest rate	0.27% to 1.39%
Expected volatility	65.02%

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 the sale of up to 290,782 shares of convertible preferred stock in three separate tranches of Series B-1, Series B-2 and Serie Simultaneously with the execution of the Series B Purchase Agreement, the Company issued and sold an aggregate of 66,147 s price per share of $0.1297. The Series B Purchase Agreement provided that the holders of the Series B-1 shares (Series B hol an aggregate of 140,542 shares of Series B-2 preferred stock for an aggregate purchase price equal to $18,228 (the second tra shares of Series B-3 preferred stock for an aggregate purchase price equal to $10,722 (the third tranche). The price per sha exchange for such amount was to be determined separately for each tranche by reference to which, if any, of three milestone 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 seats on the board of directors, the decision to complete the second and third tranche was deemed to be outside the control of t recorded, at the time of entry into the Series B Purchase Agreement, a Series B purchase right liability of $1,723 for the fair va the Series B-2 and Series B-3 preferred stock in the second and third tranches. The Series B purchase right liability was value Black-Scholes option-pricing method to assign a value to the purchase right relating to that series under each of the possible ap on which milestones were met, with each scenario being assigned an estimated probability as of the valuation date. The agg valuations was assigned as the value of the purchase right for each tranche. The initial fair value of the Series B purchase rig second tranche and $1,111 for the third tranche. The total value allocated to the Series B purchase rights reduced the amount Series B-1 preferred stock on the Company s balance sheet.

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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)

(6) Fair Value of Financial Instruments and Investments: (Continued)

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

Assumption
Exercise price
Years to maturity
Risk-free interest rate
Volatility

The most significant and judgmental inputs driving the fair value of the Company s Series B purchase rights are the assum underlying preferred shares and the volatility factor. With all other inputs constant, an increase or decrease in the assumed fa result in a higher or lower estimate of the fair value of the Series B purchase rights, respectively, although there would not b increase or decrease in the assumed volatility factor would result in a higher or lower estimate of the fair value of the Seri

In April 2013, following the satisfaction by the Company of the first milestone, the Series B holders exercised their rights w 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 change in value of the Series B purchase right liability of $1,207 was recorded to other expense, and the $834 balance of th purchase right immediately prior to the closing of the second tranche was recorded as proceeds of the issuance of the

The Company reports the change in fair value during each period as a non-operating gain or loss recorded as a component of ot of operations. The table presented below is a summary of changes in the fair value of the Company s Level 3 valuation for w rights for the fiscal years ended June 30, 2012 and 2013:

Beginning balance as of July 1, 2011	
Fair value of warrants issued	
Change in fair value of during period	
Ending balance as of June 30, 2012	
Fair value of warrants issued	
Fair value of Series B purchase rights issued	

Change in fair value of during period	
Series B purchase rights converted to Series B-2 convertible preferred stock	
Ending balance as of June 30, 2013	

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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)

(7) **Debt and Capital Lease:**

Debt and capital lease are summarized as follows:

Term loans, net of original issue discount	
Convertible notes payable, net of original issue discount	
Capital lease	
Less current portion	
Debt and capital lease, net of current portion	

Term loans In July 2010, the Company entered into a loan and security agreement with Square 1 Bank. Under the terms of
$800 in July 2010 in exchange for the issuance of a promissory note. The note carried a fixed interest rate of 7%. Interest-only
January 2011, followed by 30 equal installments of principal and interest. In consideration of this agreement, the Company iss
Series A-1 preferred stock with an exercise price of $0.9658 per share. The warrants are exercisable upon issuance and will a
seven years after issuance, if not already exercised. The estimated fair value of the warrants at issuance was $32 which was rec
This discount was amortized over the original life of the loan using the effective interest rate method. Upon early repayment co
the loss on extinguishment was not material and was classified as interest expense. The loan was collateralized by all assets
property.

In August 2012, the Company entered into an amended loan and security agreement with Square 1 Bank to provide additional
loan. Under the terms of this amended agreement, in September 2012, the Company borrowed $507 in exchange for the issu
carried an interest rate of 9% through December 2012 and 7% thereafter. Interest-only payments were paid monthly through
installments of principal and interest. In consideration of this amended agreement, the Company issued warrants to purchase 2
with an exercise price of $0.1297 per share. The warrants are exercisable upon issuance and will automatically convert upon e
not already exercised. The estimated fair value of the warrants at issuance was $22 which was recorded as a discount to the not
over the original life of the loan using the effective interest rate method. Upon early repayment concurrent with the Series B-2
was not material and was classified as interest expense. The loan was collateralized by all assets of the Company e

Interest expense for both notes for the years ended June 30, 2012 and 2013, including non-cash amortization of the discount
amended loan and security agreement was terminated in April 2013 upon payment of the note b

Capital lease In September 2011, the Company converted an operating lease for its phone system into a capital lease agree repayable monthly over 24 months, beginning October 2011. During fiscal year 2012, these assets were capitalized as office accordingly. As of June 30, 2013, the outstanding capital lease balance was $1.

