UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-K/A

(Amendment No. 1)

(Mark One)

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

For the fiscal year ended December 31, 2014

Or

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

For the transition period from to

Commission file number 001-36080

OPHTHOTECH CORPORATION

(Exact name of registrant as specified in its charter)

Delaware	**20-8185347**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
One Penn Plaza, 19th Floor New York, NY	**10119**
(Address of principal executive offices)	(Zip Code)

(212) 845-8200

(Registrant s telephone number, including area code)

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

Title of each class	**Name of each exchange on which registered**
Common Stock, $0.001 par value	The NASDAQ Stock Market LLC

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

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

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

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate website, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). x Yes o No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant s knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. o

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

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

As of June 30, 2014, the aggregate market value of the voting and non- voting common equity held by non- affiliates of the registrant was approximately $942.5 million, based on the closing price of the registrant s common stock on June 30, 2014.

The number of shares outstanding of the registrant s class of common stock, as of February 23, 2015: 34,174,090

DOCUMENTS INCORPORATED BY REFERENCE

Part III of this Annual Report incorporates by reference information from the definitive Proxy Statement for the registrant s 2015 Annual Meeting of Shareholders, which was filed with the Securities and Exchange Commission on April 30, 2015.

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EXPLANATORY NOTE

We are filing this Amendment to our Annual Report on Form 10-K/A (the Amended Report), which was originally filed with the Securities and Exchange Commission on Form 10-K on March 2, 2015 (the Original Report or Annual Report on Form 10-K), to reflect a restatement of our financial statements.

As discussed in Note 18 to our financial statements, subsequent to the issuance of the Original Report, we and the Audit Committee of our Board of Directors concluded that we should restate our balance sheets as of June 30, 2014, September 30, 2014 and December 31, 2014, and our statements of operations, comprehensive loss, cash flows and changes in stockholder s equity (deficit) for the periods ended June 30, 2014, September 30, 2014 and December 31, 2014 to correct the following errors:

- In the second quarter of 2014, we recorded an income tax benefit by reducing a portion of our valuation allowance against our gross deferred tax assets. In determining the amount of the valuation allowance release, we considered anticipated 2015 tax losses which would generate a refund of a portion of federal income taxes paid in 2014. We have now determined that, as a matter of accounting principle, the net deferred tax asset recorded on our balance sheets was overstated and the income tax provision on our statements of operations was understated as of and for the periods ended June 30, 2014, September 30, 2014 and December 31, 2014. Our cash position and operating expenses were not affected.

For the reasons discussed above, we are filing this Amended Report in order to amend our financial statements and the following items in Part II of the Original Report to the extent necessary to correct the errors discussed above and make corresponding revisions to our financial information cited elsewhere in this Amended Report:

- Item 6. Selected Financial Data
- Item 7. Management s Discussion and Analysis of Financial Condition and Results of Operations
- Item 8. Financial Statements and Supplementary Data
- Item 9A. Controls and Procedures

In addition, in accordance with applicable Securities and Exchange Commission rules, this Amended Report includes updated certifications from our chief executive officer and chief financial officer as Exhibits 31.3, 31.4, 32.3 and 32.4, as well as an updated consent from our independent registered public accounting firm, Ernst & Young LLP. Other than minor conforming revisions made to the financial information contained in Part I, 1A. Risk Factors, none of the remaining Items of our Original Report are amended hereby. Items that have not been amended have been omitted herefrom.

In order to preserve the nature and character of the disclosures set forth in the Original Report, except as expressly noted above, this Amended Report speaks as of the date of the filing of the Original Report, March 2, 2015, and we have not updated the disclosures in the Amended Report to speak as of a later date. All information contained in this Amended Report is subject to updating and supplementing as provided in our reports filed with the Securities and Exchange Commission subsequent to the date of the Original Report.

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FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K/A contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Annual Report on Form 10-K/A, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words anticipate, believe, goals, estimate, expect, intend, may, might, plan, predict, project, target, po should, continue and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this Annual Report on Form 10-K/A include, among other things, statements about:

- the timing, costs, conduct and outcome of our Phase 3 clinical trials of Fovista and other clinical trials of Fovista, in each case administered in combination with anti-VEGF drugs for the treatment of wet age-related macular degeneration, or AMD, including statements regarding the timing and the availability of, and the costs to obtain, initial top-line results from, and the completion of such trials and the timing of regulatory filings;

- the timing, costs, conduct and outcome of our planned trials for Zimura for the treatment of patients with geographic atrophy, a form of dry AMD and, in combination with anti-VEGF therapy and, potentially, Fovista, for the treatment of certain forms of wet AMD, including statements regarding the timing of the initiation of, and the costs to obtain and timing of receipt of initial results from, and the completion of related clinical trials;

- the timing, costs, conduct and outcome of our planned pre-clinical work for an ophthalmic formulation of tivozanib, including statements regarding the timing of the initiation of, and the costs to obtain and timing of receipt of results from, such work;

- the timing of and our ability to obtain marketing approval of Fovista, Zimura and other product candidates we may develop, and the ability of Fovista, Zimura and other product candidates we may develop to meet existing or future regulatory standards;

- our ability to maintain a productive collaborative relationship with Novartis Pharma AG, including our ability to achieve remaining potential milestone payments under our agreement;

- the potential advantages of Fovista and Zimura;

- the rate and degree of potential market acceptance and clinical utility of Fovista and Zimura;

- our estimates regarding the potential market opportunity for Fovista and Zimura;

- the potential receipt of revenues from future sales of Fovista and Zimura;

- our sales, marketing and distribution capabilities and strategy;

- our ability to establish and maintain arrangements for manufacture of Fovista, Zimura and other product candidates we may develop;

- our ability to in-license or acquire complementary products, product candidates or technologies;

- our intellectual property position;

- our expectations related to our use of available cash;

- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;

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- the impact of existing and new governmental laws and regulations; and
- our competitive position.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and our stockholders should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Annual Report on Form 10-K/A, particularly in the Risk Factors section, that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this Annual Report on Form 10-K/A and the documents that we have filed as exhibits to this Annual Report on Form 10-K/A completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Annual Report on Form 10-K/A are made as of the date of the Original Report, and we do not assume any obligation to update any forward- looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

PART I

Item 1A. Risk Factors.

The following risk factors and other information included in this Annual Report on Form 10-K/A should be carefully considered. The risks and uncertainties described below are not the only risks and uncertainties we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. Please see page 1 of this Annual Report on Form 10-K/A for a discussion of some of the forward-looking statements that are qualified by these risk factors. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected.

Risks Related to Our Financial Position and Need for Additional Capital

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

We were incorporated and commenced active operations in 2007. Our operations to date have been limited to organizing and staffing our company, acquiring rights to product candidates, business planning, raising capital and developing Fovista, Zimura and other product candidates.

We have not yet demonstrated our ability to successfully complete a large-scale, pivotal clinical trial, obtain marketing approval, manufacture at commercial scale, or arrange for a third party to do so on our behalf, or conduct sales, marketing and distribution activities necessary for successful product commercialization. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a product development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We have incurred significant operating losses since our inception. We expect to incur losses for at least the next several years and may never achieve or maintain profitability.

Since inception, we have experienced significant cash outflows in funding our operations. Our net loss was $116.8 million for the year ended December 31, 2014 and $51.1 million for the year ended December 31, 2013. As of December 31, 2014, we had an accumulated deficit of $299.8 million. To date, we have not generated any revenues from product sales and have financed our operations primarily through private placements of our preferred stock, venture debt borrowings, funds received under our royalty purchase and sale agreement with Novo A/S, which we refer to as the Novo Agreement, our initial public offering, which we closed in September 2013, our follow-on public offering, which we closed in February 2014 and funds we received under the Novartis Agreement, which we entered into in May 2014.

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We have devoted substantially all of our financial resources and efforts to research and development. We expect to continue to incur significant expenses and increasing operating losses over the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year.

Our product candidates, Fovista and Zimura, are in clinical development. We expect our expenses to continue to increase, particularly as we continue the development of Fovista in our Phase 3 clinical program and other additional clinical trials for the treatment of wet AMD. We initiated our pivotal Phase 3 clinical program for Fovista in August 2013. We plan to enroll a total of 1,866 patients for this program. In addition, we also expect our expenses to increase as we further evaluate the potential benefit of Fovista in wet AMD, when administered in combination with anti-VEGF drugs, and in other ophthalmic diseases and conditions with unmet need and pursue the development of Zimura for the treatment of geographic atrophy, a form of dry AMD and in combination with anti-VEGF therapy and, potentially, Fovista, for the treatment of anti-VEGF resistant wet AMD patients who are believed to have complement mediated inflammation. We expect these expenses to increase as patient enrollment increases. In addition, our expenses will increase prior to obtaining marketing approval for Fovista as we expand our infrastructure to support commercial operations and if we obtain marketing approval for Fovista, Zimura or any other product candidate that we develop, we expect our commercialization expenses related to product sales, marketing, distribution and manufacturing to increase significantly. We are party to agreements, specifically an asset acquisition agreement with OSI (Eyetech), Inc., which agreement is now held by OSI Pharmaceuticals, LLC, a subsidiary of Astellas US, LLC, and license agreements with Archemix Corp., and Nektar Therapeutics, that impose significant milestone payment obligations on us in connection with our achievement of specific clinical, regulatory and commercial milestones with respect to Fovista and Zimura. Furthermore, we expect to incur additional costs associated with being a public company, including legal, compliance, accounting and investor and public relations expenses, as well as increased insurance premiums.

Our expenses also will increase if and as we:

- undertake additional clinical development of Fovista, if it is approved, in support of our efforts to broaden the label for Fovista;
- conduct additional clinical trials of Zimura that will be required for us to seek marketing approval of Zimura for the treatment of geographic atrophy and/or wet AMD (including a second Phase 3 study);
- undertake pre-clinical and clinical development of tivozanib;
- in-license or acquire the rights to other complementary products, product candidates or technologies, including drug delivery technology, for the treatment of ophthalmic diseases and pursue pre-clinical and clinical development of such product candidates or technologies;
- seek marketing approval for any product candidates that successfully complete clinical trials;

- expand our outsourced manufacturing activities and establish sales, marketing and distribution capabilities, if we receive, or expect to receive, marketing approval for any of our product candidates;

- maintain, expand and protect our intellectual property portfolio;

- hire additional clinical, quality control and scientific personnel; and

- add operational, financial and management information systems and personnel, including personnel to support our clinical, manufacturing and planned future commercialization efforts.

If we are required by the U.S. Food and Drug Administration, or FDA, the European Medicines Agency, or EMA, or regulatory authorities in other jurisdictions to perform clinical or nonclinical trials or other studies in addition to those we currently expect to conduct, or if there are any delays in completing the clinical trials of Fovista or Zimura, or the development of any of other product candidates that we may develop, our expenses could increase. Our costs will also increase if we increase our investigator fees for our clinical trials or expand the scope of our clinical trials and programs, including, for example, by changing the geographic mix of sites at which patients are enrolled, or to increase other corporate or licensing activities or staffing.

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Our ability to become and remain profitable depends on our ability to generate revenue in excess of our expenses. We do not expect to generate and maintain significant revenue from product sales unless, and until, we obtain marketing approval for, and commercialize, Fovista, Zimura or other product candidates that we may develop. Our capital requirements will depend on many factors, including the success of our development and commercialization of our product candidates and whether we pursue the acquisition or in-licensing and subsequent development of additional product candidates. Even if we succeed in developing and commercializing one or more of our product candidates, we may never achieve sufficient sales revenue to achieve or maintain profitability. Our ability to commercialize our product candidates, in particular Fovista, will require us to be successful in a range of challenging activities, including:

- obtaining favorable results from our Phase 3 clinical program for Fovista;
- obtaining favorable results, especially with respect to safety, in our other planned clinical trials involving Fovista;
- subject to obtaining favorable results from our Phase 3 clinical program, applying for and obtaining marketing approval for Fovista;
- establishing sales, marketing and distribution capabilities to effectively market and sell Fovista in the United States with our own specialty sales force targeting retinal specialists;
- successfully maintaining our arrangement with Novartis to commercialize Fovista in markets outside the United States;
- obtaining adequate coverage and reimbursement for our product candidates, if approved, from governmental and third-party payors;
- protecting our rights to our intellectual property portfolio related to Fovista; and
- ensuring the manufacture of commercial quantities of Fovista.

We may never succeed in these activities and, even if we do, may never generate revenues from product sales that are significant enough to achieve profitability. In addition, our profitability will depend, in part, on our commercialization partners ability, including Novartis s ability, to effectively market and sell Fovista, Zimura or other product candidates we may develop, if approved outside the United States, and to obtain adequate coverage and reimbursement of such product candidates from governmental and third party payors. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or continue our operations. A decline in the value of our company could also cause our stockholders to lose all or part of their investment.

We have broad discretion in the use of our available cash and other sources of funding and we may not use them effectively.

Our management has broad discretion in the use of our available cash and other sources of funding and could spend those resources in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest our available cash in a manner that does not produce adequate income, if any, or that loses value.

We may need additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

We expect our expenses to increase substantially, particularly as we continue the development of Fovista in our Phase 3 clinical program for the treatment of wet AMD. We plan to enroll a total of 1,866 patients for this program. In addition, we also expect our expenses to increase as we further evaluate the potential benefit of Fovista in wet AMD, when administered in combination with anti-VEGF drugs, and in other ophthalmic diseases and conditions with unmet need, pursue the development of Zimura for the treatment of geographic atrophy, a form of dry AMD and in combination with anti-VEGF therapy and, potentially, Fovista, for the treatment of anti-VEGF resistant wet AMD patients who are believed to have complement mediated inflammation. In addition, our expenses will increase prior to obtaining marketing approval for Fovista as we manufacture validation production batches of API and drug product for Fovista and as we expand our infrastructure to support commercial operations, and if we obtain marketing approval for Fovista, Zimura or any other product candidate that we develop, we expect our commercialization expenses in the United States with regard to Fovista and worldwide with regards to other product candidates, related to product sales, marketing, distribution and manufacturing to increase significantly. Our expenses will increase if we suffer any delays in our Phase 3 clinical program for Fovista, including delays in receipt of regulatory clearance to begin our Phase 3 clinical trials in jurisdictions where clearance is required but not yet obtained, or delays in enrollment of patients. Furthermore, we expect to incur additional costs associated with being a public company, hiring additional personnel and expanding our facilities. Accordingly, we may need to obtain additional funding in connection with our continuing operations prior to attaining profitability. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

As of December 31, 2014, we had cash, cash equivalents, and marketable securities of $463.6 million and $351.2 million in total liabilities, including liabilities of $334.6 million relating to the Novo Agreement and deferred revenue associated with the Novartis Agreement.

We believe that our cash, cash equivalents and marketable securities, together with the receipt of the potential remaining $80.0 million enrollment-based milestone payments under Novartis Agreement, will be sufficient to fund our operations and capital expenditure requirements, as currently planned and including the expansion of our infrastructure to support commercial operations, through the end of 2017. Our capital requirements will also depend on other factors, including the success of our development and commercialization of our product candidates and whether we pursue the acquisition or in-licensing and subsequent development of additional product candidates.

We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Costs related to our clinical programs could exceed our expectations if we experience delays in our clinical trials, including because of the timing of our patient enrollment, the availability of drug supply for our clinical trials or for other reasons. Our costs will also increase if we increase investigator fees for our clinical trials or expand the scope of our clinical trials and programs, including, for example, by changing the geographic mix of sites at which patients are enrolled, or if we decide to increase other corporate or licensing activities or staffing, or if we experience issues with the process development and scale up of manufacturing activities.

Our current Phase 3 clinical program for Fovista is expected to continue through at least 2017, and substantial expenditures to complete the Phase 3 clinical program will be required after the receipt of initial, top-line data, which we expect in 2016. Moreover, we are at the early stages of formulating our clinical development plan for Zimura. We expect the clinical development of Zimura will continue for at least the next several years. At this time, we cannot reasonably estimate the remaining costs necessary to complete the clinical development of either Fovista or Zimura, complete process development and manufacturing scale-up activities associated with Fovista and Zimura and potentially seek marketing approval for Fovista or Zimura, or the nature, timing or costs of the efforts necessary to complete the development of any other product candidate we may develop.

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

- the scope, progress, costs and results of our Phase 3 clinical program for Fovista;

- the progress, costs and results of our planned additional clinical trials to further evaluate the potential benefit of Fovista in wet AMD, when administered in combination with anti-VEGF drugs, and in other ophthalmic diseases and conditions with unmet need;

- the scope, progress, results and costs of (i) our planned Phase 2/3 clinical trial evaluating Zimura for the treatment of geographic atrophy and additional clinical trials (including an additional Phase 3 trial) required by regulatory authorities for us to seek marketing approval in this indication, (ii) our very small Phase 2 clinical trial evaluating Zimura in combination with anti-VEGF therapy for the treatment of polypoidal choroidal vasculopathy, a specific type of wet AMD, in patients who do not respond adequately to treatment with anti-VEGF monotherapy or for whom anti-VEGF monotherapy fails, and (iii) our planned Phase 2 clinical trial evaluating Zimura in combination with anti-VEGF therapy and Fovista, for the treatment of anti-VEGF resistant wet AMD patients who are believed to have complement mediated inflammation;

- the costs and timing of process development and manufacturing scale-up activities associated with Fovista and Zimura;

- the costs, timing and outcome of regulatory review of Fovista and Zimura;

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- the costs of commercialization activities for Fovista or Zimura if we receive, or expect to receive, marketing approval for either product candidate, including the costs and timing of expanding our outsourced manufacturing activities and establishing product sales, marketing and distribution capabilities;

- subject to receipt of marketing approval, revenue received from commercial sales of Fovista or Zimura, after milestone payments and royalties;

- the scope, progress, results and costs of our clinical trials for any other product candidates that we may acquire or in-license and subsequently develop;

- our ability to establish additional collaborations on favorable terms, if at all;

- the scope, progress and results of our pre-clinical and clinical plans for tivozanib;

- the extent to which we in-license or acquire rights to complementary products, product candidates or technologies; and

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

Our commercial revenues, if any, will be derived from sales of Fovista, Zimura or any other products that we successfully develop, none of which we expect to be commercially available for several years, if at all. In addition, if approved, Fovista or Zimura or any product that we acquire or in-license may not achieve commercial success. If that is the case, we may need to obtain substantial additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans.

If we fail to enroll patients in our Phase 3 clinical trials of Fovista as planned or fail to comply with our obligations in the Novartis Agreement, we could lose access to funds that are important to our business, which may force us to delay or terminate the development of Fovista. In addition, a default under the Novo Agreement would permit Novo A/S to foreclose on the Fovista intellectual property.

In May 2014, we entered into the Novartis Agreement. Among other payments, Novartis is obligated under the agreement to pay us up to an aggregate of $130.0 million if we achieve specified patient enrollment milestones for our ongoing pivotal Phase 3 clinical program for Fovista, $50.0 million of which we received in October 2014. We are subject to diligence and other obligations under the Novartis Agreement. If we fail to enroll the specified numbers of patients in our Phase 3 clinical trials of Fovista or fail to satisfy our other obligations, we may fail to trigger the remaining enrollment-based milestone payments. This could limit our ability to continue the development programs for our product candidates. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay or terminate our research and development programs, including those for Fovista, or any future commercialization efforts.

We are also subject to diligence and other obligations under the Novo Agreement. Our obligations under the Novo Agreement are secured by collateral, which includes certain intellectual property rights, including all of our intellectual property rights relating to Fovista and regulatory approvals, if any, of Fovista. If we fail to satisfy our diligence obligations or breach any other of our obligations under the Novo Agreement and fail to cure the breach within any applicable grace period, Novo A/S could declare an event of default. In such event, Novo A/S could seek to foreclose on the collateral securing our obligations. If Novo A/S successfully does so, we would lose our rights to develop and commercialize Fovista.

Our obligations under the Novo Agreement and the pledge of our intellectual property rights in and regulatory approvals, if any, of Fovista as collateral under such agreement may limit our ability to obtain debt financing.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our capital needs through a combination of our current cash, cash equivalents, and marketable securities balances, potential milestone payments under collaborations, strategic alliances and marketing, distribution or licensing arrangements, and equity offerings and debt financings. The remaining potential milestone payments under the Novartis Agreement are subject to our achievement of specified clinical, regulatory and commercial events related to Fovista. We do not have any other committed external source of funds besides the Novartis Agreement. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our existing stockholders rights as holders of our common stock. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Our pledge of assets, including intellectual property rights, as collateral to secure our obligations under the Novo Agreement may limit our ability to obtain debt financing.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, products or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves.

Risks Related to Product Development and Commercialization

We depend heavily on the success of our lead product candidate, Fovista, which we are developing to be administered in combination with anti-VEGF drugs for the treatment of patients with wet AMD. In addition, we also depend on the success of Zimura, which we are developing for the treatment of geographic atrophy, a form of dry AMD, and for the treatment of anti-VEGF resistant wet AMD patients who are believed to have complement-mediated inflammation. If we are unable to complete the clinical development of either of these product candidates, if we are unable to obtain marketing approvals for either of these product candidates, or if either of these product candidates is approved and we or our commercialization partner for Fovista outside the United States, Novartis, fail to successfully commercialize the product candidate or experience significant delays in doing so, our business will be materially harmed.

We have invested and will continue to invest a significant portion of our efforts and financial resources in the development of Fovista to be administered in combination with anti-VEGF drugs for the treatment of patients with wet AMD. There remains a significant risk that we will fail to successfully develop Fovista. The results of our Phase 2b clinical trial may not be predictive of the results of our Phase 3 clinical program due, in part, to the fact that we have no clinical data on Fovista combination therapy in any clinical trial longer than 24 weeks, that we have modified the methodology used to determine a patient s eligibility under certain of the inclusion and exclusion criteria for our Phase 3 clinical trials as compared to our Phase 2b clinical trial, that we have very limited clinical data on the effects of Fovista when administered in combination with Avastin or Eylea and that we plan to conduct our Phase 3 clinical trials at many clinical centers that were not included in our Phase 2b clinical trial.

We do not expect to have initial, top-line data from our Phase 3 clinical program for Fovista until 2016. The timing of the availability of such top-line data and the completion of our Phase 3 clinical program is dependent, in part, on our ability to locate and enroll a sufficient number of eligible patients in our Phase 3 clinical program on a timely basis. The timing of the availability of initial, top-line data from our Phase 3 clinical trial evaluating the safety and efficacy of Fovista administered in combination with each of Avastin or Eylea may be subject to particular variability because, prior to the initiation of our Phase 3 clinical program, we had no clinical experience testing Fovista administered in combination with Avastin or Eylea. Avastin is not approved for intravitreal use in treating wet AMD, and regulatory authorities in certain countries may not allow, or physicians and patients may choose not to participate in, a clinical trial in which Avastin is administered in combination with Fovista for the treatment of wet AMD. Even if we ultimately obtain statistically significant, positive results from our Phase 3 clinical program, it is possible that such data may not be clinically relevant.

If we are not able to obtain data from our Phase 3 clinical trial evaluating Fovista administered in combination with each of Avastin or Eylea when data from our other two Phase 3 clinical trials evaluating Fovista administered in combination with Lucentis are available, we may nonetheless decide to proceed with submitting applications for marketing approval for Fovista administered only in combination with Lucentis, or we may choose to delay our application for marketing approval until data from all three Phase 3 clinical trials are available. If we submit applications for marketing approval for Fovista only in combination with Lucentis, we may determine either to delay seeking approval of Fovista in combination with Avastin or Eylea until after regulatory authorities have considered and acted on our applications for Fovista in combination with Lucentis, or to amend our applications once data from our third Phase 3 clinical trial become available. If we were to delay seeking approval of Fovista in combination with Avastin or Eylea pending regulatory action on our applications for Fovista in combination with Lucentis, the FDA or other regulatory authorities could defer taking action on our applications while data remain outstanding from our third Phase 3 clinical trial. Moreover, if we subsequently amend our applications for marketing approval when data

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from our third Phase 3 clinical trial become available, we may experience further delays in our application process. The manner in which we seek marketing approval may differ in the United States and in the European Union. Additionally, we expect that our Phase 3 clinical trials will continue in accordance with their protocols after we submit applications for marketing approval, and the conclusions of those trials may yield data that are inconsistent with the initial data used to support our applications. Furthermore, we expect to commence additional clinical trials to further evaluate the potential benefit of Fovista in wet AMD, when administered in combination with anti-VEGF drugs, and in other ophthalmic diseases and conditions with unmet medical need during the course of our ongoing Phase 3 clinical development program, and to evaluate Zimura, in combination with anti-VEGF therapy and, potentially, Fovista, for the treatment of anti-VEGF resistant wet AMD patients who are believed to have complement mediated inflammation. We are also supplying Fovista for third-party sponsored clinical trials. In addition, Novartis may commence additional preclinical and clinical trials for Fovista including those which it deems necessary for regulatory and/or reimbursement approvals outside of the United States. Adverse safety events or negative or inconclusive efficacy results in any of these trials may impact the progress of our Phase 3 clinical program, including our ability to receive marketing approval, and, if such data is received following a potential approval, our future sales of Fovista. As a result of these and other factors, we cannot accurately predict when or if Fovista will prove effective or safe in humans or will receive marketing approval.

In addition, we have invested substantial financial resources in the development of Zimura for the treatment of patients with both dry and wet AMD. There remains a significant risk that we will fail to successfully develop Zimura. We have very limited data from our completed Phase 2a clinical trial evaluating the safety and effectiveness of Zimura for the treatment of dry AMD and our completed Phase 2a clinical trial evaluating the safety and effectiveness of Zimura administered in combination with Lucentis for the treatment of wet AMD. These trials enrolled 47 patients and 60 patients, respectively, and neither trial included a control arm. Furthermore, we have no preclinical or clinical data on the effects of Zimura when administered in combination with both Fovista and an anti-VEGF drug.

The timing of the completion of and the availability of initial results from these planned clinical trials is difficult to predict and is dependent, in part, on our ability to complete manufacturing scale-up activities and to locate and enroll a sufficient number of eligible patients in our planned trials on a timely basis. The timing of the receipt of initial results from our Phase 2 clinical trial evaluating the safety and efficacy of Zimura, administered in combination with anti-VEGF therapy, and potentially, Fovista, may be subject to particular variability because we have no clinical experience testing Zimura administered in combination with Fovista and an anti-VEGF drug.

Although our current development plan for Zimura calls for us to initiate a Phase 2/3 clinical trial evaluating the safety and efficacy of Zimura in treating patients with geographic atrophy, we may not initiate or complete this clinical trial for Zimura or any other clinical trial for Fovista, Zimura or any other product candidates that we may develop in accordance with our plans.

Although our plans for additional clinical trials reflect our current expectations regarding the endpoints, duration and number of patients to be included in these trials, we have not had formal meetings with regulatory authorities regarding our trial designs. Our plans may change significantly based on feedback we may receive from such regulatory authorities.

Our ability to generate revenues from product sales, which we do not expect will occur before 2017, if ever, will depend heavily on our obtaining marketing approval for and commercializing our product candidates, and in particular, Fovista and Zimura. The success of these product candidates will depend on several factors, including the following:

- obtaining favorable results from clinical trials;

- making arrangements with third-party manufacturers and receiving regulatory approval of our manufacturing processes and our third-party manufacturers facilities from applicable regulatory authorities;

- for Fovista, receipt of marketing approvals from applicable regulatory authorities for the use of Fovista in combination with anti-VEGF drugs for the treatment of wet AMD, and in particular, which anti-VEGF drugs are included in any such approval given that Avastin, one of the current standard of care anti-VEGF drugs, is not approved for intravitreal use;

- for Zimura, receipt of marketing approvals from applicable regulatory authorities for the use of Zimura for the treatment of dry AMD or the use of Zimura, administered in combination with anti-VEGF therapy and, potentially, Fovista for the treatment of wet AMD;

- the scope of the label that may be approved by applicable regulatory authorities, including the specific indication for which the product may be approved;

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- launching commercial sales of the product candidate, if and when approved, whether alone or in collaboration with others, including Novartis for Fovista;

- acceptance of the product candidate, if and when approved, by patients, the medical community and third-party payors;

- for Fovista, continued, widespread use of anti-VEGF therapies in the treatment of wet AMD in combination with which Fovista will be used;

- effectively competing with other therapies, including the existing standard of care, and other forms of drug delivery;

- maintaining a continued acceptable safety profile of the product candidate following approval;

- obtaining and maintaining patent and trade secret protection and regulatory exclusivity; and

- protecting our rights in our intellectual property portfolio.

Successful development of Fovista for the further treatment of wet AMD, the treatment of additional ophthalmic conditions, if any, or for use in other patient populations and our ability, if it is approved, to broaden the label for Fovista will depend on similar factors.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize Fovista, Zimura or any other product candidates that we may develop, which would materially harm our business.

If clinical trials of Fovista, Zimura or any other product candidate that we may develop fail to demonstrate safety and efficacy to the satisfaction of the FDA, the EMA or other regulatory authorities or do not otherwise produce positive or supportive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of Fovista, Zimura or any other product candidate.

Before obtaining approval from regulatory authorities for the sale of any product candidate, we must conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

Our Phase 2b clinical trial evaluated a combination of Fovista and Lucentis. In this trial, patients treated with a combination of 0.3 mg of Fovista and Lucentis did not achieve statistically significant superiority compared to Lucentis monotherapy based on the pre-specified primary endpoint of mean change in visual acuity from baseline at the 24 week time point. Although a combination of 1.5 mg of Fovista and Lucentis demonstrated statistically significant superiority in this trial compared to Lucentis monotherapy based on the pre-specified primary endpoint of mean change in visual acuity from baseline at the 24 week time point, we may nonetheless fail to achieve success in our Phase 3 clinical trials involving a combination of 1.5 mg of Fovista and Lucentis for a variety of potential reasons.

- The primary endpoint of mean change in visual acuity in our Phase 2b clinical trial was measured 24 weeks after the first dose of Fovista. The primary endpoint of mean change in visual acuity in our Phase 3 clinical program will be measured 12 months after the first dose of Fovista. We have no clinical data on Fovista combination therapy in any clinical trial longer than 24 weeks. We have modified the methodology used to determine a patient s eligibility under certain of the inclusion and exclusion criteria for our Phase 3 clinical trials as compared to our Phase 2b clinical trial. If the positive results we observed at 24 weeks in our Phase 2b clinical trial are not observed at 12 months, we likely will not receive marketing approval for Fovista.

- Retrospective subgroup analyses that we performed on the results of our Phase 2b clinical trial may not be predictive of the results of our Phase 3 clinical program. While we believe that our retrospective analyses further support the results from our primary endpoint and our proposed mechanisms of action, retrospective analyses performed after unmasking trial results can result in the introduction of bias and are given less weight by regulatory authorities than pre-specified analyses. In particular, our proposed mechanism of action as it relates to the inhibition of subretinal fibrosis, although scientifically rational and while supported by retrospective subgroup

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analysis, may not be supported by our future clinical trials. Our belief regarding Fovista s potential, when administered in combination with an anti-VEGF drug, to inhibit subretinal fibrosis and retinal scarring, may change based on our subsequent clinical trials or other factors.

- We are conducting our Phase 3 clinical trials at many clinical centers that were not included in our Phase 2b clinical trial. The introduction of new centers, and the resulting involvement of new treating physicians, can introduce additional variability into the conduct of the trials in accordance with their protocols and may result in greater variability of patient outcomes, which could adversely affect our ability to detect statistically significant differences between patients treated with 1.5 mg of Fovista administered in combination with an anti-VEGF drug and anti-VEGF drug monotherapy.

Furthermore, our Phase 3 clinical program involves two Phase 3 clinical trials testing a combination of 1.5 mg of Fovista and Lucentis for the treatment of wet AMD and one trial testing a combination of 1.5 mg of Fovista with each of Avastin or Eylea for the treatment of wet AMD. We have very limited clinical data on the effects of Fovista when administered in combination with intraocular injections of either Avastin or Eylea for the treatment of patients with wet AMD. Avastin is not approved for such use.

Fovista administered in combination with Lucentis was generally well tolerated in our Phase 1 and Phase 2b clinical trials. However, the results of these clinical trials may not be predictive of the results of our Phase 3 clinical program for Fovista. We have clinical data for Fovista administered in combination with Lucentis from only these two studies with a limited follow-up of a maximum of 24 weeks. As compared to our Phase 2b clinical trial, our three Phase 3 studies are longer in duration (24 months) with a 12 month timepoint for the primary endpoint, have a greater number of patients (approximately 1866), have a greater number of sites (more than 225), which encompass a much larger geographical recruitment area, and result in chronic exposure to a higher rate of intraocular pressure due to an increased injection volume. Consequently, there is potential for an increase in cumulative side effects resulting from two separate intraocular injections and increased intraocular pressure in the Fovista combination therapy patients as compared to the patients receiving monotherapy anti-VEGF treatment and there is a much longer duration of therapy and greater geographic diversity of patients in our Phase 3 trials. This increase in the number of intraocular injections and treatment burden, increased variability of patient care due to the larger number of clinical trial sites and the broader genetic profile of the enrolled patients from a larger geographic region may result in increased susceptibility to side effects of Fovista and/or resulting from treatment procedure. Therefore there is the potential for an unfavorable safety and tolerability profile in the Fovista combination therapy arm of the study as compared to our Phase 2b study and monotherapy anti-VEGF studies which may be reflected in an increase in adverse events and/or serious adverse event rates (either ocular, systemic or both) in patients receiving Fovista combination therapy. For example, there may be, among others, an increase in the rates of intraocular infections, or endophthalmitis, intraocular pressure, glaucoma, retinal tears, cataracts, retinal detachment, intraocular inflammation, retinal and/or choroidal circulation compromise, cardiovascular disease such as myocardial infarctions, stroke, blood clots or emboli, or hospitalizations in the Fovista combination therapy patients.

In general, the FDA and similar regulatory authorities outside the United States require two adequate and well controlled clinical trials demonstrating safety and effectiveness for marketing approval. If a combination of 1.5 mg of Fovista and Lucentis fails to achieve superiority over Lucentis monotherapy with statistical significance on the primary endpoint of mean change in visual acuity from baseline at 12 months in both of our Phase 3 clinical trials evaluating the safety and efficacy of this combination, we likely will not receive marketing approval for Fovista even if the combination of 1.5 mg of Fovista with Avastin or Eylea achieves superiority over Avastin or Eylea monotherapy with statistical significance on the primary endpoint in one of our Phase 3 clinical trials. There are a variety of other possible outcomes of our Phase 3 clinical trials. As described below, positive outcomes in one or more of our Phase 3 clinical trials may not be sufficient for the FDA or similar regulatory authorities outside the United States to grant marketing approval for Fovista.

- If a combination of 1.5 mg of Fovista and Lucentis achieves superiority over Lucentis monotherapy with statistical significance on the primary endpoint in only one of our Phase 3 clinical trials and the combination of 1.5 mg of Fovista with Avastin or Eylea does not achieve superiority over Avastin or Eylea monotherapy with statistical significance on the primary endpoint in our other Phase 3 clinical trials, we likely will not receive marketing approval for Fovista.

- If a combination of 1.5 mg of Fovista and Lucentis achieves superiority over Lucentis monotherapy with statistical significance on the primary endpoint in only one of our Phase 3 clinical trials and the combination of 1.5 mg of Fovista with Avastin or Eylea achieves superiority over Avastin or Eylea monotherapy with statistical

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significance on the primary endpoint in our other Phase 3 clinical trial, the FDA or similar regulatory authorities outside the United States may nonetheless not grant marketing approval for Fovista.

- Even if a combination of 1.5 mg of Fovista and an anti-VEGF drug achieves superiority over an anti-VEGF drug monotherapy with statistical significance on the primary endpoint in two or all three of our Phase 3 clinical trials, the FDA or similar regulatory authorities outside the United States may nonetheless not grant marketing approval for Fovista if such regulatory authorities do not believe that the benefits offered by Fovista administered in combination with an anti-VEGF drug are clinically meaningful or that such benefits outweigh the observed or potential risks.

In the United States, Avastin and Eylea are widely used for the treatment of wet AMD. If a combination of 1.5 mg of Fovista with Avastin or Eylea does not achieve superiority over Avastin or Eylea monotherapy with statistical significance on the primary endpoint of mean change in visual acuity from baseline at 12 months in our Phase 3 clinical program, our ability to successfully commercialize Fovista in combination with any anti-VEGF drug could be harmed materially. In addition, any failure of Fovista administered in combination with Avastin or Eylea to achieve superiority over Avastin or Eylea monotherapy with statistical significance on the primary endpoint could cause the FDA or similar regulatory authorities outside the United States to require additional clinical trials or other research before granting marketing approval of Fovista for use in combination with any anti-VEGF drug, including Lucentis, for the treatment of patients with wet AMD. In addition, Avastin is not approved for use in treating wet AMD, either in the United States or outside of the United States, and regulatory authorities may not permit the product label for Fovista to include the use of Fovista in combination with Avastin if we were otherwise able to obtain marketing approval for Fovista for use in combination with other anti-VEGF drugs.

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The protocols for our Phase 3 clinical trials and other supporting information are subject to review by the FDA and regulatory authorities outside the United States. The FDA is not obligated to comment on our protocols within any specified time period or at all or to affirmatively clear or approve our Phase 3 clinical program. We submitted the protocols to the FDA for our two Phase 3 clinical trials investigating Fovista administered in combination with Lucentis in August 2013 and for our Phase 3 clinical trial investigating Fovista administered in combination with Avastin and Eylea in April 2014, and have initiated the three trials in our Phase 3 clinical program in the United States without waiting for any such comments. The FDA or other regulatory authorities may request additional information, require us to conduct additional non-clinical trials or require us to modify our proposed Phase 3 clinical program, including its endpoints, patient enrollment criteria or selection of anti-VEGF drugs, to receive clearance to initiate such program or to continue such program once initiated.

Outside the United States, we have made regulatory submissions in selected countries to initiate Phase 3 clinical trials of Fovista. We have obtained all but one of the necessary country approvals to proceed with the two trials evaluating Fovista administered in combination with Lucentis in those countries and substantially all of the necessary country approvals for the trial of Fovista administered in combination with Eylea and Avastin. In the European Union, as further described below, in addition to filing in selected countries with national competent authorities responsible for approving clinical trial applications, we have had interactions regarding our planned application for marketing approval with the EMA s CHMP, which is the committee responsible for preparing opinions on questions concerning medicines for human use. The national competent authorities in those countries from which we have not yet received approval may follow the advice described below of the CHMP that we consider toxicity studies with Fovista administered in combination with Avastin or Eylea prior to initiating our corresponding Phase 3 clinical trial in those countries. In addition, any modifications to our Phase 3 clinical program for Fovista may result in our incurring increased expense or in a delay in the enrollment or completion of such program.

In the fourth quarter of 2013, the CHMP provided scientific advice on our proposed Phase 3 clinical program for Fovista and our plan to seek regulatory approval for Fovista in the European Union. As part of that scientific advice, the CHMP advised us that we should justify our proposal to initiate, at the Phase 3 clinical trial stage, certain previously untested combinations of Fovista with Avastin or Eylea, and, as described above, that we should consider conducting toxicity studies with Fovista administered in combination with Avastin or Eylea prior to initiating our corresponding Phase 3 clinical trial. It is possible that the national competent authorities in those countries from which we have not yet received approval for our Phase 3 clinical trial evaluating Fovista administered in combination with Avastin or Eylea may follow the advice of the CHMP that we consider toxicity studies with Fovista administered in combination with Avastin or Eylea prior to initiating our corresponding Phase 3 clinical trial in those countries. In addition, the CHMP informed us that the final label for Fovista, if it receives marketing approval, may be required to specify the licensed anti-VEGF drugs that were studied in combination with Fovista, given that Avastin is not approved for intravitreal use, rather than a label specifying Fovista for use in combination with any anti-VEGF drug.

We are continuing, internally and with our consultants, to refine our clinical and regulatory strategies for our planned Phase 2/3 clinical program evaluating Zimura for the treatment of geographic atrophy. We have not had formal meetings with regulatory authorities regarding our trial design. Our plans may change significantly based on feedback we may receive from such regulatory authorities. We will need to conduct an additional Phase 3 study, and we may be required by regulatory authorities to conduct other additional clinical trials of Zimura, prior to seeking marketing approval in this indication.

If we are required to conduct additional clinical trials or other testing of Fovista, Zimura or any other product candidate that we may develop beyond those that we contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our product candidates;

- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

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If we experience any of a number of possible unforeseen events in connection with our clinical trials, potential marketing approval or commercialization of our product candidates could be delayed or prevented.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

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

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

- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;

- regulators or institutional review boards may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;

- we may experience delays in reaching, or fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;

- we may decide, or regulators or institutional review boards may require us, to suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;

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

- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates, such as the anti-VEGF drugs we need to use in combination with Fovista, may become insufficient or inadequate.

Our product development costs will also increase if we experience delays in testing or marketing approvals. We do not know whether clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We may not be able to initiate new or continue ongoing clinical trials for Fovista, Zimura or any other product candidate that we develop if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside the United States. In addition, some of our competitors have ongoing clinical trials for product candidates that treat the same indications as Fovista and Zimura, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors product candidates.

Patient enrollment is affected by other factors, including:

- severity of the disease under investigation;
- the ability of current technology to adequately define the disease state;
- eligibility criteria for the study in question;
- perceived risks and benefits of the product candidate under study;

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- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- proximity and availability of clinical trial sites for prospective patients.

The Novartis Agreement contains provisions for milestone payments by Novartis upon our achievement of certain levels of patient enrollment. We will not be entitled to receive the remaining enrollment-based milestone payments unless and until we enroll the specified number of patients. In addition, our inability to locate and enroll a sufficient number of patients for our clinical trials would result in significant delays in our clinical trials, could require us to abandon one or more clinical trials altogether and could delay or prevent our receipt of necessary regulatory approvals. Enrollment delays in our clinical trials also may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

If serious adverse or unacceptable side effects are identified during the development of Fovista, Zimura or any other product candidate that we may develop, we may need to abandon or limit our development of Fovista, Zimura or any other product candidate.

If Fovista, Zimura or any other product candidates we may develop are associated with serious adverse events or undesirable side effects in clinical trials or have characteristics that are unexpected, we may need to abandon their development or limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause side effects that prevented further development of the compound.

Fovista administered in combination with Lucentis was generally well tolerated in our Phase 1 clinical trial and our Phase 2b clinical trials. However, we have clinical data for Fovista administered in combination with Lucentis from only two studies with a limited follow-up of a maximum of 24 weeks. As compared to our Phase 2b clinical trial, our three Phase 3 studies are longer in duration (24 months) with a 12 month timepoint for the primary endpoint, have a greater number of patients (approximately 1866), have a greater number of sites (more than 225), which encompass a much larger geographical recruitment area, and result in chronic exposure to a higher rate of intraocular pressure due to an increased injection volume. Consequently, there is potential for an increase in cumulative side effects resulting from two separate intraocular injections and increased intraocular pressure in the Fovista combination therapy patients as compared to the patients receiving monotherapy anti-VEGF treatment and there is a much longer duration of therapy and greater geographic diversity of patients in our Phase 3 trials. This increase in the number of intraocular injections and treatment burden, increased variability of patient care due to the larger number of clinical trial sites and the broader genetic profile of the enrolled patients from a larger geographic region may result in increased susceptibility to side effects of Fovista and/or resulting from treatment procedure. Therefore there is the potential for an unfavorable safety and tolerability profile in the Fovista combination therapy arm of the study as compared to our Phase 2b study and monotherapy anti-VEGF studies which may be reflected in an increase in adverse events and/or serious adverse event rates (either ocular, systemic or both) in patients receiving Fovista combination therapy. For example, there may be, among others, an increase in the rates of intraocular infections, or endophthalmitis, intraocular pressure, glaucoma, retinal tears, cataracts, retinal detachment, intraocular inflammation, retinal and/or choroidal circulation compromise, cardiovascular disease such as myocardial infarctions, stroke, blood clots or emboli, or hospitalizations in the Fovista combination therapy patients.

In addition, we have very limited clinical and safety data with respect to the effects of Fovista administered in combination with intraocular injections of either Avastin or Eylea. The safety results of our trials are dependent, in part, on the safety and tolerability of the anti-VEGF drug(s) administered in combination with Fovista. Avastin is not approved for the treatment of wet AMD, and according to third-some party clinical trials, may be associated with a greater risk of serious adverse events or undesirable side effects as compared to Lucentis.

We have very limited data regarding the safety, tolerability and efficacy of Zimura for the treatment of geographic atrophy, a form of dry AMD. We have no pre-clinical or clinical data on the effects of Zimura when administered in combination with Fovista and/or an anti-VEGF drug for the treatment of wet AMD. Our clinical trials for Zimura may involve multiple intraocular injections over an extended period of time and, as such, may involve risks regarding multiple and chronic intraocular injections.

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Even if Fovista, Zimura or any other product candidate that we may develop receives marketing approval, such product candidate may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success and the market opportunity for any of our products and product candidates may be smaller than we estimate.

If any of our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. For example, current treatments for wet AMD, including Lucentis, Eylea and low cost, off-label use of Avastin, are well established in the medical community, and doctors may continue to rely upon these treatments without Fovista. If Fovista does not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of Fovista, Zimura or any other product candidate that we may develop, if approved for commercial sale, will depend on a number of factors, including:

- efficacy and potential advantages compared to alternative treatments, including the existing standard of care;
- any restrictions on the use of our products in combination with other medications, such as a Fovista label requiring a waiting period after the intravitreal injection of the anti-VEGF drug and prior to the intravitreal injection of Fovista;
- any restrictions on the use of our products to a subgroup of patients, such as by excluding from the Fovista label patients with pure occult subtype wet AMD;
- restrictions in the label on the use of Fovista with a particular anti-VEGF drug;
- any changes in the dosing regimen of, or the means of administering or delivering, an anti-VEGF drug with which Fovista will be used;
- our and our commercialization partners ability to offer our products at competitive prices, particularly in light of the additional cost of Fovista together with an anti-VEGF drug;
- availability of third-party coverage and adequate reimbursement, particularly by Medicare given our target market for persons over age 55;

- increasing reimbursement pressures on retinal specialists due to the formation of accountable care organizations and the shift away from traditional fee-for-service reimbursement models to reimbursement based on quality of care and patient outcomes;

- willingness of the target patient population to try new therapies and of physicians to prescribe these therapies, particularly in light of the existing available standard of care;

- prevalence and severity of any side effects;

- whether competing products or other alternatives are more convenient or easier to administer, including whether co-formulated alternatives, alternatives that can be co-administered in a single syringe or alternatives that offer a less invasive method of administration than intravitreal injection come to market; and

- the strength of our marketing and distribution support and that of Novartis, our partner for commercialization outside of the United States.

In addition, the potential market opportunity for Fovista is difficult to estimate precisely. If Fovista receives marketing approval for the treatment of wet AMD, it will be approved solely for use in combination with an anti-VEGF drug. The market opportunity for Fovista will be dependent upon the continued use of anti-VEGF drugs in the treatment of wet AMD and the market share of such anti-VEGF drugs for which Fovista is approved as a combination therapy. In addition, because physicians, patients and third-party payors may be sensitive to the addition of the cost of Fovista to the cost of treatment with anti-VEGF drugs, we may experience downward pressure on the price we can charge for Fovista.

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Our Phase 3 clinical program enrolls patients based on a specific definition of the presence of neovascularization with certain characteristics using the commonly employed modality of spectral domain optical coherence tomography, or SD-OCT. We are not aware of any third-party clinical trials that have used this criteria to assess patient inclusion and as such do not know the proportion of total cases of subfoveal choroidal neovascularization that are represented using this specific definition of SD-OCT guided inclusion criteria. Therefore, we cannot easily assess the impact on the potential market opportunity should Fovista receive marketing approval and the approved label exclude patients based on this criteria.

Our Phase 3 clinical program provides for a 30-minute delay in the injection of Fovista after the anti-VEGF drug to minimize the risk in our clinical trials of an unacceptable increase in intraocular pressure as a result of the amount of the two agents injected. If Fovista receives marketing approval for the treatment of wet AMD and the approved label requires such a waiting period, the potential market opportunity for Fovista may be limited to the extent that physicians and patients find such a waiting period unacceptable.