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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)

(7) Debt and Capital Lease: (Continued)

Convertible note In May 2012, the Company entered into a convertible note and warrant purchase agreement with existin proceeds of $750. The term notes had an interest rate of 8.0% per annum, with principal and interest payable at the stated date earlier due to a liquidity event or a financing with gross proceeds to the Company of at least $10,000 (a Trigger Financing maturity date with a rate of 10.0% per annum beyond the stated due date. The warrants issued were for the purchase of Series price of $0.1297 per share. The estimated fair value of the warrants at issuance was $79 which was recorded as a discount to amortized over the original life of the loan using the effective interest rate method until these notes converted into Series B p described further below.

All unpaid principal and accrued but unpaid interest on these convertible notes would be converted automatically into prefer 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 equal to the quotient obtained by dividing (i) the outstanding principal and accrued but unpaid interest under the note by (ii) th price per share of such New Preferred, and the issuance of such shares upon such conversion would be upon the same terms a 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 clos occurred any transaction or series of related transactions resulting in the (a) acquisition of greater than 50% of the voting equity stock purchase, share exchange or other form of corporate reorganization, (b) acquisition, consolidation, merger or like transac the shareholders of the Company immediately prior to such transaction own less than 50% of the voting power of the surviv transfer or other conveyance of all or substantially all of the assets of the Company (a Liquidity Event), the holder of eac convert the note into shares of the Company s Series A-1 Preferred Stock (or, at the holder s election, shares of the Compa Existing Preferred). The number of shares of Existing Preferred to be issued upon such conversion would be equal to th outstanding principal and accrued but unpaid interest under the note by (ii) the price per share obtained by dividing (A) $42,2 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 payable with a carrying value of $709 and related accrued interest of $32 into 5,970 shares of Series B-1 Preferred Stock. The beneficial conversion feature related to a Trigger Financing. The issuance of the Series B preferred stock met the definitio contingency was resolved. Accordingly, upon conversion of the notes payable, the Company recognized a beneficial conversio conversion charge has been included as a component of interest expense in the statement of operations. Interest expense for th 2012 and 2013, including non-cash amortization of the discount and the beneficial conversion charge discussed above,

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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)

(7) Debt and Capital Lease: (Continued)

The aggregate future maturities of the Company s capital lease as of June 30, 2013 were as f

Fiscal Year Ending June 30,	
2014	
Less: amount representing interest	
Total future maturities	

(8) Commitments and Contingencies:

Operating leases The Company leases office equipment, office space, and lab space under operating leases expiring throug June 30, 2012 and 2013, rent expense under these and other operating leases was $82 and $102, respectively. Minimum future operating leases as of June 30, 2013 in the aggregate are:

Fiscal Year Ending June 30,	
2014	
2015	
Total minimum future lease payments	

Other contingencies Under various agreements, the Company will be required to pay royalties and milestone payments u commercialization of products. The Company has entered into funding agreements with various not-for-profit organizations. T pay royalties on net product sales of any collaboration product that it successfully develops and subsequently commerciali collaboration product, a specified percentage of certain payments it receives from its licensee. The Company is not obligated t annual sales of a collaboration product exceed a designated threshold. The Company s obligation to make such payments wo amount.

The Company is also party to various agreements entered into in the ordinary course of its business, principally relating to lic payments relating to milestones or royalties on future sales of specified products. At June 30, 2013, the Company had nine l entities, including five with the University of Florida Research Foundation. Several of these entities are stockholders of the C

pay minimum annual royalty and license maintenance for all licenses until such time when the license is terminated by eithe
voluntary termination by either party per the agreement. Once a product reaches commercialization, the above-mentioned mini
by annual royalties ranging from 0.5% to 4.0% on net sales. The Company is responsible for all costs related to preparatio
maintenance of the underlying patents covered in the license agreements. As of June 30, 2013, the Company held one license w
that requires additional royalty payments. The Company may terminate its license agreements with zero to ninety days writte
each specific agreement. The Company paid annual royalty and license maintenance payments of $41 and $61 for the fiscal
respectively. All royalty and license maintenance payments are included in research and development expenses on

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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)