The current standard of care for wet AMD is monotherapy administration of anti-VEGF drugs, principally Avastin, Lucentis and Eylea, which are well established therapies and are widely accepted by physicians, patients and third- party payors. When used for the treatment of wet AMD, Avastin is inexpensive. Physicians, patients and third-party payors may not accept the addition of Fovista to their current treatment regimens for a variety of potential reasons, including:

- if they do not wish to incur the additional cost of Fovista;

- if they perceive an additional injection to administer Fovista as undesirable and we and Novartis are unsuccessful in developing and marketing a co-formulated product;

- if they perceive the addition of Fovista to be of limited benefit to patients; or

- if they wish to treat with anti-VEGF drugs as monotherapy first and add Fovista only if and when resistance to continued anti-VEGF therapy limits further enhancement of visual outcome with anti-VEGF monotherapy.

Our estimates of the potential market opportunity for each of Fovista and Zimura include several key assumptions based on our industry knowledge, industry publications, market response to marketed AMD drugs, third-party research reports and other surveys. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions. If any of these assumptions proves to be inaccurate, then the actual market for Fovista or Zimura could be smaller than our estimates of our potential market opportunity. If the actual market for Fovista or Zimura is smaller than we expect, our product revenue may be limited and it may be more difficult for us to achieve or maintain profitability.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to Fovista and Zimura from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of product candidates for the treatment of wet AMD or other disease indications for which we may develop Fovista. Although there are currently no therapies approved by the FDA or the EMA for the treatment of dry AMD, there are also a number of pharmaceutical and biotechnology companies that are currently pursuing the development of products for this indication. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. We also will face similar competition with respect to any other products or product candidates that we may seek to develop or commercialize in the future for the treatment of wet AMD, dry AMD or other diseases.

There are also a number of products in preclinical research and clinical development by third parties to treat wet AMD, including product candidates that inhibit the function of PDGF, the molecule whose function Fovista also inhibits, product candidates that inhibit the function of both VEGF and PDGF that could obviate the separate use of an anti-PDGF agent, such as Fovista, and anti-VEGF and/or anti-PDGF gene therapy products that may substantially reduce the number and frequency of intravitreal injections when treating wet AMD. These companies include pharmaceutical companies, biotechnology companies, and specialty pharmaceutical and generic drug companies of various sizes, such as Regeneron Pharmaceuticals, Inc., which is working in collaboration with Bayer HealthCare and has recently announced that it plans to initiate a Phase 2 clinical trial of its combination anti- VEGF/anti-PDGF clinical candidate in the first quarter of 2015, Allergan, Inc., Ohr Pharmaceutical, Inc., Xcovery Vision LLC, Santen, Neurotech Pharmaceuticals, Inc., Avalanche

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Biotechnologies, Inc., Somalogic, Inc. and others. Several companies are pursuing the manipulation of stem cells to provide a novel approach to treating retinal diseases, including wet AMD.

In addition, other companies are undertaking efforts to develop technologies to allow for a less frequent dosing schedule for anti-VEGF therapies that are currently in use. If such technologies are successfully developed and approved for use, we may need to conduct additional clinical trials of Fovista using a less frequent dosing schedule than the dosing schedule we are currently using in our ongoing Phase 3 clinical program. Any such trials may not be successful.

Moreover, there are a number of products in preclinical research and clinical development by third parties to treat dry AMD, including product candidates that are designed to suppress inflammation, such as complement system inhibitors and coricosteroids, visual cycle modulators, antioxidants and neuroprotectants, cell and gene therapies and vascular enhancers. These companies include pharmaceutical companies, biotechnology companies, and specialty pharmaceutical and generic drug companies of various sizes. In particular, with respect to complement system inhibition, these companies include Genentech, Novartis s Alcon division, Alexion Pharmaceuticals, Inc. and MophoSys. Moreover, we are aware that the following companies are pursuing the clinical development of ophthalmic product candidates with other mechanisms of action for the treatment of dry AMD: Alimera Sciences, Acucela, Colby Pharmaceuticals, Allergan, Pfizer, GlaxoSmithKline and Macuclear.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient to use or are less expensive than Fovista, Zimura or other products that we may develop. The commercial opportunity for Fovista also could be reduced or eliminated if our competitors develop and commercialize products that reduce or eliminate the use of anti- VEGF drugs for the treatment of patients with wet AMD. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

In addition, our ability to compete may be affected in many cases by insurers or other third-party payors, particularly Medicare, seeking to encourage the use of less expensive or more convenient products. We expect that if Fovista is approved, the cost of treatment of wet AMD with a combination of Fovista with an anti-VEGF drug will be significantly higher than the cost of treatment of wet AMD with Avastin, Lucentis or Eylea monotherapy. Insurers and other third- party payors may encourage the use of anti-VEGF drugs as monotherapy and discourage the use of Fovista in combination with these drugs. This could limit sales of Fovista.

Many of our competitors have significantly greater financial and human resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

We have no experience manufacturing Fovista or Zimura at commercial scale. As a result, delays in regulatory approval of Fovista or Zimura may occur. Also, manufacturing issues may arise that could cause delays or increase costs.

We have no experience manufacturing the chemically synthesized aptamers comprising the API of Fovista or Zimura at commercial scale. We currently rely upon a single third-party manufacturer, Agilent Technologies, to supply us with API, also referred to as drug substance, for both Fovista and Zimura and a different, single third-party manufacturer to provide fill-finish services for both Fovista and Zimura. Other than our agreement with Agilent Technologies with respect to our clinical supply of Fovista API, all of our manufacturing arrangements are on a purchase order basis. In order to obtain regulatory approval for Fovista or Zimura, these third-party manufacturers will be required to consistently produce the API used in Fovista or Zimura in commercial quantities and of specified quality or execute fill-finish services on a repeated basis and document their ability to do so. This is referred to as process validation. If the third-party manufacturers are unable to satisfy this requirement, our business will be materially and adversely affected.

Our third-party manufacturer of API for Fovista and Zimura has made only a limited number of lots of Fovista and Zimura to date and has not made any commercial lots. The manufacturing processes for Fovista and Zimura have never been tested at commercial scale, and the process validation requirement has not yet been satisfied for either product candidate. These manufacturing processes and the facilities of our third-party manufacturers, including our third-party API manufacturer and our third-party manufacturer providing fill-finish services, will be subject to inspection and approval by the FDA before we can commence the manufacture and sale of Fovista or Zimura, and thereafter on an ongoing basis. Our third-party manufacturer for

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API has never been inspected by the FDA and has not been through the FDA approval process for a commercial product. Our third-party manufacturer providing fill-finish services is subject to FDA inspection from time to time. Failure by our third-party manufacturers to pass such inspections and otherwise satisfactorily complete the FDA approval regimen with respect to our product candidates may result in regulatory actions such as the issuance of FDA Form 483 notices of observations, warning letters or injunctions or the loss of operating licenses. Additionally, on October 22, 2014, the FDA issued its final guidance on the circumstances that constitute delaying, denying, limiting or refusing a drug inspection pursuant to Section 707 of the Food and Drug Administration Safety and Innovation Act of 2012. If any of our third-party manufacturers are found to have delayed, denied, limited or refused a drug inspection, our API or drug product could be deemed adulterated. Based on the severity of the regulatory action, our clinical or commercial supply of API or our fill-finish services could be interrupted or limited, which could have a material adverse effect on our business.

The standards of the International Conference on Harmonization of Technical Requirements for Registration of Pharmaceuticals for Human Use, which establishes basic guidelines and standards for drug development in the United States, the European Union, Japan and other countries, do not apply to oligonucleotides, including aptamers. As a result, there is no established generally accepted manufacturing or quality standard for the production of Fovista or Zimura. Even though the FDA has reviewed the quality standards for Fovista to be used in our Phase 3 clinical program, the FDA has the ability to modify these standards at any time and foreign regulatory agencies may impose differing quality standards and quality control on the manufacture of Fovista. The lack of uniform manufacturing and quality standards among regulatory agencies may delay regulatory approval of Fovista or Zimura.

Also, as we or any manufacturer we engage scales up manufacturing of any approved product, we may encounter unexpected issues relating to the manufacturing process or the quality, purity and stability of the product, and we may be required to refine or alter our manufacturing processes to address these issues. Resolving these issues could result in significant delays and may result in significantly increased costs. If we experience significant delays or other obstacles in producing any approved product for commercial scale, our ability to market and sell any approved products may be adversely affected and our business could suffer.

If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution agreements with third parties, we may not be successful in commercializing Fovista, Zimura or any other product candidate that we develop if and when Fovista, Zimura or any other product candidate is approved.

We do not have a sales, marketing or distribution infrastructure and have no track record in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product, we must either develop a sales, marketing and distribution organization or outsource those functions to third parties. If Fovista receives marketing approval, we plan to commercialize it in the United States with our own specialty sales force targeting retinal specialists. Pursuant to the Novartis Agreement, we have granted to Novartis the exclusive right to commercialize Fovista outside of the United States in consideration for royalties on any such sales.

There are risks involved with establishing our own sales, marketing and distribution capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing and distribution capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;

- the inability of sales personnel to obtain access to adequate numbers of physicians who may prescribe our products;

- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and

- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenues and our profitability, if any, are likely to be lower than if we were to market, sell and distribute ourselves any products that we develop. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute our product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control

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over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

If we do not maintain a productive collaborative relationship with Novartis, to whom we have granted exclusive commercialization rights for Fovista outside of the United States, or if Novartis is unable to meets its contractual obligations, we may be forced to focus our efforts internally to commercialize Fovista outside of the United States without the assistance of a commercialization partner or seek another commercialization partner, either of which would result in us incurring greater expenses and could cause a delay in market penetration while we expand our commercial operations or seek an alternative commercialization partner. Such costs may exceed the increased revenues we would receive from direct Fovista sales outside of the United States, at least in the near term. We would also be forced to declare a breach of the Novartis Agreement and seek a termination of the agreement which could result in an extended and uncertain dispute with Novartis, including arbitration or litigation, any of which will be costly.

Even if we are able to commercialize Fovista, Zimura or any other product candidate that we may develop, the product may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our or our commercialization partners commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Our ability and the ability of our commercialization partners, including Novartis, to commercialize Fovista, Zimura or any other product candidate successfully also will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A major trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third- party payors, particularly Medicare, have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for Fovista, Zimura or any other product that we commercialize or our commercialization partners commercialize on our behalf, and, even if these are available, the level of reimbursement may not be satisfactory.

Reimbursement may affect the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining and maintaining adequate reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician and because, in the case of Fovista, our drug will be administered in combination with other drugs that may carry high prices. In addition, physicians, patients and third-party payors may be sensitive to the addition of the cost of Fovista to the cost of treatment with anti-VEGF drugs. We or our commercialization partners may be required to conduct expensive pharmacoeconomic studies to justify coverage and reimbursement or the level of reimbursement relative to other therapies, including in the case of Fovista, relative to monotherapy with anti-VEGF drugs. If coverage and adequate reimbursement are not available or reimbursement is available only to limited levels, we may not be able to successfully commercialize Fovista, Zimura or any other product candidate for which we obtain marketing approval.

There may be significant delays in obtaining reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or similar regulatory authorities outside the United States. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs, and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States.

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Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our and our commercialization partners inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Our strategy of obtaining rights to complementary products, product candidates or technologies for the treatment of a range of ophthalmic diseases through in-licenses and acquisitions may not be successful.

We plan to expand our product pipeline through opportunistically in- licensing or acquiring the rights to complementary products, product candidates or technologies for the treatment of ophthalmic diseases. Because we expect generally that we will not engage directly in early stage research and drug discovery, the future growth of our business will depend significantly on our ability to in-license or acquire the rights to approved products, additional product candidates or technologies. However, we may be unable to in-license or acquire the rights to any such products, product candidates or technologies from third parties. The in-licensing and acquisition of pharmaceutical products is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire products, product candidates or technologies that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to in-license or acquire the rights to the relevant complementary product, product candidate or technology on terms that would allow us to make an appropriate return on our investment. Furthermore, we may be unable to identify suitable products, product candidates or technologies within our area of focus. If we are unable to successfully obtain rights to suitable products, product candidates or technologies, our business, financial condition and prospects for growth could suffer.

Product liability lawsuits against us or our commercialization partners could divert our resources, cause us to incur substantial liabilities and limit commercialization of any products that we may develop or in-license.

We face an inherent risk of product liability exposure related to the testing of Fovista, Zimura and any other product candidate that we develop in human clinical trials and we and our commercialization partners will face an even greater risk if we commercially sell any products that we develop or in- license. Because our Phase 3 clinical program for Fovista involves the administration of Fovista in combination with anti-VEGF drugs, including off-label use by intravitreal injection of Avastin provided by us, we also face an inherent risk of product liability exposure related to the testing of such anti-VEGF drugs. If we become subject to or otherwise cannot successfully defend ourselves against claims that our product candidates, anti-VEGF drugs administered in combination with our product candidates or our products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop or in-license;
- injury to our reputation and significant negative media attention;

- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced time and attention of our management to pursue our business strategy; and
- the inability to commercialize any products that we may develop or in-license.

We currently hold $10.0 million in product liability insurance coverage in the aggregate, with a per incident limit of $10.0 million, which may not be adequate to cover all liabilities that we may incur. We will need to increase our insurance coverage when and if we begin commercializing Fovista, Zimura or any other product candidate that receives marketing approval. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. In addition, if Novartis or one of our other future

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commercialization or collaboration partners were to become subject to product liability claims or were unable to successfully defend themselves against such claims, any such commercialization or collaboration partners could be more likely to terminate such relationship with us and therefore substantially limit the commercial potential of our products.

Risks Related to Our Dependence on Third Parties

We may enter into collaborations with third parties for the development or commercialization of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates.

If either of Fovista or Zimura receives marketing approval, we plan to commercialize such product candidate in the United States with our own specialty sales force targeting retinal specialists. In May 2014, we entered into the Novartis Agreement pursuant to which we granted Novartis the exclusive right to commercialize Fovista outside of the United States. We expect to utilize a variety of types of collaboration, distribution and other marketing arrangements with third parties to commercialize Zimura in markets outside the United States. We also may seek third-party collaborators for development and commercialization of other product candidates we may develop. Our likely collaborators for any sales, marketing, distribution, development, licensing or broader collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we do enter into any additional arrangements with third parties in the future, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements and our arrangement with Novartis for Fovista will depend on our collaborators and Novartis s abilities and efforts to successfully perform the functions assigned to them in these arrangements. Collaborations involving our product candidates, including our collaboration with Novartis, could pose numerous risks to us, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations and may not perform their obligations as expected;

- collaborators may deemphasize or not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators strategic focus, changes in product candidate priorities or available funding or changes in priorities as a result of a merger, acquisition or other corporate restructuring;

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

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

- we could grant exclusive rights to our collaborators, which would prevent us from collaborating with others;

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

- collaborators with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;

- collaborators may not properly maintain or defend our intellectual property rights, may infringe the intellectual property rights of third parties, may misappropriate our trade secrets or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to litigation and potential liability; and

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- collaborations may be terminated for the convenience of the collaborator, our breach of the terms of the collaboration or other reasons and, if terminated, we may need to raise additional capital to pursue further development or commercialization of the applicable product candidates.

If a collaborator of ours, including Novartis, were to be involved in a business combination, the foregoing risks would be heightened, and the business combination may divert attention or resources or create competing priorities. The collaborator may delay or terminate our product development or commercialization program. If one of our collaborators, including Novartis, terminates its agreement with us, we could find it more difficult to attract new collaborators and the perception of our company in the business and financial communities could be adversely affected.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all.

We will depend heavily on our commercialization arrangement with Novartis for Fovista outside of the United States. If Novartis terminates our agreement or is unable to meet its contractual obligations, it could negatively impact our revenues and harm our business until appropriate measures have been taken.

On May 19, 2014, we entered into the Novartis Agreement pursuant to which we granted exclusive rights to Novartis to commercialize Fovista outside of the United States. The agreement continues until the date on which we are no longer entitled to receive a royalty on Fovista or any co-formulated product containing Fovista developed under the agreement. The agreement is subject to early termination in the event of certain customary defaults, such as material breach of the agreement and bankruptcy. In addition, the agreement is subject to early termination by either us or Novartis if the other party challenges or assists a third party in challenging the validity or enforceability of certain patent rights controlled by the terminating party, or if the parties are prevented in any manner that materially adversely affects the progression of the development or commercialization of licensed products for a specified period as a result of specified governmental actions. Novartis may also terminate the agreement at any time without cause, or within a specified period after a change in control of us, as defined in the agreement, or for specified safety reasons, effective at the end of a specified period following Novartis s written notice to us of Novartis s election to terminate the agreement. We may also terminate the agreement if Novartis determines to seek marketing approval of an alternative anti-PDGF product outside the United States. If we do not maintain a productive collaborative relationship with Novartis or if Novartis is unable to meet its contractual obligations or if there is an early termination of the agreement as described above, we will be forced to either establish a commercial infrastructure outside of the United States so that we could undertake the commercialization efforts which had been theretofore undertaken by Novartis or we will need to seek an alternative partner. The establishment of a commercial infrastructure and assumption by us of commercialization activities outside of the United States would require substantial resources, financial and otherwise, and could result in us incurring greater expenses than the increase in revenues from our direct sales of Fovista. It could also cause a delay in market penetration while we expand our commercial operations. Seeking and obtaining an alternative commercial partner outside the United States could also adversely impact sales of Fovista and market penetration outside of the United States.

If we are not able to establish collaborations, we may have to alter our development and commercialization plans.

The development and potential commercialization of Zimura and other product candidates that we may develop will require substantial additional cash to fund expenses. For some of our product candidates, we may decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for collaboration will depend, among other things, upon our assessment of the collaborator s resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator s evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such collaboration could be more attractive than the one with us for our product candidate. We may also be restricted under future license agreements from entering into agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

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

We rely upon third parties in conducting our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We have relied on third-party clinical research organizations, or CROs, in conducting our completed clinical trials of Fovista and Zimura. We expect to continue to rely upon third parties, such as CROs, clinical data management organizations, medical institutions (including reading centers) and clinical investigators, in conducting our clinical trials for Fovista and Zimura, including the clinical trials in our Phase 3 clinical program for Fovista, and expect to rely upon these third parties to conduct clinical trials of any other product candidate that we may develop. We or these third parties may terminate their engagements with us at any time for a variety of reasons, including a failure to perform by the third parties. If we need to enter into alternative arrangements, that would delay our product development activities and could potentially be very costly.

Our reliance on these third parties for clinical development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as Good Clinical Practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database within specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors.

We also rely upon other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

We contract with third parties for the manufacture of both Fovista and Zimura for clinical trials and expect to continue to do so in connection with the commercialization of Fovista and for clinical trials and commercialization of any other product candidates that we develop or may develop. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not currently own or operate manufacturing facilities for the production of clinical or commercial quantities of Fovista or Zimura and have limited personnel with manufacturing experience. We currently rely upon and expect to continue to rely upon third-party contract manufacturers to manufacture clinical and commercial supplies of Fovista and Zimura, preclinical and clinical supplies of other product candidates we may develop and commercial supplies of products if and when approved for marketing by applicable regulatory authorities. Our current and anticipated future dependence upon others for the manufacture of Fovista, Zimura and any other product candidate or product that we develop may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis. In addition, any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. Under the Novartis Agreement, we are responsible for supplying to Novartis drug substance for Fovista for clinical and commercial supply.

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We currently rely exclusively upon a single third-party manufacturer to provide clinical supplies of both Fovista drug substance and Zimura drug substance. We also engage a single third-party manufacturer to provide fill- finish services for clinical supplies of both Fovista and Zimura. Other than our agreement with Agilent Technologies with respect to our clinical supply of Fovista drug substance, we obtain these supplies and services from each of these manufacturers on a purchase order basis. We do not currently have any contractual commitments for commercial supply of bulk drug substance for either Fovista or Zimura or for fill-finish services. We also do not currently have arrangements in place for redundant supply or a second source for bulk drug substance for Fovista or Zimura or for fill-finish services. The prices at which we are able to obtain supplies of drug substance for Fovista or Zimura and fill-finish services may vary substantially over time and adversely affect our financial results. Furthermore, we currently rely upon sole-source suppliers of certain raw materials and other specialized components of production used in the manufacture and fill-finish of each of Fovista and Zimura.

We currently rely exclusively upon Nektar to supply us with a proprietary polyethylene glycol, or PEG, reagent for Fovista under a manufacturing and supply agreement. PEG reagent is a chemical we use to modify the chemically synthesized aptamer in Fovista. The PEG reagent made by Nektar is proprietary to Nektar and, to our knowledge, is not currently available from any other third party.

We obtain a different proprietary PEG reagent used to modify the chemically synthesized aptamer in Zimura from a different supplier on a purchase order basis. We do not currently have any contractual commitments for supply of the PEG reagent we use for Zimura.

If our third-party manufacturers for Fovista drug substance, Zimura drug substance or the PEG reagent we use for Zimura fail to fulfill our purchase orders, if Nektar breaches its obligations to us under our supply agreement, or if any of these manufacturers should become unavailable to us for any reason, we believe that there are a limited number of potential replacement manufacturers, and we likely would incur added costs and delays in identifying or qualifying such replacements. We could also incur additional costs and delays in identifying or qualifying a replacement manufacturer for fill-finish services for Fovista or Zimura if our existing third-party fill-finish provider should become unavailable for any reason. We may be unable to establish any agreements with such replacement manufacturers or fill-finish providers or to do so on acceptable terms.

Under the supply agreement with Nektar, we must purchase our entire requirements for PEG reagent for Fovista exclusively from Nektar at agreed prices based on volume. In the event Nektar breaches its supply obligations as specified in the agreement, Nektar has agreed to enable a third-party manufacturer, if one is available, to supply us with PEG reagent until Nektar demonstrates that Nektar has the ability to supply all of our requirements for PEG reagent. The agreement of Nektar to enable a third-party manufacturer may be difficult to enforce in the context of a breach by Nektar of its supply obligations. We may not be able to reach an agreement with any third-party manufacturer to take on the supply of PEG reagent under such circumstances because, to our knowledge, no third party currently manufactures the PEG reagent we currently use in making the Fovista drug substance. Furthermore, the third party s right to supply us with PEG reagent would be subject to termination at any time once Nektar demonstrates that Nektar has the ability to supply all of our requirements for PEG reagent, which may limit the interest of potential third-party manufacturers in undertaking such an engagement. In addition, the process of transferring any necessary technology or process to a third-party manufacturer would entail significant delay in or disruption to the supply of PEG reagent and, as a result, a significant delay in or disruption to the manufacture of Fovista. Furthermore, the FDA or other regulatory authorities might require additional studies to demonstrate equivalence between the Fovista drug substance made using the Nektar PEG reagent and the Fovista drug substance made using any replacement PEG reagent we propose to use or between the Nektar PEG reagent itself and any replacement PEG reagent we propose to use to make Fovista. We ultimately may be unable to demonstrate such equivalence.

Reliance on third-party manufacturers entails additional risks, including:

- Fovista, Zimura and any other product that we may develop may compete with other product candidates and products for access to a limited number of suitable manufacturing facilities that operate under current good manufacturing practices, or cGMP, regulations;
- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- the possible breach of our supply obligations to Novartis;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how; and
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

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Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products and harm our business and results of operations.

We depend on licenses and sublicenses for development and commercialization rights to our products, product candidates and technologies. Termination of these rights or the failure by us or our licensees, including our commercialization or collaboration partners to comply with obligations under these or other agreements under which we obtain such rights or have obtained funding could materially harm our business and prevent us from developing or commercializing our products and product candidates.

We are party to various agreements, including an acquisition agreement with OSI Pharmaceuticals and license agreements with Archemix and Nektar that we depend on for rights to Fovista, Zimura and other product candidates and technology. These agreements impose, and we may enter into additional licensing arrangements or other agreements with third parties that may impose, diligence, development and commercialization timelines, milestone payment, royalty, insurance and other obligations on us. Under our acquisition agreement with OSI Pharmaceuticals and our licensing agreement with Nektar, we are obligated to pay royalties on net product sales of Fovista or other product candidates or related technologies to the extent they are covered by the agreement. Under our license agreements with Archemix and Nektar, we would not be able to avoid our payment obligations even if we believed a licensed patent right was invalid or unenforceable because the license agreements provide that our licenses to all licensed patent rights would terminate if we challenge the validity or enforceability of any licensed patent right.

We also have diligence and development obligations under our acquisition agreement with OSI Pharmaceuticals and our license agreements with Archemix and Nektar. Generally, these diligence obligations require us to use commercially reasonable efforts to develop, seek regulatory approval for and commercialize our products in the United States, the European Union and, in some cases, certain other specified countries. Although the Novartis Agreement provides that Novartis will be responsible for performing certain of these obligations with respect to specified countries, we still remain liable under our agreements with OSI Pharmaceuticals, Archemix and Nektar. If we fail to comply with our obligations under current or future acquisition, license and funding agreements, or otherwise breach an acquisition, license or funding agreement as a result of our own actions or inaction or the actions or inactions of our commercialization or collaboration partners, our counterparties may have the right to terminate these agreements, in which event we might not have the rights or the financial resources to develop, manufacture or market any product that is covered by these agreements. Such a failure to comply or breach by us under any of these agreements could also lead to a breach by us of the Novartis Agreement. Our counterparties also may have the right to convert an exclusive license to non-exclusive in the territory in which we fail to satisfy our diligence obligations, which could materially adversely affect the value of the product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or restated agreements with less favorable terms, seek alternative sources of financing or cause us to lose our rights under these agreements, including our rights to Fovista, Zimura and other important intellectual property or technology. Any of the foregoing could prevent us from commercializing Fovista, Zimura or other product candidates we may develop, which could have a material adverse effect on our operating results and overall financial condition.

In addition to the generally applicable diligence obligations set forth above, we have specific obligations with respect to the licensing agreements described below:

- Under the terms of the agreement with OSI Pharmaceuticals under which we acquired certain rights to develop and commercialize Fovista, if we or our commercialization or collaborative partners fail to meet certain obligations, OSI Pharmaceuticals may terminate the agreement as to such countries with respect to which such failure has occurred, and upon such termination we will be obligated to grant, assign and transfer to OSI Pharmaceuticals specified rights and licenses related to our anti-PDGF aptamer technology and other related assets, and if we are manufacturing such anti-PDGF products at the time of such termination, may be obligated to provide transitional supply to OSI Pharmaceuticals of covered anti-PDGF products, for such countries.

- Under the terms of the amended license, manufacturing and supply agreement with Nektar, pursuant to which we obtained, among other licenses, an exclusive, worldwide license to make, develop, use, import, offer for sale and sell certain products that incorporate a specified PEG reagent linked with the active ingredient in Fovista, if we

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fail to use commercially reasonable efforts to achieve the first commercial sale of Fovista in the United States by December 31, 2016, we and Nektar may agree in good faith to extend such date in specified circumstances. If such date is not extended, Nektar may either terminate our license or convert our license for such country to a non- exclusive license. In addition, if we fail to use commercially reasonable efforts to develop Fovista and file and seek approval of NDAs on a schedule permitting us to make first commercial sales of Fovista in specified countries by December 31, 2017, do not make such first commercial sales of Fovista by such date, or thereafter fail to use commercially reasonable efforts to continue to commercialize and market Fovista in such countries, we will be in material breach of the agreement and Nektar will have the right to terminate the agreement.

In addition to the above risks, certain of our intellectual property rights are sublicenses under intellectual property owned by third parties, in some cases through multiple tiers. The actions of our licensors may therefore affect our rights to use our sublicensed intellectual property, even if we are in compliance with all of the obligations under our license agreements. For example, the licenses from Archemix include sublicenses to us of rights to specified technology, which we refer to as the SELEX technology, licensed by University License Equity Holdings, Inc. to Gilead Sciences, Inc., or Gilead, and sublicensed by Gilead to Archemix, as well as other technology owned by Gilead and licensed to Archemix. In addition, the licenses we have obtained from Nektar include sublicenses of certain rights. Should our licensors or any of their upstream licensors fail to comply with their obligations under the agreements pursuant to which they obtain the rights that are sublicensed to us, or should such agreements be terminated or amended, our ability to develop and commercialize Fovista, Zimura and other product candidates may be materially harmed and could also lead to a breach by us of the Novartis Agreement. While the applicable agreements may contain contractual provisions that would in many instances protect our rights as a sublicensee in these circumstances, these provisions may not be enforceable and may not protect our rights in all instances. Further, we do not have the right to control the prosecution, maintenance and enforcement of all of our licensed and sublicensed intellectual property, and even when we do have such rights, we may require the cooperation of our licensors and their upstream licensors, which may not be forthcoming. Our business could be materially adversely affected if we are unable to prosecute, maintain and enforce our licensed and sublicensed intellectual property effectively.

Risks Related to Our Intellectual Property

The patent prosecution process is expensive and time-consuming, is highly uncertain and involves complex legal and factual questions. Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

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

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. In addition, we may not pursue or obtain patent protection in all major markets. Moreover, in some circumstances, we do not have the right to control the preparation, filing or prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties or covering technology that our collaboration or commercialization partners may develop, the eventual commercialization of which could potentially entitle us to royalty payments. In some circumstances, our licensors have the right to enforce the licensed patents without our involvement or consent, or to decide not to enforce or to allow us to enforce the licensed patents. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. If any such licensors fail to maintain such patents, or lose rights to those patents, the rights that we have licensed may be reduced or eliminated and our ability to develop and commercialize any of our products that are the subject of such licensed rights could be adversely affected.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign jurisdictions may not protect our rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions. Moreover, the United States Patent and Trademark Office might require that the term of a patent issuing from a pending patent application be

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disclaimed and limited to the term of another patent that is commonly owned or names a common inventor. As a result, the issuance, scope, validity, term, enforceability and commercial value of our patent rights are highly uncertain.

Our and our collaboration and commercialization partners pending and future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. In particular, during prosecution of any patent application, the issuance of any patents based on the application may depend upon our or their ability to generate additional preclinical or clinical data that support the patentability of our proposed claims. We or our collaboration and commercialization partners may not be able to generate sufficient additional data on a timely basis, or at all. Moreover, changes in either the patent laws or interpretation of the patent laws in the United States or other countries may diminish the value of our or our collaboration and commercialization partners patents or narrow the scope of our or their patent protection.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art, may affect patent litigation and switch the U.S. patent system from a first-to-invent system to a first-to-file system. Under a first-to-file system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to the patent on an invention regardless of whether another inventor had made the invention earlier. The U.S. Patent and Trademark Office recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first-to-file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

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

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

The last to expire of the U.S. patent rights covering the composition of matter of Fovista is expected to expire in 2017. Such expiration date is not long after the date by which we expect Fovista to be commercialized in the United States if we obtain marketing approval and may even be prior to such date. We own an issued U.S. patent covering methods of treating wet AMD with Fovista in combination with Avastin or Lucentis, which is expected to expire in 2024. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch- Waxman Act, permits a patent restoration term of up to five years as partial compensation for patent term effectively lost during product development and the FDA regulatory review process occurring after the issuance of a patent. We may be able to obtain a patent term extension for one of these U.S. patents. However, we may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration or the term or scope of any such extension is less than we request, any period during which we have the right to exclusively market our product will be shorter than we would otherwise expect, and our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced, possibly materially.

The European patent rights covering the composition of matter of Fovista are expected to expire in 2018. Such expiration date is shortly after the date by which we expect Fovista to be commercialized in Europe, and may even be prior to such date. We own a granted European patent covering a combination of Fovista and Lucentis or Avastin for use in a method for treating wet AMD. This European patent is expected to expire in 2024.

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We also have filed in the United States patent applications covering a method of treating wet AMD in patients with Fovista in combination with Eylea and in Europe and Japan patent applications covering a combination of Fovista and Eylea for use in a method for treating wet AMD. These patent applications are in the early stages of prosecution and may not result in patents being issued which protect the use of Fovista in combination with Eylea for treating wet AMD or effectively prevent others from commercializing competitive technologies and products. If a patent is granted following prosecution of any such application, the latest protected patent expiry would be in 2030.

Method-of-treatment patents are more difficult to enforce than composition-of-matter patents because of the risk of off-label sale or use of a drug for the patented method. The FDA does not prohibit physicians from prescribing an approved product for uses that are not described in the product s labeling. Although use of a product directed by off-label prescriptions may infringe our method-of-treatment patents, the practice is common across medical specialties, particularly in the United States, and such infringement is difficult to detect, prevent or prosecute. Off-label sales of other products having the same API as Fovista, Zimura or any other product candidates we may develop would limit our ability to generate revenue from the sale of Fovista, Zimura or such other product candidates, if approved for commercial sale. In addition, European patent law generally makes the issuance and enforcement of patents that cover methods of treatment of the human body difficult. Further, once the composition-of-matter patents relating to Fovista, Zimura or any other product candidate in a particular jurisdiction, if any, expire, competitors will be able to make, offer and sell products containing the same API as Fovista, Zimura or such other product candidate in that jurisdiction so long as these competitors do not infringe any other of our patents covering Fovista s or Zimura s composition of matter or method of use or manufacture, do not violate the terms of any marketing or data exclusivity that may be granted to us by regulatory authorities and obtain any necessary marketing approvals from applicable regulatory authorities. In such circumstances, we also may not be able to detect, prevent or prosecute off- label use of such competitors products containing the same API as Fovista or Zimura in combination with any anti-VEGF drug, even if such use infringes any of our method-of-treatment patents.

The Hatch-Waxman Act also permits the manufacture, use, offer for sale, sale or importation of a patented invention other than a new animal drug or veterinary biological product, if the manufacture, use, offer for sale, sale or importation is solely for uses that are reasonably related to development of information that could be submitted to the FDA. For this reason, our competitors might be able under certain circumstances to perform activities within the scope of the U.S. patents that we own or under which we are licensed without infringing such patents. This might enable our competitors to develop during the lifetime of these patents drugs that compete with Fovista or Zimura, if approved.

The U.S. patent rights covering Zimura as a composition of matter are expected to expire in 2025. Such expiration date may be prior to the date by which we would be able to commercialize Zimura in the United States if we seek and obtain marketing approval. The U.S. patent rights covering methods of treating certain complement protein mediated disorders with Zimura are expected to expire in 2026. As a result, if we obtain marketing approval for Zimura, we may not be able to exclude competitors from commercializing products similar or identical to ours if such competitors do not use or promote our claimed methods of treatment or do use or promote our methods of treatment after our patents expire. Depending on potential delays in the regulatory review process for Zimura, we may be able to obtain a patent term extension for one of these patents in the United States, but we can provide no assurances that such an extension will be obtained.

Our issued patents may not be sufficient to provide us with a competitive advantage. For example, competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. Even if our owned or licensed patent applications issue as patents, they may not issue with a scope broad enough to provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. We could also fail to take the required actions and pay the necessary governmental fees to maintain our patents.

The issuance of a patent is not conclusive as to its inventorship, ownership, scope, term, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. For example, if we receive marketing approval for our product candidates, other pharmaceutical companies may seek approval of generic versions of our products with the FDA or regulatory authorities in other jurisdictions. We may then be required to initiate proceedings against such companies in an attempt to prevent them from launching such generic versions. The risk of being involved in such proceedings is likely to increase if our products are commercially successful. In any such proceedings, the inventorship, ownership, scope, term, validity and enforceability of our patents may be challenged. These and other challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to prevent others from using or commercializing similar or identical technology and products or from launching generic versions of our products, or could limit the duration of the patent protection of our

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technology and products. The launch of a generic version of one of our products in particular would be likely to result in an immediate and substantial reduction in the demand for our product, which could have a material adverse effect on our business. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

Competitors may infringe or otherwise violate our patents, trademarks, copyrights or other intellectual property. To counter infringement or other violations, we may be required to file claims, which can be expensive and time consuming. Any such claims could provoke these parties to assert counterclaims against us, including claims alleging that we infringe their patents or other intellectual property rights. In addition, in a patent infringement proceeding, a court may decide that one or more of the patents we assert is invalid or unenforceable, in whole or in part, construe the patent s claims narrowly or refuse to prevent the other party from using the technology at issue on the grounds that our patents do not cover the technology. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In such a case, we could ultimately be forced to cease use of such marks. In any intellectual property litigation, even if we are successful, any award of monetary damages or other remedy we receive may not be commercially valuable. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation.

Third parties may initiate legal proceedings alleging that we are infringing or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability and the ability of our collaboration and commercialization partners to develop, manufacture, market and sell our product candidates and products and use our proprietary technologies without infringing or otherwise violating the intellectual property and other proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries. We or our collaboration and commercialization partners may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference, derivation, re-examination, post-grant review, opposition, cancellation or similar proceedings before the U.S. Patent and Trademark Office or its foreign counterparts. The risks of being involved in such litigation and proceedings may also increase as our or their product candidates near commercialization and as we gain the greater visibility associated with being a public company. Third parties may assert infringement claims against us or our collaboration or commercialization partners based on existing or future intellectual property rights. We or they may not be aware of all such intellectual property rights potentially relating to our product candidates and their manufacture and uses. Thus, we do not know with certainty that Fovista, Zimura or any other product candidate, or our intended commercialization thereof, does not and will not infringe or otherwise violate any third party s intellectual property.

If we are or one of our collaboration or commercialization partners is found to infringe or otherwise violate a third party s intellectual property rights, we or they could be required to obtain a license from such third party to continue developing and marketing our or their products and technology or to continue using a trademark. However, we or our collaboration and commercialization partners may not be able to obtain any required license on commercially reasonable terms or at all. Even if we or they were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us or our collaboration and commercialization partners and could require us or them to make substantial licensing and royalty payments. We or our collaboration and commercialization partners could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages, including treble damages and attorneys fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us or our collaboration and commercialization partners from commercializing our or their product candidates or force us or them to cease some of our business operations, which could materially harm our business. Claims that we or our collaboration and commercialization partners have misappropriated the confidential information or trade secrets of third parties could expose us or them to similar liabilities and have a similar negative impact on our business.

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We may be subject to claims by third parties asserting that we or our employees have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our employees and contractors were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees and contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees or contractors have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee s or contractor s former employer. Litigation may be necessary to defend against these claims.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact conceives or develops intellectual property that we regard as our own. Moreover, because we acquired rights to Fovista from Eyetech, Archemix and Nektar and rights to Zimura from Archemix, we must rely upon these parties practices, and those of their predecessors, with regard to the assignment of intellectual property therein. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel.

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

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

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

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and patent offices in foreign countries in several stages over the lifetime of the patent. The USPTO and patent offices in foreign countries require compliance with a number of procedural, documentary, fee payment and other requirements during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of a patent or patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, our competitors might be able to enter the market, which would have a material adverse effect on our business.

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

In addition to seeking patents for some of our technology and products, we also rely upon trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees

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and consultants. We cannot guarantee that we have executed such agreements with each party that may have or have had access to our trade secrets. Moreover, because we acquired certain rights to Fovista from Eyetech, Archemix and Nektar, we must rely upon these parties practices, and those of their predecessors, with regard to the protection of Fovista-related trade secrets before we acquired them. Any party with whom we or they have executed a non-disclosure and confidentiality agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Our proprietary information may also be obtained by third parties by other means, such as breaches of our physical or computer security systems.

Detecting the disclosure or misappropriation of a trade secret and enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

Risks Related to Regulatory Approval and Other Legal Compliance Matters

If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize Fovista, Zimura or any other product candidate that we may develop, and our ability to generate revenue will be materially impaired.

Our product candidates, including Fovista and Zimura, and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries.

Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market Fovista, Zimura or any other product candidate from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely upon third-party CROs and Novartis to assist us in this process. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate s safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. The FDA or other regulatory authorities may determine that Fovista, Zimura or any other product candidate that we may develop is not effective, is only moderately effective or has undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use. The FDA or other regulatory authority may limit the approval of Fovista to use with only specified anti-VEGF drugs rather than with all anti-VEGF drugs. Such limitation could limit sales of Fovista.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

Marketing approval of novel product candidates such as Fovista and Zimura manufactured using novel manufacturing processes can be more expensive and take longer than for other, more well-known or extensively studied pharmaceutical or biopharmaceutical products, due to regulatory agencies lack of experience with them. We believe that the FDA has only granted marketing approval for one aptamer product to date. This lack of experience may lengthen the regulatory review process, require us to conduct additional studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post-approval limitations or restrictions.

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If we experience delays in obtaining approval or if we fail to obtain approval of Fovista, Zimura or any other product candidate that we develop, the commercial prospects for such product candidate may be harmed and our ability to generate revenues will be materially impaired.

A fast track designation or grant of priority review status by the FDA may not actually lead to a faster development or regulatory review or approval process.

In the United States, our lead product candidate, Fovista, received fast track designation and may be eligible for priority review status. If a drug is intended for the treatment of a serious or life-threatening disease or condition and the drug demonstrates the potential to address unmet medical needs for this disease or condition, the drug sponsor may apply for FDA fast track designation. If a drug offers major advances in treatment, the drug sponsor may apply for FDA priority review status. The FDA has broad discretion whether or not to grant fast track designation or priority review status, so even if we believe a particular product candidate is eligible for such designation or status the FDA could decide not to grant it. Even though Fovista has received fast track designation for the treatment of wet AMD and may be eligible for priority review status, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program.

A breakthrough therapy designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval

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

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

Failure to obtain marketing approval in international jurisdictions would prevent our product candidates from being marketed abroad.

In order to market and sell Fovista, Zimura and any other product candidate that we may develop in the European Union and many other jurisdictions, we or our third-party commercialization partners, including Novartis, must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the

United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We or our third-party commercialization partners, including Novartis, may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We and our third party commercialization partners may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market.

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Any product candidate, including Fovista and Zimura, for which we obtain marketing approval could be subject to post-marketing restrictions or withdrawal from the market and we or our third-party commercialization partners may be subject to penalties if we or our third-party commercialization partners fail to comply with regulatory requirements or if we or our third-party commercialization partners experience unanticipated problems with our products, when and if any of them are approved.

Any product candidate, including Fovista and Zimura, for which we or our commercialization partners obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to manufacturing, quality control, quality assurance, complaints and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or may be subject to significant conditions of approval.

The FDA may also impose requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the product, including the adoption and implementation of risk evaluation and mitigation strategies. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling and regulatory requirements. The FDA imposes stringent restrictions on manufacturers communications regarding off-label use and if we do not restrict the marketing of our products only to their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws.

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

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions and warnings in the labeling and marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing clinical trials;
- warning or untitled letters;

- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Non-compliance with European Union requirements regarding safety monitoring or pharmacovigilance can also result in significant financial penalties. Similarly, failure to comply with the European Union s requirements regarding the protection of personal information can lead to significant penalties and sanctions.

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

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of any product candidates, including Fovista, for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us and our commercialization partners to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we and our commercialization partners market, sell and distribute any products for which we or they obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

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

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

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

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

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

- the federal transparency requirements under the Patient Protection and Affordable Care Act, as amended by the Heath Care and Education Affordability Reconciliation Act, and analogous state laws require manufacturers of drugs, devices, biologics and medical supplies to report information related to payments and other transfers of value to physicians and teaching hospitals and physician ownership and investment interests; and

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

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

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

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

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of Fovista, Zimura or any other product candidate that we may develop, restrict or regulate post- approval activities and affect our and our commercialization partners ability to generate revenue from, sell profitably or commercialize any product candidates, including Fovista and Zimura, for which we or they obtain marketing approval or products that we may develop or in-license. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we or our commercialization partners receive for any approved product.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or MMA, changed the way Medicare covers and pays for pharmaceutical products and could decrease the coverage and price that we receive for any approved products. While the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively ACA. Among the provisions of ACA of importance to our potential products are the following:

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;

- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;

- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance;

- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices;

- extension of manufacturers Medicaid rebate liability;

- expansion of eligibility criteria for Medicaid programs;

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

- new requirements to report financial arrangements with physicians and teaching hospitals;

- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and

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

In addition, other legislative changes have been proposed and adopted since ACA was enacted. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect on April 1, 2013, and will remain in effect through 2024. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding. Additionally, current legal challenges to the ACA could adversely affect coverage and/or reimbursement.

Legislative and regulatory proposals have been made to expand post- approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, or in-licensed products, if any, may be.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenues, if any.

The pricing of prescription pharmaceuticals is also subject to governmental control outside of the United States. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we or our commercialization partners may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

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

We and our third-party manufacturers are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and produce hazardous waste products. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain workers compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Further, with respect to the operations of our third-party contract manufacturers, it is possible that if they fail to operate in compliance with applicable environmental, health and safety laws and regulations or properly dispose of wastes associated with our products, we could be held liable for any resulting damages, suffer reputational harm or experience a disruption in the manufacture and supply of our product candidates or products.

Risks Related to Employee Matters and Managing Growth and Our Operations

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

We are highly dependent on David R. Guyer, M.D., our Chief Executive Officer, Samir Patel, M.D., our President, and Michael G. Atieh, our Chief Financial and Business Officer, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into employment agreements with our executive officers, each of them may terminate their employment with us at any time. We do not maintain key person insurance for any of our executives or other employees.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain marketing approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms, if at all, given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

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We are rapidly expanding our development, regulatory and sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We are currently experiencing significant and rapid growth in the number of our employees and the scope of our operations, particularly in the areas of clinical development and manufacturing development. Between January 1, 2014 and December 31, 2014, we hired more than half of our 70 employees. We also expect to continue to hire additional employees and expand the scope of our operations in the area of clinical development and, as we approach potential marketing approval for any of our product candidates, in the area of sales, marketing and distribution. To manage our growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the inherent challenges associated with managing such rapid growth, we may not be able to manage effectively the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Risks Related to Information Technology

We rely significantly upon information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cyber security incidents, could harm our ability to operate our business effectively.

Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. System failures, accidents or security breaches could cause interruptions in our operations, and could result in a material disruption of our clinical and commercialization activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. The loss of clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate public disclosure of confidential or proprietary information, we could incur liability and our product research, development and commercialization efforts could be delayed.

Risks Related to Our Common Stock

Our executive officers, directors and principal stockholders maintain the ability to significantly influence all matters submitted to stockholders for approval.

As of December 31, 2014, our executive officers, directors and principal stockholders and their affiliates, in the aggregate, beneficially owned shares representing approximately 30% of our capital stock. As a result, if these stockholders were to choose to act together, they would be able to significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, could significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire.

A significant portion of our total outstanding shares may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.

A significant portion of our total outstanding shares may be sold into the market in the near future, which could cause the market price of our common stock to drop 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 that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. As of December 31, 2014, we had outstanding 33,994,520 shares of common stock. Of these shares, approximately 10,258,000 shares are restricted securities under Rule 144 under the Securities Act. Any of our remaining shares that are not restricted securities under Rule 144 under the Securities Act, including, for example, shares sold in our initial public offering or our follow-on public offering, may be resold in the public market without restriction unless purchased by our affiliates. Moreover, holders of an aggregate of approximately 10,170,000 shares of our common stock, including shares issuable pursuant to outstanding warrants, have rights, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We have filed, or

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plan to file, registration statements on Form S-8 registering all shares of common stock that we may issue under our equity compensation plans prior to awards becoming exercisable. As of December 31, 2014, we had outstanding stock options to purchase an aggregate of approximately 3,680,000 shares of our common stock, of which options to purchase approximately 993,000 shares were vested. Once registered on Form S-8, these shares can be freely sold in the public market upon issuance, subject to volume, notice and manner of sale limitations applicable to affiliates and the applicable lock-up agreements entered into in connection with our public offerings.

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

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

- provide for a classified board of directors such that only one of three classes of directors is elected each year;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board of directors;
- provide for advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a poison pill that would work to dilute the stock ownership of a potential hostile acquirer, effectively

preventing acquisitions that have not been approved by our board of directors; and

- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our certificate of incorporation or by-laws.

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

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

Our stock price may be volatile. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their shares of common stock at or above the price at which they purchased their shares. The market price for our common stock may be influenced by many factors, including:

- the success of competitive products or technologies;

- results of clinical trials of Fovista, Zimura and any other product candidate that we may develop;

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- results of clinical trials of product candidates of our competitors;

- regulatory or legal developments in the United States and other countries;

- developments or disputes concerning patent applications, issued patents or other proprietary rights;

- the recruitment or departure of key personnel;

- the level of expenses related to any of our product candidates or clinical development programs;

- the results of our efforts to in-license or acquire the rights to other products, product candidates and technologies for the treatment of ophthalmic diseases, the costs of commercializing any such products and the costs of development of any such product candidates or technologies;

- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;

- variations in our financial results or those of companies that are perceived to be similar to us;

- changes in the structure of healthcare payment systems;

- market conditions in the pharmaceutical and biotechnology sectors;

- general economic, industry and market conditions; and

- the other factors described in this Risk Factors section.