(8) Commitments and Contingencies: (Continued)

Minimum annual royalty and license maintenance payments under these agreements are as fo

Fiscal Year Ending June 30,
2014
2015
2016 and every fiscal year thereafter

The Company enters into standard indemnification agreements in the ordinary course of business. Pursuant to the agreemen harmless, and agrees to reimburse the indemnified party for losses suffered or incurred by the indemnified party, generally customers, in connection with any U.S. patent or any copyright or other intellectual property infringement claim by any thir products. The term of these indemnification agreements is generally perpetual. The maximum potential amount of future paym make under these indemnification agreements is unlimited. The Company has never incurred costs to defend lawsuits or settle agreements. From time to time, the Company is involved in various claims and legal actions that arise in the normal course of outcome of such legal actions will not have a significant adverse effect on the Company s financial position, result

(9) Concentrations:

The Company has demand deposits and money market funds in a regional bank that are insured by the FDIC up to FDIC li short-term investments in certificates of deposits at various financial institutions that are 100% FD

All of the Company s grant receivables at June 30, 2012 and 2013 are derived or due from government grants. Any future cha 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 t

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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)

(10) Income Taxes: (Continued)

Deferred taxes are recognized for temporary differences between the basis of assets and liabilities for financial statement and
components of the Company s deferred tax assets (liabilities) are comprised of the follow

Deferred tax assets:	
Net operating loss carryforwards	
Research and development credit carryforwards	
Accruals and other	
Gross deferred tax assets	
Deferred tax asset valuation allowance	
Total deferred tax assets	
Deferred tax liabilities:	
Depreciation and amortization	
Total deferred tax liabilities	
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 in
2022 to 2033. At June 30, 2013, the Company also has research and development tax credits of approximately $873 that may
from 2027 to 2042.

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based
losses, the Company has concluded that it is more likely than not that the benefit of its deferred tax assets will not be realize
realized in future years as a result of the utilization of the Company s net operating loss carry forwards as of June 30, 2013, c
state rates, are completely offset by valuation allowances established since realization of the deferred tax benefits are not co
valuation allowance increased approximately $909 during the fiscal year ended June 30, 2013, due primarily to net operating

A reconciliation of income tax expense computed at the statutory federal income tax rate to income taxes as reflected in the

Federal income tax benefit at statutory rate	
State income tax, net of federal benefit	
Permanent differences	
Research and development credit	
Other	
Change in valuation allowance	
Effective income tax rate	

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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)

(10) Income Taxes: (Continued)

Under the provisions of the Internal Revenue Code, the Company s net operating loss and tax credit carry forwards are subje the Internal Revenue Service and state tax authorities. Net operating loss and tax credit carry forwards may become subject t certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50 perce 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attribute future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Compan change. Subsequent ownership changes may further affect the limitation in future years. The Company has completed severa may have resulted in a change in control as defined by Sections 382 and 383 of the Internal Revenue Code, or could result

For fiscal years through June 30, 2013, the Company generated research credits but has not conducted a study to document th result in an adjustment to the Company s research and development credit carry forwards; however, until a study is comple amounts are being presented as an uncertain tax position for these two years. A full valuation allowance has been provided development credits and, if an adjustment is required, this adjustment would be offset by an adjustment to the deferred tax a 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 genera tax years ended June 30, 2009 through June 30, 2013. To the extent the Company has tax attribute carry forwards, the tax yea may still be adjusted upon examination by the Internal Revenue Service, or state authorities, to the extent utili

The Company s policy is to recognize interest and penalties related to uncertain tax positions in income tax expense. As of Ju no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Compa

(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 2 its Certificate of Incorporation to increase the number of shares authorized to be issued to 410,000 shares of $0.001 par value 2013, the Company had 109 shares issued and outstanding.

The following shares of common stock are reserved for future issuance:

Conversion of preferred stock and preferred stock warrants	
Stock options issued and outstanding	

Authorized for future grant under the 2011 Stock Incentive Plan

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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)

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	Aggrega A
Series A-1	29,737	$ 0.9658	22,466	$
Series A-1A	11,572	$ 0.9658	11,479	
Balance at June 30, 2012	41,309		33,945	$
Series A-1	29,737	$ 0.9658	22,466	$
Series A-1A	11,572	$ 0.9658	11,479	
Series B-1	67,570	$ 0.1297	66,147	
Series B-2	140,542	$ 0.1485	122,750	
Series B-3	82,670			
Balance at June 30, 2013	332,091		222,842	$