In the past, following periods of volatility in the market price of a company s securities, securities class-action litigation has often been instituted against that company. We also may face securities class-action litigation if we cannot obtain regulatory approvals for or if we otherwise fail to commercialize Fovista. Such litigation, if instituted against us, could cause us to incur substantial costs to defend such claims and divert management s attention and resources, which could seriously harm our business.

We incur increased costs as a result of operating as a public company, and our management is now required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, we incur and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The NASDAQ Global Select Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations have increased our legal and financial compliance costs and will make some activities more time-consuming and costly.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to furnish with our periodic Exchange Act reports a report by our management on our internal control over financial reporting. We are also required to include with our annual report an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404, we are engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe, or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

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

We have never declared or paid cash dividends on our common stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be our stockholders sole source of gain for the foreseeable future.

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PART II

Item 6. Selected Financial Data

The following selected financial data should be read together with our financial statements and the related notes appearing elsewhere in this Annual Report on Form 10-K/A and the Management s Discussion and Analysis of Financial Condition and Results of Operations section of this Annual Report on Form 10-K/A. We have derived the statements of operations data and the balance sheet data appearing below from our audited financial statements included elsewhere in this Annual Report on Form 10-K/A or from other audited financial statements appearing in filings made with the Securities and Exchange Commission, all of which have been audited by Ernst & Young LLP, an independent registered public accounting firm. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period, and our results for any interim period are not necessarily indicative of results to be expected for a full fiscal year.

	Year Ended December 31,			
	2014 (Restated)(1)	2013	2012	2011
	(in thousands)			
Statement of Operations Data:				
Collaboration revenue	$ 41,259	$	$	$
Operating Expenses:				
Research and development	88,385	33,215	6,792	13,896
General and administrative	33,387	14,210	6,889	5,738
Total operating expenses	121,772	47,425	13,681	19,634
Loss from operations	(80,513)	(47,425)	(13,681)	(19,634)
Interest (expense) income	217	(1,454)	(507)	2
Loss on extinguishment of debt		(1,091)		
Other loss		(1,175)	(374)	(30)
Net loss before income tax provision	(80,296)	(51,145)	(14,562)	(19,662)
Income tax provision (benefit) (1)	36,476			(1,029)
Net loss (1)	(116,772)	(51,145)	(14,562)	(18,633)
Add: accretion of preferred stock dividends		(5,891)	(7,063)	(6,838)
Net loss attributable to common stockholders (1)	$ (116,772)	$ (57,036)	$ (21,625)	$ (25,471)
Net loss attributable to common stockholders per share:				
Basic and diluted(1)	$ (3.51)	$ (6.34)	$ (14.89)	$ (18.27)
Weighted average common shares outstanding:				
Basic and diluted	33,258	9,003	1,452	1,394

	As of December 31,			
	2014 (Restated)(1)	2013	2012	2011
	(in thousands)			
Balance sheet data:				
Cash, cash equivalents and available for sale securities	$ 463,560	$ 210,596	$ 4,304	$ 6,396
Deferred tax asset (1)	$ 50	$	$	$
Total current assets (1)	$ 473,382	$ 217,400	$ 4,837	$ 7,497
Deferred tax assets, non-current (1)	$ 4,467	$	$	$
Total assets(1)	$ 479,786	$ 217,682	$ 4,879	$ 7,728
Deferred revenue	$ 209,624	$	$	$

Royalty purchase liability	$	125,000	$	41,667	$		$	
Total liabilities	$	351,249	$	47,962	$	14,410	$	3,338
Preferred stock	$		$		$	113,939	$	106,877
Additional paid-in capital	$	428,390	$	352,739	$		$	
Accumulated deficit(1)	$	(299,822)	$	(183,050)	$	(126,471)	$	(105,488)
Total stockholders equity (deficit)(1)	$	128,537	$	169,720	$	(123,470)	$	(102,487)
Total liabilities and stockholder equity (deficit) (1)	$	479,786	$	217,682	$	4,879	$	7,728

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(1) Amounts have been restated to correct errors in the accounting for valuation allowances related to deferred tax assets as described in Note 18 of our financial statements for the year ended December 31, 2014 contained elsewhere in this Annual Report.

Item 7. Management s Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis of our financial condition and results of operations should be read together with our financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K/A. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K/A, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties and should be read together with the Risk Factors section of this Annual Report on Form 10-K/A for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Restatement of Previously Issued Financial Statements

Financial data when presented throughout this Management s Discussion and Analysis of Financial Condition and Results of Operations includes the effect of the restatement of our balance sheets and our statements of operations as of and for the three and six months ended June 30, 2014, as of and for the three and nine months ended September 30, 2014, as of and for the three months and year ended December 31, 2014, as described in Notes 2, 4, 10, 17 and 18 to our audited financial statements appearing elsewhere in this Annual Report. The following tables summarize the effect of the restatement by major financial statement line item for these periods. The restatement reflected below did not impact our cash position or operating results.

Balance Sheet

	June 30, 2014		September 30, 2014		December 31, 2014	
	As Reported (unaudited)	As Restated	As Reported (unaudited)	As Restated	As Reported	As Restated
Deferred tax assets	$ 837	$	$ 1,069	$ 49	$ 293	$ 50
Total current assets	$ 442,155	$ 441,318	$ 462,644	$ 461,624	$ 473,625	$ 473,382
Deferred tax assets, non-current	$ 19,635	$	$ 26,187	$ 4,404	$ 22,808	$ 4,467
Total assets	$ 478,126	$ 457,654	$ 490,486	$ 467,683	$ 498,370	$ 479,786
Income taxes payable	$ 29,540	$ 29,559	$ 7,785	$ 7,785	$	$
Total current liabilities	$ 35,362	$ 35,381	$ 24,039	$ 24,039	$ 19,831	$ 19,831
Total liabilities	$ 318,695	$ 318,714	$ 314,591	$ 314,591	$ 351,249	$ 351,249
Accumulated deficit	$ (256,231)	$ (276,722)	$ (245,367)	$ (268,170)	$ (281,238)	$ (299,822)
Total stockholders equity	$ 159,431	$ 138,940	$ 175,895	$ 153,092	$ 147,121	$ 128,537
Total liabilities and stockholders equity	$ 478,126	$ 457,654	$ 490,486	$ 467,683	$ 498,370	$ 479,786

Statements of Operations

	Three Months Ended June 30, 2014		Six Months Ended June 30, 2014	
	As Reported	As Restated	As Reported	As Restated
	(unaudited)		(unaudited)	
Income tax provision	$ 10,294	$ 30,785	$ 10,294	$ 30,785
Net loss	$ (52,499)	$ (72,990)	$ (73,181)	$ (93,672)
Basic loss per common share	$ (1.57)	$ (2.19)	$ (2.23)	$ (2.85)
Diluted loss per common share	$ (1.57)	$ (2.19)	$ (2.23)	$ (2.85)

	Three Months Ended September 30, 2014		Nine Months Ended September 30, 2014	
	As Reported	As Restated	As Reported	As Restated
	(unaudited)		(unaudited)	
Income tax provision	$ 2,867	$ 5,179	$ 13,161	$ 35,964
Net income (loss)	$ 10,864	$ 8,552	$ (62,317)	$ (85,120)
Basic earnings (loss) per common share	$ 0.32	$ 0.26	$ (1.88)	$ (2.57)
Diluted earnings (loss) per common share	$ 0.31	$ 0.25	$ (1.88)	$ (2.57)

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	Three Months Ended December 31, 2014		Year Ended December 31, 2014	
	As Reported (unaudited)	As Restated	As Reported	As Restated
Income tax provision	$ 4,731	$ 512	$ 17,892	$ 36,476
Net loss	$ (35,871)	$ (31,652)	$ (98,188)	$ (116,772)
Basic loss per common share	$ (1.06)	$ (0.94)	$ (2.95)	$ (3.51)
Diluted loss per common share	$ (1.06)	$ (0.94)	$ (2.95)	$ (3.51)

Statements of Comprehensive Income (Loss)

	Three Months Ended June 30, 2014		Three Months Ended September 30, 2014	
	As Reported (unaudited)	As Restated	As Reported (unaudited)	As Restated
Net income (loss)	$ (52,499)	$ (72,990)	$ 10,864	$ 8,552
Comprehensive income (loss)	$ (52,492)	$ (72,983)	$ 10,874	$ 8,562

	Six Months Ended June 30, 2014		Nine Months Ended September 30, 2014		Year Ended December 31, 2014	
	As Reported (unaudited)	As Restated	As Reported (unaudited)	As Restated	As Reported (unaudited)	As Restated
Net loss	$ (73,181)	$ (93,672)	$ (62,317)	$ (85,120)	$ (98,188)	$ (116,772)
Comprehensive loss	$ (73,156)	$ (93,647)	$ (62,282)	$ (85,085)	$ (98,253)	$ (116,837)

Statement of Cash Flows

	Six Months Ended June 30, 2014		Nine Months Ended September 30, 2014		Year Ended December 31, 2014	
	As Reported (unaudited)	As Restated	As Reported (unaudited)	As Restated	As Reported (unaudited)	As Restated
Net loss	$ (73,181)	$ (93,672)	$ (62,317)	$ (85,120)	$ (98,188)	$ (116,772)
Deferred income taxes	$ (19,246)	$ 1,226	$ (24,947)	$ (2,144)	$ (18,798)	$ (214)
Income taxes payable	$ 29,540	$ 29,559	$ 7,785	$ 7,785	$	$

The restatement did not have any effect on the previously reported amounts of total operating, investing or financing cash flows in our statements of cash flows for the six months ended June 30, 2014, the nine months ended September 30, 2014, and the year ended December 31, 2014.

Overview

We are a biopharmaceutical company specializing in the development of novel therapeutics to treat diseases of the back of the eye, with a focus on developing therapeutics for age-related macular degeneration, or AMD. Our most advanced product candidate is Fovista, which is in Phase 3 clinical development for use in combination with anti-VEGF drugs that represent the current standard of care for the treatment of wet AMD. We have completed one Phase 1 and one Phase 2b clinical trial of Fovista administered in combination with the anti-VEGF drug Lucentis. We are also developing our product candidate Zimura, for the treatment of patients with geographic atrophy, a form of dry AMD, and in combination with anti-VEGF therapy and, potentially, Fovista, for the treatment of anti-VEGF resistant wet AMD patients who are believed to have complement mediated inflammation.

We have initiated a pivotal Phase 3 clinical program for Fovista, which consists of three separate Phase 3 clinical trials to evaluate the safety and efficacy of Fovista administered in combination with anti-VEGF drugs for the treatment of wet AMD compared to anti-VEGF monotherapy and are actively enrolling patients in these trials. Two of these trials are evaluating Fovista in combination with Lucentis and the other is evaluating Fovista in combination with each of Eylea or Avastin. We plan to enroll a total of 1,866 patients at more than 225 centers internationally across the three trials. Based on our estimates regarding patient enrollment, we expect to have initial, top-line data from our Phase 3 clinical program for Fovista available in 2016. If the results of this Phase 3 clinical program are favorable, we will submit applications for marketing approval for Fovista in the United States and, together with our commercialization partner Novartis, in the European Union. We also initiated, in the third quarter of 2014, a Phase 2a open-label trial designed to investigate the potential effect of the administration of Fovista in combination with anti-VEGF therapy in reducing the formation of subretinal fibrosis in wet AMD patients, and, in the fourth quarter of 2014, a Phase 2a clinical trial, which will evaluate the potential benefit of Fovista in wet AMD, when administered in combination with anti-VEGF drugs, in the reduction of treatment burden. We are also planning additional clinical trials to assess the potential therapeutic benefit of Fovista in other ophthalmic conditions.

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On May 19, 2014, we entered into a licensing and commercialization agreement with Novartis Pharma AG, which we refer to as the Novartis Agreement. Under the Novartis Agreement, we granted Novartis exclusive rights under specified patent rights, know-how and trademarks controlled by us to manufacture, from bulk API supplied by us, standalone Fovista products and products combining Fovista with an anti-VEGF product to which Novartis has rights in a co-formulated product, for the treatment, prevention, cure or control of any human disease, disorder or condition of the eye, and to develop and commercialize those licensed products in all countries outside of the United States, which we refer to as the Novartis Territory. We have agreed to use commercially reasonable efforts to complete our ongoing pivotal Phase 3 clinical program for Fovista and Novartis has agreed to use commercially reasonable efforts to develop a standalone Fovista product and a co-formulated product containing Fovista and an anti-VEGF to which Novartis has rights, as well as a pre-filled syringe presentation of such products and to use commercially reasonable efforts, subject to obtaining marketing approval, to commercialize licensed products in the Novartis Territory in accordance with agreed development and marketing plans.

Novartis paid us a $200.0 million upfront fee upon execution of the Novartis Agreement. Novartis is also obligated to pay us up to an aggregate of $130.0 million if we achieve specified patient enrollment milestones for our Phase 3 clinical program for Fovista, $50.0 million of which we received in October 2014, and up to an aggregate of an additional $300.0 million upon achievement of specified marketing approval milestones in certain countries in the Novartis Territory. In addition, Novartis has agreed to pay us up to an aggregate of an additional $400.0 million if Novartis achieves specified sales milestones in the Novartis Territory. Novartis also is obligated to pay us royalties with respect to standalone Fovista products and combination Fovista products that Novartis successfully commercializes. We will receive royalties at a mid-thirties percentage of net sales of standalone Fovista products and a royalty of approximately equal value for sales of combination Fovista products. Such royalties are subject to customary deductions, credits, and reductions for lack of patent coverage or market exclusivity. Novartis s obligation to pay such royalties will continue on a licensed product-by-licensed product and country-by-country basis until Novartis s last actual sale of such licensed product in such country. We will continue to be responsible for royalties we owe to third parties on sales of Fovista products.

We have retained control over the design and execution of our pivotal Phase 3 clinical program for Fovista and remain responsible for funding the costs of that program, subject to Novartis s responsibility to provide Lucentis, an anti-VEGF agent to which Novartis has rights in the Novartis Territory, for use in the Phase 3 trials in the Novartis Territory following the effective date of the Novartis Agreement. Novartis will have control over, and will be responsible for the costs of, all other clinical trials that may be required to obtain marketing approvals in the Novartis Territory for licensed products under the agreement. Novartis is also responsible for costs associated with co-formulation development, pre-filled syringe development and other development costs in the Novartis Territory, but excluding regulatory filing fees in the European Union for the standalone Fovista product, for which we will be responsible.

We plan to initiate a Phase 2/3 clinical trial to evaluate the safety and efficacy of Zimura monotherapy in patients with geographic atrophy in the second half of 2015. We also initiated in late 2014 a very small, open-label Phase 2 trial investigating Zimura administered in combination with anti- VEGF therapy for the treatment of polypoidal choroidal vasculopathy, or PCV, a specific type of wet AMD, in patients who do not respond adequately to treatment with anti-VEGF monotherapy or for whom anti-VEGF monotherapy has failed.

We were incorporated and commenced active operations in 2007. Our operations to date have been primarily limited to organizing and staffing our company, acquiring rights to product candidates, business planning, raising capital and developing Fovista and Zimura. We acquired our rights to Fovista from (OSI) Eyetech, Inc., or Eyetech, in July 2007. The acquisition included an assignment of license rights and obligations under an agreement with Archemix Corp. We have licensed rights to our product candidate Zimura from Archemix Corp. Since inception, we have incurred significant operating losses. Our net loss was $116.8 million for the year ended December 31, 2014, and $51.1 million for the year ended December 31, 2013. As of December 31, 2014, we had an accumulated deficit of $299.8 million. We have not generated any revenues from product sales and have financed our operations primarily through private placements of our preferred stock, venture debt borrowings, funding under our royalty purchase and sale agreement with Novo A/S, which we refer to as the Novo Agreement, our initial public offering, which we closed in September 2013, our follow-on public offering of common stock, which we closed in February 2014, and the Novartis Agreement. We received net proceeds from the initial public offering of $175.6 million, after deducting underwriting discounts and commissions and other offering expenses payable by us. We received net proceeds from the follow-on public offering of $55.4 million, after deducting underwriting discounts and commissions and other offering expenses payable by us. We have received $125.0 million of funding under the Novo Agreement, which constitutes the full amount of funding under that agreement. We also received an upfront payment of $200.0 million from Novartis upon the execution of the Novartis Agreement and a $50.0 million enrollment-based milestone payment from Novartis in October 2014. In connection with the receipt of the upfront payment from Novartis, we made a milestone payment in June 2014 of approximately $19.8 million under one of our agreements. We made income tax payments

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of $40.2 million during the year ended December 31, 2014. These payments relate to estimated taxable income that resulted from the receipt of the $250.0 million in upfront and milestone payments from Novartis and $83.3 million in proceeds from the Novo Agreement in 2014.

We initiated our pivotal Phase 3 clinical program for Fovista in August 2013. We expect our expenses to continue to increase substantially, particularly as we continue the development of Fovista in our Phase 3 clinical program for the treatment of wet AMD. We plan to enroll a total of 1,866 patients for this program. In addition, we also expect our expenses to increase as we further evaluate the potential benefit of Fovista in wet AMD, when administered in combination with anti-VEGF drugs, and in other ophthalmic diseases and conditions with unmet need and pursue the development of Zimura for both the treatment of geographic atrophy, a form of dry AMD, and in combination with anti-VEGF therapy and, potentially, Fovista, for the treatment of anti- VEGF resistant wet AMD patients who are believed to have complement mediated inflammation. We expect these expenses to increase as patient enrollment increases in these clinical trials and as we manufacture validation production batches of API and drug product for Fovista. In addition, our expenses will increase prior to obtaining marketing approval for Fovista as we expand our infrastructure to support commercial operations and, if we obtain marketing approval for Fovista, Zimura or any other product candidate that we may develop, we expect our commercialization expenses related to product sales, marketing, distribution and manufacturing to increase significantly. Furthermore, we are incurring and will continue to incur additional costs associated with operating as a public company, hiring additional personnel and expanding our facilities. These costs include significant legal, accounting, investor relations and other expenses that we did not incur as a private company. Moreover, additional rules and regulations applicable to public companies will increase our legal and financial compliance costs and will make some activities more time-consuming and costly.

Our ability to become and remain profitable depends on our ability to generate revenue in excess of our expenses. We do not expect to generate and maintain significant product revenue unless, and until, we obtain marketing approval for, and commercialize, Fovista, Zimura or other product candidates that we may develop. We may be unsuccessful in our efforts to develop and commercialize these product candidates. Even if we succeed in developing and commercializing one or more of our product candidates, we may never achieve sufficient sales revenue to achieve or maintain profitability. Our capital requirements will also depend on many other factors, including whether we pursue the acquisition or in-licensing and subsequent development of additional product candidates. We may need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

Financial Overview

Revenue

Prior to 2014, we had not generated any revenue. In September 2014, we earned a $50.0 million enrollment-based milestone under the Novartis Agreement. We recognized approximately $41.3 million as collaboration revenue during the year ended December 31, 2014. Using the relative selling price method, the Company allocated revenue of $38.4 million to the license it delivered to Novartis under the Novartis Agreement, $2.0 million to the research and development activities we performed under the Novartis Agreement, and $0.9 million to the transfer of API to Novartis. In the future, we may generate additional revenue from a combination of product sales and license fees, milestone payments and research and development activity-related payments and royalties in connection with the Novartis Agreement. We expect that any revenue we generate will fluctuate from quarter to quarter as a result of the timing and amount of certain milestone and other payments, if any, that we may receive from Novartis and the amount and timing of payments that we receive upon the sale of our products, to the extent any are successfully commercialized. We do not expect to generate revenue from products sales until 2017 at the earliest. If we fail to complete the development of Fovista, Zimura or other product candidates we may develop, in a timely manner or obtain regulatory approval for them, our ability to generate future revenue and our results of operations and financial position, would be materially adversely affected.

Research and Development Expenses

Research and development expenses primarily consist of costs associated with the development and clinical testing of Fovista and Zimura. Our research and development expenses consist of:

- external research and development expenses incurred under arrangements with third parties, such as contract research organizations, or CROs, and other vendors, contract manufacturing organizations and consultants, including for the production of drug substance and drug product; and

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

All research and development costs are charged to operations as incurred in accordance with Financial Accounting Standards Board Accounting Standards Codification, or ASC, 730 Topic, *Research and Development*. We account for non-refundable advance payments for goods and services that will be used in future research and development activities as expenses when the service has been performed or when the goods have been received, rather than when the payment is made.

To date, the large majority of our research and development work has been related to Fovista and Zimura. We anticipate that our research and development expenses will increase substantially in connection with our ongoing activities, particularly as we continue the development of and seek marketing approval for Fovista, Zimura and, possibly, other product candidates.

We do not currently utilize a formal time allocation system to capture expenses on a project-by-project basis because we record expenses by functional department. Accordingly, we do not allocate expenses to individual projects or product candidates, although we do allocate some portion of our research and development expenses by functional area and by compound, as shown below.

The following table summarizes our research and development expenses for the years ended December 31, 2014, 2013 and 2012:

	Years ended December 31,		
	2014	**2013**	**2012**
		(in thousands)	
Fovista	$ 66,095	$ 26,206	$ 3,619
Zimura	4,377	15	36
Personnel-related	9,514	4,770	2,725
Share-based compensation	7,594	2,062	412
Other	805	162	
	$ 88,385	$ 33,215	$ 6,792

We expect to spend significant additional funds on our Phase 3 clinical program for Fovista, our other planned clinical programs, including additional clinical trials to further evaluate the potential benefit of Fovista in wet AMD, when administered in combination with anti-VEGF drugs, and in other ophthalmic diseases and conditions with unmet medical need, a planned clinical trial evaluating Zimura for the treatment of geographic atrophy and a clinical trial evaluating Zimura administered in combination with anti-VEGF therapy and, potentially, Fovista, for the treatment of anti-VEGF resistant wet AMD patients who are believed to have complement mediated inflammation, and for general corporate purposes and working capital. Costs related to our clinical programs could exceed our expectations if we experience delays in our clinical trials, including because of the timing of our patient enrollment, the availability and costs of drug supply for our clinical trials or for other reasons. Our costs will also increase if we increase investigator fees for our clinical trials or expand the scope of our clinical trials and programs, or change the geographic mix of sites at which patients are enrolled, or increase other corporate or licensing activities, or staffing.

Our current Phase 3 clinical program for Fovista is expected to continue through at least 2017, and substantial expenditures to complete the Phase 3 clinical program will be required after the receipt of initial, top-line data and to fund our other development programs. Moreover, we are at the early stages of formulating our clinical development plan for Zimura. We expect the clinical development of Zimura will continue for at least the next several years. At this time, we cannot reasonably estimate the remaining costs necessary to complete the clinical development of either Fovista or Zimura, complete process development and manufacturing scale-up activities associated with Fovista and Zimura and seek marketing approval for Fovista or Zimura, or the nature, timing or costs of the efforts necessary to complete the development of any other product candidate we may develop.

The successful development of our product candidates is highly uncertain. See Risk Factors. This is due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

- the scope, rate of progress and expense of our research and development activities;
- the potential benefits of our product candidates over other therapies;

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- our and our commercialization partner s ability to market, commercialize and achieve market acceptance for any of our product candidates;
- clinical trial results;
- the terms and timing of regulatory approvals; and
- our ability to successfully file, prosecute, defend and enforce patent claims and other intellectual property rights, together with associated expenses.

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

See the Liquidity and Capital Resources section on page 54 of this Annual Report on Form 10-K/A for more information regarding our current and future financial resources.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related costs for personnel, including share-based compensation expense, in our executive, legal, finance and business development functions. Other general and administrative expenses include facility costs and professional fees for legal, patent, consulting and accounting services.

We anticipate that our general and administrative expenses will increase in future periods to support increases in our research and development, manufacturing, and commercialization activities and as a result of increased personnel, including management personnel to support our clinical, manufacturing and commercialization activities, expanded infrastructure, increased legal, compliance, accounting and investor and public relations expenses associated with being a public company and increased insurance premiums, among other factors.

Interest Income

Our cash, cash equivalents and marketable securities are invested primarily in U.S. Treasury money market funds and U.S. Treasury securities, which generate a nominal amount of interest income.

Critical Accounting Policies and Significant Judgments and Estimates

Our management s discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued expenses and share-based compensation described in greater detail below. We base our estimates on our limited historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

Our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this Annual Report on Form 10-K/A. Of those policies, we believe that the following accounting policies are the most critical to aid our stockholders in fully understanding and evaluating our financial condition and results of operations.