Rights and privileges of preferred stock In November 2012, in connection with the transactions contemplated by the Serie amended and restated its certificate of incorporation. The material rights and privileges of the Company s preferred stock as 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 wh Preferred Stock, 11,572 are designated Series A-1A Preferred Stock, 67,570 are designated Series B-1 Preferred Stock, 140,5 Stock and 82,670 are designated Series B-3 Preferred Stock. The Series A-1 and Series A-1A Preferred Stock are referred to the Series B-1, B-2 and B-3 Preferred Stock are referred to collectively as the Series B Preferred and the Series A Preferre 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 each such share. Such dividends are payable only when, as and if declared by the board of directors of the Company and are conversion of Preferred Stock to common stock in the event a successful initial public offering, any dividends declared and u 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 involuntar distribution or payment shall be made to the holders of any Series A Preferred or common stock, the holders of shares of the S paid, out of the assets of the Company legally available for distribution (or the consideration received in any Deemed Liquidat of incorporation), an amount equal to the purchase price of such Series B Preferred share plus all declared and unpaid dividen B-2 Preferred or Series B-3 Preferred, as the case may be (the Series B Liquidation Preference). If, upon any such Liquidat the consideration received in the Deemed Liquidation Event) shall be insufficient to make payment in full to all holders of Liquidation Preference, then such assets (or consideration) shall be distributed among the holders of Series B Preferred at the t 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 avai

Upon any Liquidation Event, after the payment in full of the Series B Liquidation Preference to the holders of Series B Pref payment shall be made to the holders of any common stock, the holders of shares of the Series A Preferred shall be entitled Company legally available for distribution (or the consideration received in the Deemed Liquidation Event), for each share of the purchase price of such Series A Preferred share plus all declared and unpaid dividends on the Series A-1 Preferred and Ser (the Series A Liquidation Preference). If, upon any such Liquidation Event, the assets of the Company (or the considerati Event) shall be insufficient to make payment in full to all holders of Series A Preferred of the Series A Liquidation Preferenc shall be distributed among the holders of Series A Preferred at the time outstanding, ratably in proportion to the amounts to wh to such shares of Series A Preferred if sufficient assets were available to make such payment i

Upon any Liquidation Event, after the payment in full of the Series B Liquidation Preference and the Series A Liquidation P legally available for distribution in such Liquidation Event (or the consideration received in the Deemed Liquidation Event), if holders of the common stock and the Preferred Stock on an as-if-converted to common stock

Conversion Rights. Each share of Preferred Stock is convertible, at the option of the holder thereof, at any time and from tim additional consideration by the holder thereof, into fully-paid and nonassessable shares of common stock. As of June 30, 2013, to which a holder of shares of Preferred Stock was entitled upon conversion was as follows: Series A Preferred, 0.06 shares;

The conversion ratios applicable to the Preferred Stock are subject to adjustment in the event that the Company effects any sub dividend or distribution in shares of common stock, in each case in respect of its common stock, or if the common stock issuab Stock is changed into the same or a different number of shares of any class or classes of stock, whether by recapitalization, rec otherwise. The conversion ratios applicable to the Preferred Stock are also subject to anti-dilution adjustment in the event Company of its common stock.

Each share of Preferred Stock shall automatically be converted into shares of common stock, based on the then-effective appl upon the written consent of the holders of at least a majority of the then outstanding shares of Preferred Stock, voting as a sin closing of a firmly underwritten public offering pursuant to an effective registration statement under the Securities Act of 193 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 splits, dividends, recapitalizations and the like after the filing date hereof), or, in the event that the closing of the third tranche Purchase Price has not been determined, then the per share price is at least $0.7425 (as adjusted for stock splits, dividends, r filing date hereof) and (y) the gross cash proceeds to the Company (before underwriting discounts, commissions an

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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 of Preferred Stock could be converted immediately after the close of business on the record date for voting. Except as othe incorporation or as required by law, holders of shares of Preferred Stock vote together with the holders of shares of commo

Subject to supermajority votes for some matters, matters submitted to a vote of the Company s stockholders shall be dec stockholders having a majority in voting power of the votes cast by the stockholders present or represented and

As long as at least 10% of the authorized shares of Series B Preferred remain outstanding, the holders of the outstanding sha separate class, shall be entitled to elect five (5) members of the board of directors and to remove from office such directors a 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 ou voting as a separate class on an as-if-converted to common stock basis, shall be entitled to elect three (3) members of the b pursuant to each consent of the Company s stockholders for the election of directors, and to remove from office such directo 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 o basis, shall be entitled to elect the remaining member of the board of directors, which member shall be the Company s chief office such director and to fill any vacancy caused by the resignation, death or removal of such