Accrued Research and Development Expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued expenses. This process involves reviewing quotations and contracts, identifying services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. The significant estimates in our accrued research and development expenses are related to expenses incurred with respect to CROs, contract manufacturing organizations and other vendors in connection with research and development activities for which we have not yet been invoiced.

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We base our expenses related to CROs and contract manufacturing organizations on our estimates of the services received and efforts expended pursuant to quotes and contracts with such vendors that conduct research and development activities on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the research and development expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in us reporting amounts that are too high or too low in any particular period. There have been no material changes in estimates for the periods presented.

Revenue Recognition

Collaboration Revenue

Prior to 2014, we had not generated any revenue. In May 2014, we received an up-front payment of $200.0 million in connection with the Novartis Agreement, which has not been recorded as revenue due to certain contingencies associated with the payment. In September 2014, we achieved a $50.0 million enrollment-based milestone under the Novartis Agreement. We recognized collaboration revenue of approximately $41.3 million during the year ended December 31, 2014. We use the relative selling price method to allocate arrangement consideration to our performance obligations under the Novartis Agreement. Below is a summary of the components of our collaboration revenue for the years ended December 2014, 2013 and 2012:

	Year ended December 31, 2014	2013	2012
License revenue	$ 38,372	$	$
Research and development activity revenue	2,004		
API transfer revenue	883		
Total collaboration revenue	$ 41,259	$	$

In the future, we may generate additional revenues from a combination of products sales and license fees, milestone payments and research and development activity-related payments and royalties in connection with the Novartis Agreement. The terms of this agreement and other potential collaboration or commercialization agreements we may enter into generally contain multiple elements, or deliverables, which may include (i) licenses, or options to obtain licenses, to our technology, (ii) research and development activities to be performed on behalf of the collaborative partner, and (iii) in certain cases, services in connection with the manufacturing of pre-clinical and clinical material. Payments to us under these arrangements typically include one or more of the following: non-refundable, up-front license fees; option exercise fees; funding of research and/or development efforts; milestone payments; and royalties on future product sales.

When evaluating multiple element arrangements, we consider whether the deliverables under the arrangement represent separate units of accounting. This evaluation requires subjective determinations and requires management to make judgments about the individual deliverables and whether such deliverables are separable from the other aspects of the contractual relationship. In determining the units of accounting, management evaluates certain criteria, including whether the deliverables have standalone value, based on the consideration of the relevant facts and circumstances for each arrangement. The consideration received is allocated among the separate units of accounting using the relative selling price method, and the applicable revenue recognition criteria are applied to each of the separate units.

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

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evaluate whether changes in the key assumptions used to determine the best estimate of selling price will have a significant effect on the allocation of arrangement consideration among multiple deliverables.

When management believes the license to its intellectual property and products has stand-alone value, we generally recognize revenue attributed to the license upon delivery. When management believes such a license does not have stand-alone value from the other deliverables to be provided in the arrangement, we generally recognize revenue attributed to the license on a straight-line basis over our contractual or estimated performance period, which is typically the term of our research and development obligations. If management cannot reasonably estimate when our performance obligation ends, then revenue is deferred until management can reasonably estimate when the performance obligation ends. The periods over which revenue should be recognized are subject to estimates by management and may change over the course of the research and development agreement. Such a change could have a material impact on the amount of revenue we record in future periods.

At the inception of arrangements that include milestone payments, we evaluate whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether (a) the consideration is commensurate with either (1) the entity s performance to achieve the milestone, or (2) the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the entity s performance to achieve the milestone, (b) the consideration relates solely to past performance, and (c) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. We evaluate factors such as the scientific, regulatory, commercial and other risks that must be overcome to achieve the respective milestone, the level of effort and investment required to achieve the respective milestone and whether the milestone consideration is reasonable relative to all deliverables and payment terms in the arrangement in making this assessment.

We aggregate our milestones into three categories: (i) clinical and development milestones, (ii) regulatory milestones, and (iii) commercial milestones. Clinical and development milestones are typically achieved when a product candidate advances into a defined phase of clinical research or completes such phase or when a contractually specified clinical trial enrollment target is attained. Regulatory milestones are typically achieved upon acceptance of the submission for marketing approval of a product candidate or upon approval to market the product candidate by the FDA or other global regulatory authorities. For example, a milestone payment may be due to us upon the FDA s acceptance of an NDA. Commercial milestones are typically achieved when an approved pharmaceutical product reaches certain defined levels of net sales by the licensee, such as when a product first achieves global sales or annual sales of a specified amount.

Revenues from clinical and development and regulatory milestone payments, if the milestones are deemed substantive and the milestone payments are nonrefundable, are recognized upon successful accomplishment of the milestones. With regards to the Novartis Agreement, we have concluded that the clinical and development milestones and certain regulatory milestones are not substantive and that the regulatory approval milestones pursuant to the Novartis Agreement are substantive. Milestones payments received that are not considered substantive are included in the allocable arrangement consideration and are recognized as revenue in proportion to the relative-selling price allocation established at the inception of the arrangement. Revenues from commercial milestone payments are accounted for as royalties and are recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met.

Royalty Purchase Liability

The proceeds from the financing we received under the Novo Agreement have been recorded as a liability on our balance sheet in accordance with ASC Topic 730, *Research and Development*. Because there is a significant related party relationship between us and Novo A/S, we are treating our obligation to make royalty payments under the Novo Agreement as an implicit obligation to repay the funds advanced by Novo A/S, and thus have recorded the proceeds as a liability on our balance sheet. As we make royalty payments to Novo A/S in accordance with the Novo

Agreement, we will reduce the liability balance. At the time that such royalty payments become probable and estimable, and if such amounts exceed the liability balance, we will impute interest accordingly on a prospective basis based on such estimates, which would result in a corresponding increase in the liability balance.

Share-Based Compensation

We account for all share-based compensation payments issued to employees, directors, and non-employees using an option pricing model for estimating fair value. Accordingly, share-based compensation expense is measured based on the estimated fair value of the awards on the date of grant, net of forfeitures. We recognize compensation expense for the portion of the award that is ultimately expected to vest over the period during which the recipient renders the required services to us using

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the straight-line single option method. In accordance with authoritative guidance, we re-measure the fair value of non-employee share-based awards as the awards vest, and recognize the resulting value, if any, as expense during the period the related services are rendered.

We apply the fair value recognition provisions of ASC Topic 718, *Compensation Stock Compensation*. Determining the amount of share- based compensation to be recorded requires us to develop estimates of the fair value of stock options as of their grant date. We recognize share-based compensation expense ratably over the requisite service period, which in most cases is the vesting period of the award. Calculating the fair value of share- based awards requires that we make highly subjective assumptions.

We use the Black-Scholes option pricing model to value our stock option awards. Use of this valuation methodology requires that we make assumptions as to the volatility of our common stock, the expected term of our stock options, and the risk free interest rate for a period that approximates the expected term of our stock options and the expected dividend yield of our common stock. As a new public company, we do not have sufficient history to estimate the volatility of our common stock price or the expected life of the options. We calculate expected volatility based on reported data for similar publicly traded companies for which historical information is available and will continue to do so until the historical volatility of our common stock is sufficient to measure expected volatility for future option grants.

We use the simplified method as prescribed by the Securities and Exchange Commission Staff Accounting Bulletin No. 107, *Share-Based Payment*, to calculate the expected term of stock option grants to employees as we do not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term of stock options granted to employees. The risk-free interest rate used for each grant is based on the U.S. Treasury yield curve in effect at the time of grant for instruments with a similar expected life. We utilize a dividend yield of zero based on the fact that we have never paid cash dividends and have no current intention to pay cash dividends. The weighted-average assumptions used to estimate grant date fair value of stock options using the Black-Scholes option pricing model were as follows for the years ended December 31, 2014, 2013 and 2012:

	Year ended December 31,		
	2014	**2013**	**2012**
Expected common stock price volatility	82%	83%	81%
Risk-free interest rate	1.61% - 2.13%	0.89% - 2.94%	0.94% - 1.77%
Expected term of options (years)	6.2	6.1	6.6
Expected annual dividend per share	$	$	$

We are also required to estimate forfeitures at the time of grant, and revise those estimates in subsequent periods if actual forfeitures differ from our estimates. We use historical data to estimate pre-vesting option forfeitures and record share-based compensation expense only for those awards that are expected to vest. To the extent that actual forfeitures differ from our estimates, the difference is recorded as a cumulative adjustment in the period the estimates were revised. Through December 31, 2014, actual forfeitures have not been material.

Share-based compensation expense for equity grants to employees and non- employees was $13.0 million, $2.9 million, and $0.6 million for the years ended December 31, 2014, 2013 and 2012, respectively. As of December 31, 2014, we had $42.8 million of total unrecognized share-based compensation expense, which we expect to recognize over a weighted-average remaining vesting period of approximately 3.0 years. We expect our share- based compensation for our share-based awards to employees and non-employees to increase as a result of recognizing our existing unrecognized share-based compensation for awards that will vest and as we issue additional share-based awards to attract and retain our employees.

For the years ended December 31, 2014, 2013 and 2012, we allocated share- based compensation as follows:

	Year ended December 31,		
	2014	2013	2012
Research and development	$ 7,594	$ 2,062	$ 412
General and administrative	5,446	809	228
Total	$ 13,040	$ 2,871	$ 640

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Income Taxes

In each of January 2014 and November 2014, we received $41.7 million from Novo A/S under the Novo Agreement, or $83.3 million in the aggregate, which will be reported as revenue for income tax purposes. In May 2014, we received $200.0 million from Novartis upon execution of the Novartis Agreement, a portion of which will be reported as revenue for income tax purposes. In October 2014, we received a milestone payment of $50.0 million from Novartis which will be reported as revenue for income tax purposes. As a result of these payments, and after taking into account the utilization of our federal net operating loss carry-forwards and utilization of our research and development tax credits, we expect to report taxable income for the 2014 tax year. We made income tax payments of $40.2 million during the year ended December 31, 2014. These payments relate to estimated taxable income that resulted from the payments we received from Novo A/S and Novartis in 2014. In addition, we expect that the valuation allowance on certain of our deferred tax assets will be released, where appropriate. See Note 10 to our financial statements in Item 8 of this Annual Report on form 10-K/A for further information regarding our expectations with respect to our income tax provision.

JOBS Act

Prior to January 1, 2015, as an emerging growth company under the Jumpstart Our Business Startups Act of 2012, we were entitled to take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. Prior to January 1, 2015, we elected to delay our adoption of such new or revised accounting standards. As a result of this election, our financial statements for periods prior to January 1, 2015, may not be comparable to the financial statements of other public companies. Commencing in January 1, 2015, we no longer qualify for such status and, as such, we will comply with all new or revised accounting standards applicable to other public companies. We do not expect this change in status to have a material impact on our 2015 financial position or results of operations.

Results of Operations

Comparison of Years Ended December 31, 2014 and 2013

	Years ended December 31, 2014 (Restated)	2013	Increase (Decrease)
	(in thousands)		
Statement of Operations Data:			
Collaboration revenue	$ 41,259	$	$ 41,259
Operating Expenses:			
Research and development	88,385	33,215	55,170
General and administrative	33,387	14,210	19,177
Total operating expenses	121,772	47,425	74,347
Loss from operations	(80,513)	(47,425)	33,088
Interest income (expense)	217	(1,454)	1,671
Loss on extinguishment of debt		(1,091)	(1,091)
Other loss		(1,175)	(1,175)
Net loss before income tax provision	(80,296)	(51,145)	29,151

Income tax provision		36,476				36,476
Net loss		(116,772)		(51,145)		65,627
Add: accretion of preferred stock dividends				(5,891)		(5,891)
Net loss attributable to common stockholders	$	(116,772)	$	(57,036)	$	59,736

Collaboration Revenue

Collaboration revenue for the year ended December 31, 2014 were approximately $41.3 million. Using the relative selling price method, we allocated $38.4 million to the license delivered to Novartis under the Novartis Agreement, $2.0 million to research and development activities we performed under the Novartis Agreement and $0.9 million to the transfer of API to Novartis during the year ended December 31, 2014.

We did not recognize any revenue during the year ended December 31, 2013.

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Research and Development Expenses

Our research and development expenses were $88.4 million for the year ended December 31, 2014, an increase of $55.2 million compared to $33.2 million for the year ended December 31, 2013. The increase was due to a milestone payment of $19.8 million that we made in June 2014 in connection with our entry into the Novartis Agreement and costs associated with our Fovista Phase 3 clinical program, including clinical trial costs and the costs to manufacture Fovista for the trials as we continue to progress the Fovista Phase 3 clinical program. Other contributing factors include increased personnel costs associated with additional management and research and development staffing, including share-based compensation expense. We initiated our pivotal Phase 3 clinical program for Fovista in August 2013.

General and Administrative Expenses

Our general and administrative expenses for the year ended December 31, 2014 were $33.4 million, an increase of $19.2 million compared to $14.2 million for the year ended December 31, 2013. The increase was primarily due to an increase in costs to support the expansion of the Company s operations, including our public company infrastructure, and relates to the hiring of additional management and corporate staffing, professional services and consulting fees, increased share-based compensation, and costs related to an executive retirement and other one-time post-employment costs.

Interest Income (Expense), Net

Net interest income for the year ended December 31, 2014 was $0.2 million compared to net interest expense of $1.5 million for the year ended December 31, 2013. Net interest income earned during the year ended December 31, 2014 was a result of a significant increase in our cash, cash equivalents and marketable securities average balances during the year ended December 31, 2014 as compared to the year ended December 31, 2013. The amounts recorded in the year ended December 31, 2013 were related to interest expense associated with our venture debt facility that we entered into in June 2012. The debt facility was paid off in May 2013 and as such, there was no corresponding interest expense during the year ended December 31, 2014.

Other Loss

There was no other loss recorded for the year ended December 31, 2014 compared to other loss of $1.2 million for the year ended December 31, 2013. Amounts recorded as other loss were due to the change in fair value of the preferred stock warrant liability recorded in the first half of 2013. Upon completion of our initial public offering on September 30, 2013, the preferred stock warrants were converted to common stock warrants and are now treated as permanent equity.

Provision for Income Taxes

The provision for income taxes recorded for the year ended December 31, 2014 was $36.5 million. This primarily relates to the payments we received from Novartis and Novo A/S in 2014, a significant portion of which contributed to an increase in taxable income for 2014. For the year ended December 31, 2013, we did not record a provision for income taxes due to our significant operating losses.

Comparison of Years Ended December 31, 2013 and 2012

	Years ended December 31, 2013	Years ended December 31, 2012	Increase (Decrease)
	(in thousands)		
Statement of Operations Data:			
Collaboration revenue	$	$	$
Operating Expenses:			
Research and development	33,215	6,792	26,423
General and administrative	14,210	6,889	7,321
Total operating expenses	47,425	13,681	33,744
Loss from operations	(47,425)	(13,681)	33,744
Interest expense, net	(1,454)	(507)	947
Loss on extinguishment of debt	(1,091)		1,091
Other loss	(1,175)	(374)	801
Net loss before income tax provision	(51,145)	(14,562)	36,583
Income tax provision			
Net loss	(51,145)	(14,562)	36,583
Add: accretion of preferred stock dividends	(5,891)	(7,063)	(1,172)
Net loss attributable to common stockholders	$ (57,036)	$ (21,625)	$ 35,411

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Collaboration Revenue

We did not recognize any revenue for the year ended December 31, 2013 or for the year ended December 31, 2012.

Research and Development Expenses

Our research and development expenses were $33.2 million for the year ended December 31, 2013, an increase of $26.4 million compared to $6.8 million for the year ended December 31, 2012. The increase was primarily due to milestone payments, manufacturing activity and clinical trial startup costs as we commenced our Phase 3 clinical program for Fovista in August 2013.

General and Administrative Expenses

Our general and administrative expenses for the year ended December 31, 2013 were $14.2 million, an increase of $7.3 million compared to $6.9 million for the year ended December 31, 2012. The increase was primarily due to an increase in intellectual property related expenses, professional services and consulting fees and personnel costs, including additional management and corporate staffing to support our public company infrastructure.

Interest Expense, net

Interest expense, net for the year ended December 31, 2013 was $1.5 million compared to $0.5 million for the year ended December 31, 2012. The amounts in both 2013 and 2012 were related to interest associated with our venture debt facility that we entered into in June 2012 and paid off in May 2013. The related interest expense for the year ended December 31, 2013 included a payment of $0.8 million that was required upon the earlier of the maturity date or the date of repayment of the venture debt facility.

Loss on Extinguishment of Debt

In May 2013, we repaid the outstanding balance on our venture debt facility. The associated $1.1 million loss on extinguishment of debt represents the related prepayment penalties and an expense for deferred costs and unamortized debt discount related to the venture debt facility.

Other Loss

Other loss was $1.2 million for the year ended December 31, 2013 compared to $0.4 million for the year ended December 31, 2012. The $0.8 million increase was primarily due to the change in fair value of the preferred stock warrant liability. These warrants were converted into warrants to purchase common stock upon the closing of our initial public offering.

Liquidity and Capital Resources

Sources of Liquidity

Since inception, we have financed our operations primarily through private placements of our preferred stock, venture debt borrowings, funding we received under the Novo Agreement, our initial public offering, which we closed on September 30, 2013, our follow-on public offering of common stock, which we completed in February 2014, and funds we received under the Novartis Agreement. In September 2013, we issued and sold an aggregate of 8,740,000 shares of common stock in our initial public offering at a public offering price of $22.00 per share. We received net proceeds from the initial public offering of $175.6 million. In February 2014, we issued and sold 1,900,000 shares of common stock and selling shareholders sold 728,571 shares of common stock in a follow-on public offering at a public offering price of $31.50 per share. We received

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net proceeds of $55.4 million from the follow-on offering. The Novo Agreement, which is described in more detail below, provides for financing of up to $125.0 million in the aggregate in return for the sale to Novo A/S of royalty interests in worldwide sales of Fovista. We received an aggregate of $125.0 million of this financing in separate tranches in May 2013, January 2014 and November 2014, which constitutes the full amount of funding under the Novo Agreement. In May 2013, we issued and sold an aggregate of 6,666,667 shares of our series C preferred stock at a price per share of $2.50, for an aggregate purchase price of $16.7 million. In August 2013, we issued and sold an aggregate of 13,333,333 additional shares of our series C preferred stock to the same purchasers at a price per share of $2.50, for an aggregate purchase price of $33.3 million.

In May 2014, we received an upfront payment of $200.0 million upon execution of the Novartis Agreement for the rights to commercialize Fovista, a product candidate currently in Phase 3 clinical trials, outside the United States. Novartis is also obligated to pay us up to an aggregate of $130.0 million if we achieve specified patient enrollment milestones for our ongoing pivotal Phase 3 clinical program for Fovista, $50.0 million of which we received in October 2014. In connection with the receipt of the upfront payment from Novartis, we made a milestone payment in June 2014 of approximately $19.8 million under one of our agreements. We made income tax payments of $40.2 million during the year ended December 31, 2014. These payments relate to estimated taxable income that resulted from the receipt of $250.0 million in upfront and milestone payments under the Novartis Agreement, and $83.3 million in proceeds from the Novo Agreement in 2014

Cash Flows

As of December 31, 2014, we had cash, cash equivalents, and available for sale securities totaling $463.6 million and no debt. We primarily invest our cash and cash equivalents in U.S. Treasury securities and money market funds that invest in U.S. Treasury securities. The following table shows a summary of our cash flows for the years ended December 31, 2014, 2013 and 2012:

	Years ended December 31,		
	2014 (Restated)	**2013**	**2012**
		(in thousands)	
Net cash provided by (used in):			
Operating Activities	$ 111,088	$ (48,775)	$ (13,104)
Investing Activities	(427,817)	(5)	
Financing Activities	145,947	255,072	11,012
Net change in cash and cash equivalents	$ (170,782)	$ 206,292	$ (2,092)

Cash Flows from Operating Activities

Net cash provided by operating activities of $111.1 million for the year ended December 31, 2014 relates primarily to the receipt of the upfront payment of $200.0 million and the first enrollment milestone payment of $50.0 million under the Novartis Agreement, offset by (i) tax payments made in 2014 totaling $40.2 million relating to our estimated taxable income in 2014, (ii) a milestone payment in June 2014 of approximately $19.8 million that we paid under one of our agreements in connection with our entry into the Novartis Agreement and (iii) costs we have incurred in our efforts to advance Fovista into Phase 3 clinical trials, including increased spending on Phase 3 clinical trial costs and manufacturing activity for Fovista, as well as increased general and administrative expenses. Net cash used in operating activities in prior periods resulted primarily from our net losses adjusted for non-cash charges and changes in the components of working capital.

In August 2013, we initiated our pivotal Phase 3 clinical program for Fovista which consists of three separate clinical trials. We expect cash used in operating activities to continue to increase substantially compared to prior periods and for the foreseeable future, particularly as our patient enrollment increases in our Phase 3 clinical program, as we manufacture validation production batches of API and drug product for Fovista, and as we continue the development of and seek marketing approval for Fovista, Zimura and, possibly, other product candidates.

Cash Flows from Investing Activities

Net cash used in investing activities for the year ended December 31, 2014 was $427.8 million and relates primarily to the purchase of marketable securities totaling $597.8 million offset by marketable security maturities of $171.6 million. Also contributing to our cash used in investing activities for this period were capital expenditures associated with new manufacturing equipment and our new office facilities in New York, New York and Princeton, New Jersey. Net cash used in investing activities was de minimis for the year ended December 31, 2013.

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Cash Flows from Financing Activities

Net cash provided by financing activities was $145.9 million for the year ended December 31, 2014 and $255.1 million for the year ended December 31, 2013. Net cash provided by financing activities for the year ended December 31, 2014 consisted primarily of $83.3 million in proceeds under the Novo Agreement received in two tranches in January 2014 and November 2014, and proceeds of $55.4 million from our follow-on public offering in February 2014. Net cash provided by financing activities for the year ended December 31, 2013 related primarily to proceeds of $175.6 million from our initial public offering in September 2013, proceeds of $49.7 million from the issuance of preferred stock and proceeds of $41.7 million from the Novo Agreement in May 2013, offset by the repayment of our former venture debt facility.

Funding Requirements

Our product candidates, Fovista and Zimura, are in clinical development. We expect our expenses to continue to increase, particularly as we continue the development of Fovista in our Phase 3 clinical program and other additional clinical trials for the treatment of wet AMD. We initiated our pivotal Phase 3 clinical program for Fovista in August 2013. We plan to enroll a total of 1,866 patients for this program. In addition, we also expect our expenses to increase as we further evaluate the potential benefit of Fovista in wet AMD, when administered in combination with anti-VEGF drugs, and in other ophthalmic diseases and conditions with unmet medical need and pursue the development of Zimura for the treatment of geographic atrophy, a form of dry AMD and in combination with anti-VEGF therapy and, potentially, Fovista, for the treatment of anti-VEGF resistant wet AMD patients who are believed to have complement mediated inflammation. We expect our expenses to increase as patient enrollment increases in these clinical trials. In addition, our expenses will increase prior to obtaining marketing approval for Fovista as we manufacture validation production batches of API and drug product for Fovista, expand our infrastructure to support commercial operations and if we obtain marketing approval for Fovista, we expect our commercialization expenses in the United States related to product sales, marketing, distribution and manufacturing to increase significantly. Outside the United States, our partner Novartis is responsible for these commercialization expenses. If we obtain marketing approval for Zimura or any other product candidate that we develop, we also expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. Furthermore, we are incurring and expect to continue to incur additional costs associated with being a public company, including legal, compliance, accounting and investor and public relations expenses as well as increased insurance premiums. We are party to agreements, specifically an asset acquisition agreement with OSI (Eyetech), Inc., which agreement is now held by OSI Pharmaceuticals, LLC, a subsidiary of Astellas US, LLC, and license agreements with Archemix Corp., or Archemix, and Nektar Therapeutics, or Nektar, that impose significant milestone payment obligations on us in connection with our achievement of specific clinical, regulatory and commercial milestones with respect to Fovista. For example, in connection with our entry into the Novartis Agreement, we made a milestone payment of $19.8 million to Nektar Therapeutics in June 2014.