The vote or written consent of the holders of at least a majority of the then outstanding shares of Preferred Stock, voting toget Company to, among other things: liquidate or dissolve; amend, alter or repeal any provision of its certificate of incorporation security convertible into or exercisable for any equity security having rights, preferences or privileges senior to or on parity wit decrease the authorized number of shares of Preferred Stock; with certain exceptions, redeem or declare any dividend on any s incur more than $2,000 of indebtedness; acquire any minority-owned subsidiary or dispose of any capital stock or assets of ar authorized number of members of the Company s board of directors; take any action that would limit, change or alter the right of the Preferred Stock; or make any acquisition of the capital stock or assets of another entity or enter into any strategic allian licensing arrangement, or other corporate partnership with any entity involving the payment, contribution or assignment by

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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:

Research and development-related	
Compensation-related	
Other	

(13) 401(k) Plan:

The Company has a 401(k) Plan (the Plan) through the Company s staff leasing company. Employees may elect to defe
Company matches employee contributions up to 4%. Total matching contributions to the Plan for the years ended June 30, 20
and $40, respectively.

(14) Subsequent Events:

(a) The Company has completed an evaluation of all subsequent events through November 4, 2013, to ensure appropr
recognized in the financial statements as of June 30, 2013, and events which occurred subsequently but were not r
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
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 Compar
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
outstanding immediately prior to that date was combined, reclassified and changed into one thirty-fifth (1/35) of a
common stock. All common share and common per share information in the accompanying financial statements h
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
ASSETS	
Current assets	
Cash and cash equivalents	$ 8,893
Short-term investments	14,000
Grants receivable	143
Other current assets	475
Total current assets	23,511
Property and equipment, net	341
Intangible assets, net	1,630
Other assets	8
Total assets	$ 25,490
LIABILITIES, CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS (DEFICIT) EQUITY	
Current liabilities	
Accounts payable	$ 792
Accrued expenses	359
Deferred revenue	212
Current portion of debt and capital lease	1
Series B purchase rights	2,096
Total current liabilities	3,460
Long-term liabilities	
Warrant liabilities	110
Total liabilities	3,570
Commitments and contingencies	
Series A-1 convertible preferred stock, par value $0.001 per share, 29,737 shares authorized at June 30, 2013 and March 31, 2014, 22,466 and 22,467 shares issued and outstanding at June 30, 2013 and March 31, 2014, respectively, and no shares issued and outstanding pro forma (aggregate liquidation preference of $21,699)	21,526
	10,998

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 March 31, 2014, and no shares issued and outstanding pro forma (aggregate liquidation preference of $11,086)	
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 March 31, 2014, and no shares issued and outstanding pro forma (aggregate liquidation preference of $8,579)	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 March 31, 2014, and no shares issued and outstanding pro forma (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, 2013 and pro forma, 58,817 shares issued and outstanding March 31, 2014 (aggregate liquidation preference of $10,722)	
Stockholders (deficit) equity	
Common stock, par value $.001 per share, 150,000 shares authorized, 109 and 166 shares issued and outstanding at June 30, 2013 and March 31, 2014, respectively, and 9,286 shares issued and outstanding pro forma	
Additional paid-in capital	12,243
Accumulated deficit	(48,426
Total stockholders (deficit) equity	(36,183
Total liabilities, convertible preferred stock and stockholders (deficit) equity	$ 25,490

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)

Revenue	
Grant revenue	
Sponsored research revenue	
Total revenue	
Operating expenses	
Research and development	
General and administrative	
Total operating expenses	
Loss from operations	
Other income (expense)	
Interest income	
Interest expense	
Fair value adjustments to warrant liabilities	
Fair value adjustments to Series B purchase rights	
Total other income (expense)	
Net loss	
Net loss per share, basic and diluted	
Weighted-average shares outstanding, basic and diluted	

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)

Cash flows from operating activities	
Net loss	
Adjustments to reconcile net loss to net cash used in operating activities:	
Share-based compensation	
Depreciation and amortization	
Non-cash interest expense	
Fair value adjustments to warrant liabilities	
Fair value adjustments to Series B purchase rights	
Change in operating assets and liabilities:	
Increase in grant receivable	
Increase in other current assets	
Increase in other assets	
Increase (decrease) in accounts payable	
Decrease in deferred revenues	
Increase (decrease) in accrued expenses	
Net cash used in operating activities	
Cash flows from investing activities	
Purchase of property and equipment	
Purchase of and costs related to intangible assets	
Maturity of short-term investments	
Purchase of short-term investments	
Net cash used in investing activities	
Cash flows from financing activities	
Proceeds from exercise of convertible preferred stock warrants	
Proceeds from exercise of common stock options	
Proceeds from issuance of preferred stock and Series B purchase rights, net of issuance costs	
Proceeds from issuance of bank term note and warrants	
Payment of bank term notes and capital lease	
Net cash provided by financing activities	

Net increase (decrease) in cash and cash equivalents
Cash and cash equivalents, beginning of period

Cash and cash equivalents, end of period

Supplemental disclosure of cash flow information
Cash paid for interest

Supplemental disclosure of non-cash financing activities
Conversion of notes payable and accrued interest to Series B-1 convertible preferred stock
Conversion of Series B purchase rights to Series B-3 convertible preferred stock

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 NINE MONTHS ENDED MARCH 31, 2014 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 Janua corporation on October 24, 2003. The Company is a clinical-stage biotechnology company developing gene therapy produc 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 any products. The Company has generated revenue from collaboration agreements, sponsored research payments and grants, bu date and is subject to a number of risks similar to those of other early stage companies in the biotechnology industry, includin difficulties inherent in the development of commercially viable products, the need to obtain additional capital necessary to f development by the Company or its competitors of technological innovations, risks of failure of clinical studies, protection o with government regulations and ability to transition to large-scale production of products. As of March 31, 2014, the Com $59,813. The Company has financed its operations to date primarily through private placements of its convertible preferre convertible debt financings, grant funding and payments for sponsored research. The Company expects to continue to incur March 31, 2014, the Company had capital resources consisting of cash, cash equivalents and short-term investments of $24,53 be sufficient to allow the Company to fund its current operating plan for at least the next 12 months. On April 1, 2014, AGTC and now trades on NASDAQ under the ticker symbol AGTC (see Note 6).

(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 stat included elsewhere in the prospectus of which these financial statements are a part.

(a) **Basis of Presentation** The accompanying financial information as of March 31, 2014 and for the nine months e prepared by the Company, without audit, pursuant to the rules and regulations of the Securities and Exchange Cor footnote disclosures normally included in financial statements prepared in accordance with U.S. generally accepte condensed or omitted pursuant to such rules and regulations. The June 30, 2013 balance sheet was derived from th statements. The financial information as of March 31, 2014 and for the nine months ended March 31, 2013 and 20 the June 30, 2013 audited annual financial statements and notes thereto included elsewhere in the prospectus of w part.

In the opinion of management, the unaudited financial information as of March 31, 2014 and for the nine months ended M adjustments, which are normal recurring adjustments, necessary to present a fair statement of the Company s financial positio The results of operations for the nine months ended March 31, 2014 are not necessarily indicative of the operating results to be future period.

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(b) **Pro forma information** The pro forma balance sheet as of March 31, 2014, gives effect to: the conversion of al shares of common stock upon the closing of the IPO; and the conversion of all outstanding warrants exercisable fo and Series B-1 preferred stock into warrants exercisable for shares of common stock, resulting in the preferred sto to additional paid-in capital. The pro forma balance sheet as of March 31, 2014 does not give effect to the Compa IPO discussed in Note 6.

(c) **Use of estimates** The preparation of financial statements in conformity with GAAP requires management to ma the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the fi 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 liabiliti assessment of the inputs used in determining the reported fair values. The Financial Accounting Standards Board Codification (ASC) Topic 820, *Fair Value Measurements and Disclosures* (ASC 820), establishes a hierarc inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained Company. Unobservable inputs are inputs that reflect the Company s assumptions about the inputs that market pa or liability, and are developed based on the best information available in the circumstances. The fair value hierarc used in determining the reported fair value of financial instruments and is not a measure of the investment credit q value hierarchy are described below:

Level 1 Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Comp measurement date.

Level 2 Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all sig directly or indirectly.

Level 3 Valuations that require inputs that reflect the Company s own assumptions that are both significant to the fair

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determ judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instrumen instrument s level within the fair value hierarchy is based on the lowest level of any input that is significant to th

Items measured at fair value on a recurring basis include short-term investments, Series B purchase rights and wa

(e) **Warrants to purchase convertible preferred stock** In conjunction with various financing transactions, the Co shares of the Company s Series A-1, Series A-1A and Series B-1 preferred stock. The Company s Series A-1, Se

are subject to redemption under circumstances outside of the Company s control. Therefore, the associated shares
Consequently, the warrants to purchase shares of Series A-1, Series A-1A and Series B-1 preferred stock are acco
fair value at the end of each reporting period. The fair value of the warrants classified as liabilities is