Our expenses also will increase if and as we:

- undertake additional clinical development of Fovista, if it is approved, in support of our efforts to broaden the label for Fovista;

- conduct additional clinical trials of Zimura that may be required by regulatory authorities, including a second Phase 3 clinical trial, for us to seek marketing approval for Zimura for the treatment of geographic atrophy and/or wet AMD;

- in-license or acquire the rights to other complementary products, product candidates or technologies, including drug delivery technology, for the treatment of ophthalmic diseases;

- seek marketing approval for any product candidates that successfully complete clinical trials;

- expand our outsourced manufacturing activities and establish sales, marketing, distribution capabilities, if we receive, or expect to receive, marketing approval for any product candidates;

- maintain, expand and protect our intellectual property portfolio;

- hire additional clinical, manufacturing, quality control and scientific personnel;

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- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts; and

- continue to develop tivozanib for the treatment of ophthalmic diseases.

As of December 31, 2014, we had cash, cash equivalents, and marketable securities of $463.6 million and $351.2 million in total liabilities, including liabilities of $334.6 million relating to the Novo Agreement and deferred revenue associated with the Novartis Agreement.

We believe that our cash, cash equivalents and marketable securities, together with the potential remaining enrollment-based milestone payments under Novartis Agreement, will be sufficient to fund our operations and capital expenditure requirements as currently planned, including the expansion of our infrastructure to support commercial operations, through the end of 2017. Our capital requirements will also depend on other factors, including the success of our development and commercialization of our product candidates and whether we pursue the acquisition or in-licensing and subsequent development of additional product candidates. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Our costs will increase if we experience delays in enrollment, the availability of drug supply for our clinical trials or for other reasons. Our costs will also increase if we increase our investigator fees for our clinical trials or expand the scope of our clinical trials and programs, including, for example by changing the geographic mix of sites at which patients are enrolled, or if we decide to increase other corporate or licensing activities or staffing, or if we experience issues with the process development and scale-up of manufacturing activities.

Our current Phase 3 clinical program for Fovista is expected to continue through at least 2017, and substantial expenditures to complete the Phase 3 clinical program will be required after the receipt of initial, top-line data. Moreover, we are at the early stages of formulating our clinical development plan for Zimura, which we expect will continue for at least the next several years. At this time, we cannot reasonably estimate the remaining costs necessary to complete the clinical development of either Fovista or Zimura, complete process development and manufacturing scale-up activities associated with Fovista and Zimura and potentially seek marketing approval for Fovista and Zimura, or the nature, timing or costs of the efforts necessary to complete the development of Zimura and any other product candidate we may develop.

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

- the scope progress, costs and results of our Phase 3 clinical program for Fovista;

- the progress, costs and results of our planned clinical trials to further evaluate the potential benefit of Fovista in wet AMD when administered in combination with anti-VEGF drugs, and in other ophthalmic diseases and conditions with unmet need;

- the scope, progress, results and costs of (i) our planned Phase 2/3 clinical trial evaluating Zimura for the treatment of geographic atrophy and additional clinical trials (including a second Phase 3 trial) required by regulatory authorities for us to seek marketing approval in this indication, (ii) our very small Phase 2 clinical trial evaluating Zimura in combination with anti-VEGF therapy for the treatment of polypoidal choroidal vasculopathy, a specific type of wet AMD in patients who do not respond adequately to treatment with anti-VEGF monotherapy or for whom anti-VEGF monotherapy fails, and (iii) our planned Phase 2 clinical trial evaluating Zimura in combination with anti-VEGF therapy and Fovista, for the treatment of anti-VEGF resistant wet AMD patients who are believed to have complement mediated inflammation;

- the costs and timing of process development and manufacturing scale-up activities associated with Fovista and Zimura;

- the costs, timing and outcome of regulatory review of Fovista and Zimura;

- the timing, scope and cost of commercialization activities for Fovista or Zimura if we receive, or expect to receive, marketing approval for either product candidate, including the costs and timing of expanding our internal commercial operations, expanding our outsourced manufacturing activities and establishing product sales, marketing and distribution capabilities;

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- subject to receipt of marketing approval, net revenue received from commercial sales of Fovista or Zimura, after milestone payments and royalties;

- the scope, progress, results and costs of clinical trials for any other product candidates that we may develop;

- our ability to establish collaborations on favorable terms, if at all;

- the scope, progress and results of our preclinical and clinical plans for tivozanib;

- the extent to which we in-license or acquire rights to complimentary products, product candidates or technologies; and

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

Until such time, if ever, as we can generate substantial product revenues, we may need to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. Our remaining potential enrollment milestone payments pursuant to the Novartis Agreement are subject to enrollment of a specified number of patients in our Phase 3 clinical trials of Fovista. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect their rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Our pledge of assets, including intellectual property rights, as collateral to secure our obligations under the Novo Agreement may limit our ability to obtain debt financing. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Licensing and Commercialization Agreement with Novartis Pharma AG

On May 19, 2014, we entered into a licensing and commercialization agreement with Novartis Pharma AG, which we refer to as the Novartis Agreement. Under the agreement with Novartis, we granted Novartis exclusive rights under specified patent rights, know-how and trademarks controlled by us to manufacture, from bulk API supplied by us, standalone Fovista products and products combining Fovista with an anti-VEGF product to which Novartis has rights in a co-formulated product, for the treatment, prevention, cure or control of any human disease, disorder or condition of the eye, and to develop and commercialize those licensed products in all countries outside of the United States, which we refer to as the Novartis Territory. We have agreed to use commercially reasonable efforts to complete our ongoing pivotal Phase 3 clinical program for Fovista and Novartis has agreed to use commercially reasonable efforts to develop a standalone Fovista product and a co-formulated product containing Fovista and an anti-VEGF product to which Novartis has rights, as well as a pre-filled syringe presentation of such products and to use commercially reasonable efforts, subject to obtaining marketing approval, to commercialize licensed products in the Novartis Territory in accordance with agreed development and marketing plans. Novartis has also granted us options, subject to specified limitations, and to the extent such rights are controlled by Novartis, to obtain exclusive rights from Novartis to develop and commercialize in the United States the co-formulated and pre-filled syringe products developed by Novartis. We and Novartis have each granted the other options, subject to specified limitations, to obtain access to study data from certain clinical trials of licensed products that we or Novartis may conduct, including for use by the other in regulatory filings in its territory. We have agreed to exclusively supply Novartis, and Novartis has agreed to exclusively purchase from us, its clinical and commercial requirements for the bulk API in Fovista for use in licensed products in the Novartis Territory. We have agreed not to commercialize any product comprising Fovista or any other anti-PDGF product in the ophthalmic field in the Novartis Territory.

Novartis paid us $200.0 million upon execution of the Novartis Agreement. Novartis is also obligated to pay us up to an aggregate of $130.0 million if we achieve specified patient enrollment milestones for its ongoing pivotal Phase 3 clinical program for Fovista, $50.0 million of which was received in October 2014, and up to an aggregate of an additional $300.0 million upon achievement of specified approval milestones, including reimbursement approval in certain countries in the Novartis Territory. In addition, Novartis has agreed to pay us up to an aggregate of an additional $400.0 million if Novartis achieves specified sales milestones in the Novartis Territory. Novartis also is obligated to pay us royalties with respect to standalone Fovista products and combination Fovista products that Novartis successfully commercializes. We will receive royalties at a mid-thirties percentage of net sales of standalone Fovista products and a royalty of approximately equal value for sales of combination Fovista products. Such royalties are subject to customary deductions, credits, and reductions for lack of patent coverage or market exclusivity. Novartis s obligation to pay such royalties will continue on a licensed product-by-licensed product and country-by-country basis until Novartis s last actual commercial sale of such licensed product in such country. We will continue to be responsible for royalties we owe to third parties on sales of Fovista products.

Novartis has agreed to pay our manufacturing costs plus a specified percentage margin for supplies of the bulk API in Fovista that we supply to Novartis. If we or Novartis exercise our respective rights to obtain access to study data from clinical trials conducted by the other party, the party exercising the option will be obligated to pay the other party s associated past development costs and share with such other party any future associated development costs. If we exercise our option to obtain Novartis-controlled rights to develop, manufacture and commercialize any co-formulated Fovista product in the United States, we will be obligated to pay a specified percentage of Novartis s associated past development costs and share with Novartis any future associated development costs. We and Novartis will also need to negotiate and agree on financial and other terms that would apply to such rights. If we exercise our option to obtain Novartis-controlled rights to develop and commercialize a pre-filled syringe product in the United States, we will be obligated to either enter into a supply agreement with Novartis under which we will pay Novartis its manufacturing cost plus a specified percentage margin for supplies of Fovista products in pre-filled syringes that Novartis supplies to us, or obtain supplies of products in pre-filled syringes from a third party manufacturer and pay Novartis a low single-digit percentage of our net sales of such products.

We have retained control over the design and execution of our pivotal Phase 3 clinical program for Fovista and remain responsible for funding the costs of that program, subject to Novartis s responsibility to provide Lucentis, an anti-VEGF agent to which Novartis has rights in the Novartis Territory, for use in our ongoing Phase 3 clinical trials and ongoing Phase 2 trials and future Phase 2 and Phase 3 trials in the Novartis Territory following the effective date of the Novartis agreement. Novartis will have control over, and will be responsible for the costs of, all other clinical trials that may be required to obtain marketing approvals in the Novartis Territory for licensed products under the agreement. Novartis is also responsible for costs associated with co-formulation development, pre-filled syringe development and other development costs in the Novartis Territory, but excluding regulatory filing fees in the European Union for the standalone Fovista product, for which we will be responsible.

The Novartis Agreement, unless earlier terminated by us or Novartis, will expire upon the expiration of Novartis s obligation to pay us royalties on net sales of licensed products. We and Novartis each may terminate the agreement if the other party materially breaches the agreement and does not cure such breach within a specified cure period, if the other party experiences any specified insolvency event, if the other party challenges or assists a third party in challenging the validity or enforceability of certain patent rights controlled by the terminating party, or if the parties are prevented in any manner that materially adversely affects the progression of the development or commercialization of licensed products for a specified period as a result of specified governmental actions. Novartis may terminate the agreement at any time without cause, or within a specified period after a change in control of us, as defined in the agreement, or for specified safety reasons, effective at the end of a specified period following Novartis s written notice to us of Novartis s election to terminate the agreement. We may also terminate the agreement if Novartis determines to seek marketing approval of an alternative anti-PDGF product in the Novartis Territory as more fully described below. If we elect to terminate the agreement because specified governmental actions prevent the parties from materially progressing the development or commercialization of licensed products as described above, we will be required to pay a substantial termination fee, with the specific amount of such fee determined based on the effective date of the termination. Following any termination, all rights to Fovista that we granted to Novartis, including, without limitation, the right to commercialize standalone Fovista products in the Novartis Territory, will revert to us, Novartis will perform specified activities in connection with transitioning to us the rights and responsibilities for the continued development, manufacture and commercialization of the standalone Fovista product for countries in the Novartis Territory, and the parties will cooperate on an orderly wind down of development and commercialization activities for other licensed products in the Novartis Territory.

Novartis has agreed to specified limitations on its ability to in-license, acquire or commercialize any anti-PDGF product that does not contain Fovista, which we refer to as an Alternative Anti-PDGF Product in the Novartis Territory and, to the extent Novartis develops, in-licenses or acquires such a product, to make such product available to us in the United States under specified option conditions. If we exercise our option, we will be obligated to make certain payments to Novartis, including specified milestone and royalty payments. The amounts of such payments will vary based on the product s stage of clinical development at the time we exercise our option, whether the product is a standalone or combination product and whether Novartis exercises an option to co-promote such product in the United States. If Novartis determines to seek marketing approval of an Alternative Anti-PDGF Product in the Novartis Territory, we will, subject to specified limitations, have the option to terminate the agreement, convert Novartis s exclusive licenses into non-exclusive licenses, or elect to receive a royalty on sales of such product by Novartis. If we elect to terminate the agreement, Novartis will, subject to specified limitations, be required to pay to us, certain payments based on achievement, with respect to such product, of the milestones that would have otherwise applied to licensed products under the agreement.

The agreement contains standstill provisions pursuant to which Novartis agrees to certain restrictions relating to our voting securities until marketing approval for a standalone Fovista product is granted in either the United States or the European Union. The agreement contains indemnification and dispute resolution provisions that are customary for agreements of its kind.

Clinical Manufacturing and Supply Agreement with Agilent Technologies, Inc.

On May 2, 2014, we entered into a Clinical Manufacturing and Supply Agreement with Agilent Technologies, Inc. pursuant to which Agilent has agreed to manufacture and supply to us, and we have agreed to purchase from Agilent, a specified percentage of our clinical requirements in specified jurisdictions of the API in Fovista. The agreement has an initial five year term, which is subject to automatic renewal absent termination by either party in accordance with the terms of the agreement. The agreement provides for pricing structured on a tiered basis with the price reduced as the volume ordered increases. We may terminate the agreement or any statement of work thereunder upon 12 months prior written notice to Agilent and Agilent may terminate the agreement if we do not, over a specified period, purchase and take delivery from Agilent of a specified minimum quantity of API for Fovista. Each party also has the right to terminate the agreement for other customary reasons such as material breach and bankruptcy. The agreement contains provisions relating to compliance by Agilent with current Good Manufacturing Practices, cooperation by Agilent in connection with marketing applications for Fovista, indemnification, confidentiality, dispute resolution and other customary matters for an agreement of this kind.

Financing Agreement with Novo A/S

In May 2013, we entered into the Novo Agreement, pursuant to which we had the ability to obtain financing in three tranches in an amount of up to $125.0 million in return for the sale to Novo A/S of aggregate royalties at mid-single-digit percentages of worldwide sales of Fovista, with the royalty percentage determined by the amount of funding provided by Novo A/S. The three tranches of financing, in which Novo A/S purchased three low single-digit royalty interests and paid us $125.0 million in the aggregate, closed in May 2013, January 2014 and November 2014.

The royalty payment period begins on the commercial launch of Fovista and ends, on a country-by-country basis, on the latest to occur of the twelfth anniversary of the commercial launch of Fovista, the expiration of certain patent rights covering Fovista, and the expiration of regulatory exclusivity for Fovista, in each applicable country. Royalty payments will be payable quarterly in arrears during the royalty period. Our obligations under the Novo Agreement may also apply to certain other anti-platelet derived growth factor, or anti-PDGF, products we may develop.

We used a portion of the proceeds that we initially received under the Novo Agreement to repay in full an aggregate of $14.4 million of outstanding principal, interest and fees under our venture debt facility and are using the remaining proceeds we received from the sale of royalty interests under the Novo Agreement, primarily to support clinical development and regulatory activities for Fovista and for general corporate expenses.

The Novo Agreement requires the establishment by us and Novo A/S of a joint oversight committee in relation to the development of Fovista in the event that Novo A/S does not continue to have a representative on our board of directors. The Novo Agreement also contains customary representations and warranties, as well as certain covenants relating to the operation of our business, including covenants requiring us to use commercially reasonable efforts to continue our development of Fovista, to file, prosecute and maintain certain patent rights and, in our reasonable judgment, to pursue claims of infringement of our intellectual property rights. The Novo Agreement also places certain restrictions on our business, including restrictions on our ability to grant security interests in our intellectual property to third parties, to sell, transfer or out-license intellectual property, or to grant others rights to receive royalties on sales of Fovista and certain other products. We reimbursed Novo A/S for specified legal and other expenses and are required to provide Novo A/S with certain continuing information rights. We have agreed to indemnify Novo A/S and its representatives with respect to certain matters, including with respect to any third-party infringement or product liability claims relating to our products. Our obligations under the Novo agreement are secured by a lien on certain of our intellectual property and other rights related to Fovista and other anti-PDGF products we may develop.

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Contractual Obligations and Commitments

The following table summarizes our contractual obligations as of December 31, 2014:

	Payments Due by Period				
	Total	Less than 1 year	1 - 3 years	3 - 5 years	More than 5 years
			($ in thousands)		
Operating Leases(1)	$ 8,298	$ 1,562	$ 3,299	$ 3,199	$ 238
Purchase Obligations(2)	12,605	12,605			
Total(3)	$ 20,903	$ 14,167	$ 3,299	$ 3,199	$ 238

(1) Operating lease obligations reflect our obligation to make payments in connection with leases for our office space.

(2) Purchase obligations represent our commitments under certain of our supply agreements.

(3) This table does not include (a) any milestone payments which may become payable to third parties under license agreements as the timing and likelihood of such payments are not known with certainty, (b) any royalty payments to third parties as the amounts, timing and likelihood of such payments are not known, (c) contracts that are entered into in the ordinary course of business which are not material in the aggregate in any period presented above and (d) the royalty purchase liability of $125.0 million as of December 31, 2014, due to the fact that the royalty payment period is not known.

Under various agreements, we may be required to pay royalties and make milestone payments. These agreements include the following:

- Under our acquisition agreement with OSI (Eyetech), Inc., which agreement is now held by OSI Pharmaceuticals, LLC., or OSI Pharmaceuticals, a subsidiary of Astellas US, LLC, for rights to particular anti-PDGF aptamers, including Fovista, we are obligated to pay to OSI Pharmaceuticals future one-time payments of $12.0 million in the aggregate upon marketing approval in the United States and the European Union of a covered anti-PDGF product. We also are obligated to pay to OSI Pharmaceuticals a royalty at a low single-digit percentage of net sales of any covered anti-PDGF product we successfully commercialize.

- Under a license agreement with Archemix Corp., or Archemix, with respect to pharmaceutical products comprised of or derived from any anti-PDGF aptamer, we are obligated to make future payments to Archemix of up to an aggregate of $14.0 million if we achieve specified clinical and regulatory milestones with respect to Fovista, up to an aggregate of $3.0 million if we achieve specified commercial milestones with respect to Fovista and, for each other anti-PDGF aptamer product that we may develop under the agreement, up to an aggregate of approximately $18.8 million if we achieve specified clinical and regulatory milestones and up to an aggregate of $3.0 million if we achieve specified commercial milestones. No royalties are payable to Archemix under this license agreement.

- Under a license agreement with Archemix with respect to pharmaceutical products comprised of or derived from anti-C5 aptamers, for each anti-C5 aptamer product that we may develop under the agreement, including Zimura, we are obligated to make future payments to Archemix of up to an aggregate of $57.5 million if we achieve specified development, clinical and regulatory milestones and, as to all anti-C5 products under the agreement collectively, up to an aggregate of $22.5 million if we achieve specified commercial milestones. We are also obligated to pay Archemix a double-digit percentage of specified non-royalty payments we may receive from any sublicensee of our rights under this license agreement. No royalties are payable to Archemix under this license agreement.

- Under a license, manufacturing and supply agreement with Nektar Therapeutics, or Nektar, for specified pegylation reagents used to manufacture Fovista, we are obligated to make future payments to Nektar of up to an aggregate of $6.5 million if we achieve specified clinical and regulatory milestones, and an additional payment of $3.0 million if we achieve a specified commercial milestone with respect to Fovista. We are obligated to pay Nektar tiered royalties at low to mid-single-digit percentages of net sales of any licensed product we successfully commercialize, with the royalty percentage determined by our level of licensed product sales and the extent of patent coverage for the licensed product and whether we have granted a third- party commercialization rights to the licensed product. In June 2014, we paid Nektar $19.8 million in connection with our entry into the Novartis Agreement.

- Under the Novo Agreement, with respect to Fovista, we will be obligated to pay Novo A/S a mid-single-digit percentage royalty based on worldwide sales of Fovista. See Note 7 Financing Agreement with Novo A/S above for further information about Novo Agreement.

- Under the clinical supply agreement with Agilent Technologies, Inc., Agilent has agreed to manufacture and supply to us, and we have agreed to purchase from Agilent, a specified percentage of our clinical requirements in specified jurisdictions of the API in our product candidate Fovista. Our agreement with Agilent has an initial five year term, which is subject to automatic renewal absent termination by either party in accordance with the terms of the Agreement. The Agreement provides for pricing structured on a tiered basis with the price reduced as the volume ordered increases. We may terminate the agreement or any statement of work thereunder upon 12 months prior written notice to Agilent.

We also have employment agreements with certain employees which require the funding of a specific level of payments, if certain events, such as a change in control, termination without cause or retirement, occur.

In addition, in the course of normal business operations, we have agreements with contract service providers to assist in the performance of our research and development and manufacturing activities. Expenditures to CROs represent a significant cost in clinical development. We can elect to discontinue the work under these agreements at any time. We could also enter into additional collaborative research, contract research, manufacturing, and supplier agreements in the future, which may require upfront payments and even long- term commitments of cash.

Off-Balance Sheet Arrangements

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

Item 8. Financial Statements and Supplementary Data

Our financial statements, together with the report of our independent registered public accounting firm, appear on pages F-1 through F-29 of this Annual Report on Form 10-K/A.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2014. The term disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

In our Annual Report on Form 10-K for the year ended December 31, 2014, filed on March 2, 2015, our management concluded that, as of December 31, 2014, our disclosure controls and procedures were effective at a reasonable assurance level. Based on a re-evaluation of our disclosure controls and procedures as of December 31, 2014, our Chief Executive Officer and Chief Financial Officer have concluded that, as of such date, our disclosure controls and procedures were not effective at the reasonable assurance level because of the material weakness in our internal control over financial reporting discussed in detail below.

Management s Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934, as amended (the Exchange Act). Our internal control over financial reporting is a process designed by, or under the supervision of our Chief Executive Officer and our Chief Financial Officer, and effected by the Company s board of directors, management and other personnel to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles.

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

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Our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2014. In making this assessment, management used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in the original *Internal Control Integrated Framework* updated in 2013.

In our Annual Report on Form 10-K for the year ended December 31, 2014, filed on March 2, 2015, our management concluded that, as of December 31, 2014, our internal control over financial reporting was effective. In July 2015, we determined that we overstated the net deferred tax asset recorded on our balance sheets and understated the income tax provision on our statements of operations as of and for the periods ending June 30, 2014, September 30, 2014 and December 31, 2014. Management believes that this situation revealed a material weakness in internal controls related to technical accounting expertise over the accounting for deferred tax assets and income tax accounting in general.

This material weakness resulted in this amendment to our Annual Report on Form 10-K/A for the year ended December 31, 2014, in order to restate the financial statement for the year ended December 31, 2014 and to restate the financial information for the second, third and fourth quarters of 2014. As a result of this material weakness, our management has revised its earlier assessment and has now concluded that our internal control over financial reporting was not effective as of December 31, 2014.

We are in the process of remediating the material weakness in internal control over financial reporting referred to above by designing and implementing new procedures as they relate to our accounting for deferred tax assets, as well as hiring or engaging additional resources with technical accounting expertise in the area of tax accounting and reporting.

The effectiveness of the Company s internal control over financial reporting as of December 31, 2014, has been audited by Ernst & Young LLP, an independent registered public accounting firm, as stated in their report which appears herein.

Report of Independent Registered Public Accounting Firm

The Board of Directors and Shareholders

Ophthotech Corporation

We have audited Ophthotech Corporation s internal control over financial reporting as of December 31, 2014, based on criteria established in Internal Control Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) (the COSO criteria). Ophthotech Corporation s management is responsible for maintaining effective internal control over financial reporting, and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management s Annual Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the company s internal control over financial reporting based on our audit.

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

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

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

In our report dated March 2, 2015, we expressed an unqualified opinion that Ophthotech Corporation maintained, in all material respects, effective internal control over financial reporting as of December 31, 2014, based on the COSO criteria. Management has subsequently identified a deficiency in controls related to technical accounting expertise over the accounting for deferred tax assets and income tax accounting in general, and has further concluded that such deficiency represented a material weakness as of December 31, 2014. As a result, management has revised its assessment, as presented in the accompanying Management s Annual Report on Internal Control Over Financial Reporting, to conclude that Ophthotech Corporation s internal control over financial reporting was not effective as of December 31, 2014. Accordingly, our present opinion on the effectiveness of Ophthotech Corporation s internal control over financial reporting as of December 31, 2014, as expressed herein, is different from that expressed in our previous report.

A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the company s annual or interim financial statements will not be prevented or detected on a timely basis. The following material weakness has been identified and included in management s assessment. Management has identified a material weakness in controls related to the company s accounting for valuation allowances related to deferred tax assets. We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the balance sheets of Ophthotech Corporation as of December 31, 2014 and 2013, and the related statements of operations, stockholders equity, and cash flows for each of the three years in the period ended December 31, 2014. This material weakness was considered in determining the nature, timing and extent of audit tests applied in our audit of the 2014 financial statements, and this report does not affect our report dated March 2, 2015, except for Note 18, as to which the date is July 28, 2015, which expressed an unqualified opinion on those financial statements.