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estimated using the Black-Scholes option pricing model. The estimates in the Black-Scholes option pricing model assumptions, including stock price volatility, term of the warrants, risk free interest rate, dividend yield, and fair v the warrants. Such assumptions could differ materially in the future. The gain or loss associated with the change in 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 the grant date fair value of the award. That cost is recognized on a straight-line basis over the period during which service in exchange for the award. The fair value of options on the date of grant is calculated using the Black-Sch assumptions such as stock price, expected volatility and expected term. The Company s estimates of these assum valuations, historical data, peer company data and judgment regarding future trends and factors. The Company ac non-employees in accordance with the provisions of ASC Subtopic 505-50, *Equity-Based Payments to Non-emplo* 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 d probable equity financings as Other Assets until such financings are consummated. After consummation of an in- these costs are recorded in stockholders equity as a reduction of additional paid-in capital generated as a result o Company recorded deferred financing costs of $1,748 in other assets in the accompanying balance sheet in conten These costs were netted against the proceeds of the Company s initial public offering discussed in Note 6.

(h) **New Accounting Pronouncements** In July 2013, the FASB issued amended guidance on the financial statemen benefit when a net operating loss carryforward, similar tax loss, or tax credit carryforward exists. The guidance re a portion of an unrecognized tax benefit, to be presented as a reduction of a deferred tax asset when a net operatin tax credit carryforward exists, with certain exceptions. This accounting guidance is effective prospectively for the quarter of fiscal year 2015, with early adoption permitted. While the Company is currently evaluating the impact, material impact on the Company s financial statements.

(i) **Revenue recognition** The Company has primarily generated revenue through collaboration agreements, sponso nonprofit organizations for the development and commercialization of product candidates and revenues from fede programs. The Company recognizes revenue when amounts are realized or realizable and earned. Revenue is cons following criteria are met: (1) persuasive evidence of an arrangement exists; (2) delivery has occurred or services 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 ba recognized as revenue within the 12 months following the balance sheet date are classified as current liabilities. The Company of research and development costs under collaboration agreements as the services

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are performed. The Company records these reimbursements as revenue and not as a reduction of research and development ex
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 obligati
satisfied by the passage of time, revenue is recognized on a straight-line basis. In situations where the performance of the Co
when the grant is received, revenue is recognized upon receipt of the grant. Certain grants contain refund provisions. The Con
to determine the likelihood of repayment. If the likelihood of repayment of the grant is determined to be remote, the grant is re
of repayment is determined to be more than remote, the Company records the grant as a deferred revenue liability, until such
been satisfied.

(j) **Research and development** Research and development costs include costs incurred in identifying, developing
consist primarily of payroll expenses for research related employees, laboratory costs, animal and lab maintenanc
and pre-clinical expenses, as well as payments for sponsored research, scientific and regulatory consulting fees an
as incurred. Costs for certain development activities are recognized based on an evaluation of the progress to com
information and data provided to us by our vendors and our clinical sites. When outside contracts for research pro
payments, they are recorded on the balance sheet as a prepaid item and expensed when the service is provided or
the contract. Advance payments related to research and development were $614 and $350, at March 31, 2014 and
other current assets on the balance sheets.

(3) Stock Option Plans:

On September 18, 2013, the Company s board of directors approved a grant of 372 incentive stock options and 31 nonqualif
2011 Stock Incentive Plan. Effective upon the closing of the Company s sale of shares of Series B-3 preferred stock on Novem
approved an amendment to the 2011 Stock Incentive Plan to increase the total number of shares available for iss

Upon the effectiveness on March 26, 2014 of the Company s registration statement on Form S-1 relating to its IPO, the Com
Equity and Incentive Plan. The total number of shares available for issue under the 2013 Equity and Incentive plan is 1,151
approved a grant of 100 incentive stock options and 56 nonqualified stock options under this plan on M

(a) Incentive stock options Incentive stock options are granted to employees at the discretion of the board. The exe
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 d

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Incentive stock options

A summary of the employee option activity is as follows:

		Nine Mon 2013 Weighte Averag Exercis
	Shares	**Price**
Outstanding, June 30	69	$ 3.5
Granted	192	0.3
Exercised		
Terminated		
Outstanding, March 31	261	$ 1.1
Exercisable, March 31	71	
Weighted average fair value of options granted during the period	$ 0.21	

As of March 31, 2014 and June 30, 2013, there was approximately $1,542 and $30, respectively of total unrecognized com
share-based compensation arrangements granted under the Company s stock incentive pla

Nonqualified stock options issued to non-employees

A summary of non-employee option activity follows:

		Nine Mon 2013 Weighte Averag Exercis
	Shares	**Price**

Outstanding, June 30	64	$ 3.5
Granted	54	0.3
Exercised		
Terminated		
Outstanding, March 31	118	$ 2.0
Exercisable, March 31	62	
Weighted average fair value of options granted during the period	$ 0.21	

In accounting for stock options to non-employees, the value of goods and services related to the options granted is recognized consistent with receipt of services. Therefore, vested portions vary based upon services and terms of each option. The Compar options each reporting period using the estimated fair value of the Company s common stock as of the last day

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(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 ar recurring basis:

Description	Total	Quoted prices in active markets (Level 1)	Significant observable i (Level 2
Assets:			
June 30, 2013			
Short-term investments	$ 14,000	$	$ 14
March 31, 2014			
Short-term investments	$ 16,500	$	$ 16
Liabilities:			
June 30, 2013			
Series B purchase rights	$ 2,096	$	$
Warrant liabilities	110		
Total	$ 2,206	$	$
March 31, 2014			
Series B purchase rights	$	$	$
Warrant liabilities	551		
Total	$ 551	$	$

Short-term investments Short-term investments consist of certificates of deposit placed through an account registry servi which the fair market value is measured based on level 2 inputs (quoted prices for identical assets in markets

Warrant liabilities The fair value of the warrants on the date of issuance, and on each financial reporting date for those war using the Black-Scholes option pricing model. The significant assumptions used in preparing the option pricing model for val

Assumption

	Nine Mon March
Exercise price	$0.1297 t
Fair value of preferred shares	$0.23 t
Expected life (in years)	0.12 t
Risk-free interest rate	0.01% t
Expected volatility	70.00% t

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 entered into a First Amendment to Series B-1, B-2 and B-3 Preferred Stock Purchase Agreement (the Series B Amendmen Company could sell Series B-3 Shares. In November 2013, the Company issued and sold an aggregate of 58,817 shares of Se share of $0.1823. The Series B

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Amendment provides that if the two remaining milestones specified in the Series B Purchase Agreement entered into in Nov Company by September 2014, Series B holders who still hold their Series B-3 shares will be entitled to receive up to an agg Series B-3 preferred stock. The automatic conversion of the Company s preferred stock to common stock upon the consumma this right. During the nine months ended March 31, 2014, a change in value of the Series B purchase right liability of $2,838 $4,934 allocated to the Series B-3 purchase right immediately prior to the closing of the third tranche was reallocated to the car stock on the Company s balance sheet.

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

Assumption	Nine Mont March 3
Exercise price	$ 0.1485 to
Years to maturity	0.00 to
Risk-free interest rate	0.06% to
Volatility	55.00% to

The Company reports the change in fair value during each period as a non-operating gain or loss recorded as a component o statement of operations. The table presented below is a summary of changes in the fair value of the Company s Level 3 valua purchase rights for the fiscal year ended June 30, 2013 and the nine months ended March 31,

Beginning balance as of July 1, 2012	
Fair value of warrants issued	
Fair value of Series B purchase rights issued	
Change in fair value during the period	
Series B purchase rights converted to Series B-2 convertible preferred stock	
Ending balance as of June 30, 2013	
Change in fair value during the period	
Series B purchase rights converted to Series B-3 convertible preferred stock	
Ending balance as of March 31, 2014	

(5) Accrued Expenses:

Accrued expenses consist of the following:

	June 30, 201
Research and development-related	$ 61
Compensation-related	298
	$ 359

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(6) Subsequent Events:

The Company has completed an evaluation of all subsequent events through July 21, 2014, to ensure appropriate disclosure of statements as of March 31, 2014, and events which occurred subsequently but were not recognized in the fi

On April 1, 2014, the Company completed its IPO whereby the Company sold 4,167 shares of common stock at a price of $12 on the Nasdaq Global Select Market on March 27, 2014. The aggregate net proceeds received by the Company from the offer discounts and commissions and estimated offering expenses payable by the Company. Upon the closing of the IPO, all outsta stock converted into 9,120 shares of common stock; and warrants exercisable for convertible preferred stock were automatical for 50 shares of common stock, resulting in the reclassification of the related convertible preferred stock warrant liability o

On April 3, 2014, the Company sold 625 shares of common stock pursuant to the full exercise of an overallotment option gra with the IPO. The aggregate net proceeds received by the Company were $6,975, net of underwriting discou

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Applied Genetic Technologies Corporation

2,000,000 Shares

Common Stock

Prospectus

July 24, 2014

BMO Capital Markets Stifel Wedbush PacGrow Life Sc

Cantor Fitzgerald & Co. Roth Capital Partners