In our opinion, because of the effect of the material weakness described above on the achievement of the objectives of the control criteria, Ophthotech Corporation has not maintained effective internal control over financial reporting as of December 31, 2014, based on the COSO criteria.

/s/ Ernst & Young LLP

MetroPark, New Jersey

March 2, 2015, except for the effect of

the material weakness described in the sixth

paragraph above, as to which the date is

July 28, 2015

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Changes in Internal Control Over Financial Reporting

No change in our internal control over financial reporting occurred during the three months ended December 31, 2014 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. As described above, in July 2015, our management identified a material weakness in our internal control over financial reporting that resulted in a change in management s assessment of the effectiveness of internal control over financial reporting as of December 31, 2014.

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PART IV

Item 15. Exhibits and Financial Statement Schedules

The following financial statements are filed as part of this Annual Report on Form 10-K/A:

No financial statement schedules have been filed as part of this Annual Report on Form 10-K/A because they are not applicable, not required or because the information is otherwise included in our financial statements or notes thereto.

The exhibits filed as part of this Annual Report on Form 10-K/A are set forth on the Exhibit Index immediately following our financial statements. The Exhibit Index is incorporated herein by reference.

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SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Date: July 28, 2015

OPHTHOTECH CORPORATION

By: /s/ DAVID R. GUYER
David R. Guyer, M.D.

Chief Executive Officer

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

Signature	Title	Date
/s/ DAVID R. GUYER David R. Guyer, M.D.	Chief Executive Officer and Chairman of the Board of Directors (principal executive officer)	July 28, 2015
/s/ MICHAEL G. ATIEH Michael G. Atieh	Chief Financial and Business Officer (principal financial and accounting officer)	July 28, 2015

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OPHTHOTECH CORPORATION

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Report of Independent Registered Public Accounting Firm

The Board of Directors and Shareholders

Ophthotech Corporation

We have audited the accompanying balance sheets of Ophthotech Corporation (the Company) as of December 31, 2014 and 2013, and the related statements of operations, comprehensive loss, stockholders equity (deficit) and cash flows for each of the three years in the period ended December 31, 2014. These financial statements are the responsibility of the Company s management. Our responsibility is to express an opinion on these financial statements based on our audits.

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

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Ophthotech Corporation at December 31, 2014 and 2013, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2014, in conformity with US generally accepted accounting principles.

As discussed in Note 18 to the financial statements, the accompanying balance sheet as of December 31, 2014, and the related statements of operations, comprehensive loss, stockholders equity (deficit), and cash flows for the year ended December 31, 2014 have been restated to correct errors in the accounting for valuation allowances related to deferred tax assets.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), Ophthotech Corporation s internal control over financial reporting as of December 31, 2014, based on criteria established in Internal Control Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework), and our report dated March 2, 2015, except for the effect of the material weakness described in the sixth paragraph of that report, as to which the date is July 28, 2015, expressed an adverse opinion thereon.

/s/ Ernst & Young LLP

MetroPark, New Jersey

March 2, 2015, except for Note 18,

as to which the date is

July 28, 2015

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Ophthotech Corporation

Balance Sheets

(in thousands, except share and per share data)

	December 31, 2014 (Restated)	December 31, 2013
Assets		
Current assets		
Cash and cash equivalents	$ 39,814	$ 210,596
Due from Novartis Pharma, AG	960	
Available for sale securities	423,746	
Prepaid expenses and other current assets	8,812	6,804
Deferred tax assets	50	
Total current assets	473,382	217,400
Property and equipment, net	1,555	27
Deferred tax assets, non-current	4,467	
Security deposits	282	255
Other assets	100	
Total assets	$ 479,786	$ 217,682
Liabilities and Stockholders Equity		
Current liabilities		
Accounts payable and accrued expenses	$ 8,707	$ 3,810
Accrued research and development expenses	7,918	2,485
Deferred revenue	3,206	
Total current liabilities	19,831	6,295
Deferred revenue, long-term	206,418	
Royalty purchase liability	125,000	41,667
Total liabilities	351,249	47,962
Stockholders equity		
Preferred stock - $0.001 par value, 5,000,000 shares authorized, no shares issued or outstanding	$	$
Common stock - $0.001 par value, 200,000,000 shares authorized, 33,994,520 and 31,413,208 shares issued and outstanding at December 31, 2014 and 2013, respectively	34	31
Additional paid-in capital	428,390	352,739
Accumulated deficit	(299,822)	(183,050)
Accumulated other comprehensive loss	(65)	
Total stockholders equity	128,537	169,720
Total liabilities and stockholders equity	$ 479,786	$ 217,682

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

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Ophthotech Corporation

Statements of Operations

(in thousands, except per share data)

	Years ended December 31,		
	2014 (Restated)	2013	2012
Collaboration revenue	$ 41,259	$	$
Costs and expenses:			
Research and development	88,385	33,215	6,792
General and administrative	33,387	14,210	6,889
Total costs and expenses	121,772	47,425	13,681
Loss from operations	(80,513)	(47,425)	(13,681)
Interest income (expense)	217	(1,454)	(507)
Loss on extinguishment of debt		(1,091)	
Other loss		(1,175)	(374)
Loss before income tax provision	(80,296)	(51,145)	(14,562)
Income tax provision	36,476		
Net loss	(116,772)	(51,145)	(14,562)
Add: accretion of preferred stock dividends		(5,891)	(7,063)
Net loss attributable to common stockholders	$ (116,772)	$ (57,036)	$ (21,625)
Net loss attributable to common stockholders per share :			
Basic and diluted	$ (3.51)	$ (6.34)	$ (14.89)
Weighted average common shares outstanding:			
Basic and diluted	33,258	9,003	1,452

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

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Ophthotech Corporation

Statements of Comprehensive Loss

(in thousands)

	Year Ended December 31, 2014 (Restated)	2013	2012
Net loss	$ (116,772)	$ (51,145)	$ (14,562)
Other comprehensive loss:			
Unrealized loss on available for sale securities, net of taxes	(65)		
Other comprehensive loss	(65)		
Comprehensive loss	$ (116,837)	$ (51,145)	$ (14,562)

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

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Ophthotech Corporation

Statements of Stockholders Equity (Deficit)

(in thousands)

	Junior Series A Preferred Stock Shares	Junior Series A Preferred Stock Amount	Common Stock Shares	Common Stock Amount	Additional paid-in capital	Accumulated Deficit	Accumulated Other Comprehensive Loss	Total
Balance at December 31, 2011	3,000	$ 3,000	1,451	$ 1	$	$ (105,488)	$	$ (102,487)
Issuance of common stock			19		2			2
Share-based compensation					640			640
Preferred Stock dividends					(642)	(6,421)		(7,063)
Net loss						(14,562)		(14,562)
Balance at December 31, 2012	3,000	$ 3,000	1,470	$ 1	$	$ (126,471)	$	$ (123,470)
Issuance of common stock upon conversion of Series A, A-1, B, B-1 and C preferred stock			21,038	21	174,310			174,331
Issuance of common stock from initial public offering, net			8,740	9	175,546			175,555
Issuance of common stock upon conversion of Junior Series A Preferred Stock	(3,000)	(3,000)						(3,000)
Reclassification of warrant liability					2,179			2,179
Reclassification of preferred stock issuance costs					(1,804)			(1,804)
Exercise of stock options and warrants			165		94			94
Share-based compensation					2,871			2,871
Preferred Stock dividends					(457)	(5,434)		(5,891)
Net loss						(51,145)		(51,145)
Balance at December 31, 2013		$	31,413	$ 31	$ 352,739	$ (183,050)	$	$ 169,720
Exercise of stock options and warrants			682	1	2,948			2,949
Issuance from follow-on public offering, net			1,900	2	55,407			55,409
Share-based compensation					13,040			13,040
Excess tax benefit from share-based compensation					4,256			4,256
Net loss (Restated)						(116,772)		(116,772)
Unrealized loss on available for sale securities, net of tax							(65)	(65)
Balance at December 31, 2014 (Restated)		$	33,995	$ 34	$ 428,390	$ (299,822)	$ (65)	$ 128,537

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

Ophthotech Corporation

Statements of Cash Flows

(in thousands)

	Year ended December 31, 2014 (Restated)	2013	2012
Operating Activities			
Net loss	$ (116,772)	$ (51,145)	$ (14,562)
Adjustments to reconcile net loss to net cash provided by (used in) operating activities			
Depreciation	127	20	31
Amortization of debt issuance costs		88	47
Accretion of debt discount		87	59
Amortization of premium and discounts on investment securities	2,024		
Non-cash change in fair value of warrant liability		1,181	366
Loss on extinguishment of debt		1,091	
Deferred income taxes	(214)		
Share-based compensation	13,040	2,871	640
Excess tax benefits from share-based compensation	(4,256)		
Changes in operating assets and liabilities:			
Due from Novartis Pharma, AG	(960)		
Prepaid expense and other current assets	(2,008)	(6,761)	21
Accrued interest receivable	280		
Security deposits	(27)	(96)	
Other assets	(100)		1,036
Accrued research and development expenses	5,433	1,472	(485)
Accounts payable and accrued expenses	4,897	2,417	(257)
Deferred revenue	209,624		
Net cash provided by (used in) operating activities	111,088	(48,775)	(13,104)
Investing Activities			
Purchase of marketable securities	(597,762)		
Maturities of marketable securities	171,600		
Purchase of property and equipment	(1,655)	(5)	
Net cash used in investing activities	(427,817)	(5)	
Financing Activities			
Payment of debt issuance costs		(43)	(377)
Proceeds from stock option/warrant exercises	2,949	94	2
Proceeds from follow-on public offering, net	55,409		
Proceeds from initial public offering, net		175,555	
Excess tax benefits from share-based compensation	4,256		
Repayment of venture debt facility, net		(11,900)	11,387
Proceeds from issuance of preferred stock, net		49,699	
Proceeds from royalty purchase agreement	83,333	41,667	
Net cash provided by financing activities	145,947	255,072	11,012
Net change in cash and cash equivalents	(170,782)	206,292	(2,092)

Cash and cash equivalents						
Beginning of period		210,596		4,304		6,396
End of period	$	39,814	$	210,596	$	4,304
Supplemental disclosure of cash paid						
Interest	$		$	1,523	$	437
Income Taxes	$	40,159	$		$	
Supplemental disclosures of non-cash information related to investing activities						
Change in unrealized loss in marketable securities, net of tax	$	(65)	$		$	
Supplemental disclosures of cash flow information						
Conversion of preferred stock to common stock upon completion of IPO	$		$	174,310	$	
Accreted dividends on Series A, Series A-1, Series of B, B-1 and Series C Preferred Stock	$		$	5,891	$	7,063

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

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OPHTHOTECH CORPORATION

Notes to Financial Statements

(tabular dollars and shares in thousands, except per share data)

1. Business

Description of Business and Organization

Ophthotech Corporation (the Company or Ophthotech) was incorporated on January 5, 2007, in Delaware. The Company is a biopharmaceutical company specializing in the development of novel therapeutics to treat diseases of the back of the eye, with a focus on developing therapeutics for age-related macular degeneration, or AMD. The Company s most advanced product candidate is Fovista, which is in Phase 3 clinical development for use in combination with anti-VEGF drugs that represent the current standard of care for the treatment of wet AMD. The Company has completed one Phase 1 and one Phase 2b clinical trial of Fovista administered in combination with the anti-VEGF drug Lucentis. The Company is also developing its product candidate Zimura, for the treatment of patients with geographic atrophy, a form of dry AMD, in combination with anti-VEGF therapy for the treatment of polypoidal choroidal vasculopathy, a specific type of wet AMD, in patients who do not respond adequately to treatment with anti-VEGF monotherapy or for whom anti-VEGF monotherapy fails, and, potentially, in combination with anti-VEGF therapy and Fovista for the treatment of anti-VEGF resistant wet AMD patients who are believed to have complement mediated inflammation.

2. Summary of Significant Accounting Policies

Restatement of Previously Issued Financial Statements

As discussed in Note 18 to the Company s financial statements, subsequent to the issuance of the Company s Annual Report on Form 10-K for the year ended December 31, 2014, the Company and the Audit Committee of the Board of Directors concluded that the Company should restate its balance sheets as of June 30, 2014, September 30, 2014 and December 31, 2014, and its statements of operations, comprehensive loss, cash flows and changes in stockholders equity (deficit) for the periods ended June 30, 2014, September 30, 2014 and December 31, 2014 to correct the following. In the second quarter of 2014, the Company recorded an income tax benefit by reducing a portion of its valuation allowance against its gross deferred tax assets. In determining the amount of the valuation allowance release, the Company considered anticipated 2015 tax losses which would generate a refund of a portion of federal income taxes paid in 2014. The Company has now determined that, as a matter of accounting principle, the net deferred tax asset recorded on its balance sheets was overstated and the income tax provision on its statements of operations was understated as of and for the periods ended June 30, 2014, September 30, 2014 and December 31, 2014. The Company s cash position and operating expenses are not affected. The restatement has no effect on amounts reported in periods prior to the quarter ended June 30, 2014.

Basis of Presentation

The accompanying financial statements have been prepared in accordance with accounting principles generally accepted in the United States (GAAP) and include all adjustments necessary for the fair presentation of the Company s financial position for the periods presented.

Use of Estimates

The preparation of financial statements and related disclosures in conformity with GAAP requires management to make estimates and judgments that affect the amounts reported in the financial statements and accompanying notes. The Company bases its estimates and judgments on historical experience and on various other assumptions that it believes are reasonable under the circumstances. The amounts of assets and liabilities reported in the Company s Balance Sheets and the amount of expenses reported for each of the periods presented are affected by estimates and assumptions, which are used for, but not limited to, accounting for share-based compensation, accounting for research and development costs and accounting for income taxes. Actual results could differ from those estimates.

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Cash and Cash Equivalents

The Company considers all highly liquid investments with an original maturity of 90 days or less when purchased to be cash equivalents. The carrying amounts reported in the Balance Sheets for cash and cash equivalents are valued at cost, which approximates their fair value.

Available for Sale Securities

The Company considers securities with original maturities of greater than 90 days to be available for sale securities. Securities under this classification are recorded at fair value and unrealized gains and losses are recorded within accumulated other comprehensive income. The estimated fair value of the available for sale securities is determined based on quoted market prices or rates for similar instruments. In addition, the cost of debt securities in this category is adjusted for amortization of premium and accretion of discount to maturity. The Company evaluates securities with unrealized losses to determine whether such losses, if any, are other than temporary.

Revenue Recognition

Collaboration Revenue

Prior to 2014, the Company had not generated any revenue. In May 2014, the Company received an up-front payment of $200.0 million in connection with its licensing and commercialization agreement with Novartis Pharma, AG , (the Novartis Agreement), which has not been recorded as revenue due to certain contingencies associated with the payment. In September 2014, the Company achieved a $50.0 million enrollment-based milestone under the Novartis Agreement. The Company recognized revenue of approximately $41.3 million during the year ended December 31, 2014. The Company uses the relative selling price method to allocate arrangement consideration to the Company s performance obligations under the Novartis Agreement. Below is a summary of the components of the Company s collaboration revenue for the years ended December 2014, 2013 and 2012:

		Year ended December 31,	
	2014	2013	2012
License revenue	$ 38,372	$	$
Research and development activity revenue	2,004		
API transfer revenue	883		
Total collaboration revenue	$ 41,259	$	$

In the future, the Company may generate additional revenues from a combination of product sales and license fees, milestone payments and research and development activity-related payments and royalties in connection with the Novartis Agreement. The terms of this agreement and other potential collaboration or commercialization agreements the Company may enter into generally contain multiple elements, or deliverables, which may include (i) licenses, or options to obtain licenses, to certain of the Company s technology and products, (ii) research and development activities to be performed on behalf of the collaborative partner, and (iii) in certain cases, services in connection with the manufacturing of pre-clinical and clinical material. Payments to the Company under these arrangements typically include one or more of the following: non-refundable, up-front license fees; option exercise fees; funding of research and/or development efforts; milestone payments; and royalties on future product sales.

When evaluating multiple element arrangements, the Company considers whether the deliverables under the arrangement represent separate units of accounting. This evaluation requires subjective determinations and requires management to make judgments about the individual deliverables and whether such deliverables are separable from the other aspects of the contractual relationship. In determining the units of accounting, management evaluates certain criteria, including whether the deliverables have standalone value, based on the consideration of the relevant facts and circumstances for each arrangement. The consideration received is allocated among the separate units of accounting using the relative selling price method, and the applicable revenue recognition criteria are applied to each of the separate units.

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

When management believes the license to its intellectual property and products has stand-alone value, the Company generally recognizes revenue attributed to the license upon delivery. When management believes such a license does not have stand-alone value from the other deliverables to be provided in the arrangement, the Company generally recognizes revenue attributed to the license on a straight-line basis over the Company s contractual or estimated performance period, which is typically the term of the Company s research and development obligations. If management cannot reasonably estimate when the Company s performance obligation ends, then revenue is deferred until management can reasonably estimate when the performance obligation ends. The periods over which revenue should be recognized are subject to estimates by management and may change over the course of the research and development agreement. Such a change could have a material impact on the amount of revenue the Company records in future periods.

At the inception of arrangements that include milestone payments, the Company evaluates whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether (a) the consideration is commensurate with either (1) the entity s performance to achieve the milestone, or (2) the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the entity s performance to achieve the milestone, (b) the consideration relates solely to past performance, and (c) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. The Company evaluates factors such as the scientific, regulatory, commercial and other risks that must be overcome to achieve the respective milestone, the level of effort and investment required to achieve the respective milestone and whether the milestone consideration is reasonable relative to all deliverables and payment terms in the arrangement in making this assessment.

The Company aggregates its milestones into three categories: (i) clinical and development milestones, (ii) regulatory milestones, and (iii) commercial milestones. Clinical and development milestones are typically achieved when a product candidate advances into a defined phase of clinical research or completes such phase or when a contractually specified clinical trial enrollment target is attained. Regulatory milestones are typically achieved upon acceptance of the submission for marketing approval of a product candidate or upon approval to market the product candidate by the FDA or other global regulatory authorities. For example, a milestone payment may be due to the Company upon the FDA s acceptance of an NDA. Commercial milestones are typically achieved when an approved pharmaceutical product reaches certain defined levels of net sales by the licensee, such as when a product first achieves global sales or annual sales of a specified amount.

Revenues from clinical and development and regulatory milestone payments, if the milestones are deemed substantive and the milestone payments are nonrefundable, are recognized upon successful accomplishment of the milestones. With regard to the Novartis Agreement, the Company has concluded that the clinical and development milestones and certain regulatory milestones are not substantive and that the regulatory approval milestones are substantive. Milestones payments received that are not considered substantive are included in the allocable arrangement consideration and are recognized as revenue in proportion to the relative-selling price allocation established at the inception of the arrangement. Revenues from commercial milestone payments are accounted for as royalties and are recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met.

Concentration of Credit Risk

The Company s financial instruments that are exposed to concentration of credit risk consist primarily of cash and cash equivalents and available for sale securities. The Company maintains its cash in bank accounts, which, at times exceed federally insured limits. The Company maintains its cash equivalents in U.S. Treasury securities with maturities less than three months and in money market funds that invest primarily in U.S. Treasury securities.

The Company s available for sale securities are also invested in U.S. Treasury securities. The Company has not recognized any losses from credit risks on such accounts during any of the periods presented. The Company believes it is not exposed to significant credit risk on its cash, cash equivalents and available for sale securities.

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Foreign Currency Translation

The Company considers the U.S. dollar to be its functional currency. Expenses are translated at the exchange rate on the date the expense is incurred. The effect of exchange rate fluctuations on translating foreign currency assets and liabilities into U.S. dollars is included in the Statements of Operations. Foreign exchange transaction gains and losses are included in the results of operations and are not material in the Company s financial statements.

Financial Instruments

The carrying amounts of the Company s financial instruments, which include cash and cash equivalents, available for sale securities, accounts payable and accrued expenses approximate their respective fair value due to their short maturities.

Property and Equipment

Property and equipment, which consists mainly of manufacturing and clinical equipment, furniture and fixtures, computers and other equipment, and leasehold improvements, are carried at cost less accumulated depreciation. Depreciation is computed over the estimated useful lives of the respective assets, generally three to ten years, using the straight-line method rather than when the payment is made.

Research and Development

Research and development expenses primarily consist of costs associated with the development and clinical testing of Fovista, an anti-platelet derived growth factor (PDGF) aptamer that the Company is developing for use in combination with anti-VEGF drugs for treatment of wet age-related macular degeneration, or wet AMD, and Zimura, an inhibitor of complement factor C5 that the Company is developing with a focus on treatment of patients with geographic atrophy, a form of dry AMD; in combination with anti-VEGF therapy for the treatment of polypoidal choroidal vasculopathy, a specific type of wet AMD, in patients who do not respond adequately to treatment with anti-VEGF monotherapy or for whom anti-VEGF monotherapy fails; and, potentially, in combination with anti-VEGF therapy and Fovista for the treatment of anti-VEGF resistant wet AMD patients who are believed to have complement mediated inflammation. Research and development expenses consist of:

- external research and development expenses incurred under arrangements with third parties, such as contract research organizations, (CROs) and other vendors, contract manufacturing organizations and consultants, including for the production of drug substance and drug product; and

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

Research and development costs also include costs of acquired product licenses and related technology rights where there is no alternative future use, costs of prototypes used in research and development, consultant fees and amounts paid to collaborative partners.

All research and development costs are charged to operations as incurred in accordance with ASC Topic 730, *Research and Development*. The Company accounts for non-refundable advance payments for goods and services that will be used in future research and development activities as expenses when the service has been performed or when the goods have been received, rather than when the payment is made.

The Company anticipates that it will continue to incur significant research and development expenses in connection with conducting its pivotal Phase 3 clinical program for Fovista and if such trials are successful, seeking marketing approval for Fovista. The Company also anticipates that its research and development expenses will increase as a result of its plan to initiate a Phase 2/3 clinical trial to evaluate the safety and efficacy of Zimura in patients with geographic atrophy in the second half of 2015, the very small Phase 2 trial recently commenced investigating Zimura in combination with anti-VEGF therapy for the treatment of polypoidal choroidal vasculopathy, a specific type of wet AMD, in patients who do not respond adequately to treatment with anti-VEGF monotherapy or for whom anti-VEGF monotherapy fails, and the planned Phase 2 study of Zimura in combination with anti-VEGF therapy and Fovista for the treatment of anti-VEGF resistant wet AMD patients who are believed to have complement mediated inflammation. In addition, the Company also expects its research and development expenses to increase as it further evaluates the potential benefit of Fovista in wet AMD, when administered in combination with anti-VEGF drugs, and in other ophthalmic diseases and conditions with unmet medical need. The Company expects these expenses to increase as patient enrollment increases in these trials.

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Income Taxes

The Company utilizes the liability method of accounting for deferred income taxes, as set forth in ASC 740-10, *Income Taxes Overall.* Under this method, deferred tax liabilities and assets are recognized for the expected future tax consequences of temporary differences between the carrying amounts and the tax basis of assets and liabilities. The Company s U.S. federal net operating losses have occurred since its inception in 2007 and as such, tax years subject to potential tax examination could apply from that date because the utilization of net operating losses from prior years opens the relevant year to audit.

Share-Based Compensation

The Company follows the provisions of ASC 718, *Compensation Stock Compensation,* which requires the measurement and recognition of compensation expense for all share-based payment awards made to employees and non-employee directors, including employee stock options. Share-based compensation expense is based on the grant date fair value estimated in accordance with the provisions of ASC 718 and is generally recognized as an expense over the requisite service period, net of forfeitures.

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The Company estimates the fair value of stock options granted to employees on the date of grant using the Black-Scholes option-pricing model. Due to the lack of trading history, the Company s computation of stock-price volatility is based on the volatility rates of comparable publicly held companies over a period equal to the expected term of the options granted by the Company. The Company s computation of expected term is determined using the simplified method which is the midpoint between the vesting date and the end of the contractual term. The Company believes that it does not have sufficient reliable exercise data in order to justify the use of a method other than the simplified method of estimating the expected exercise term of employee stock option grants. The Company has paid no dividends to stockholders. The risk-free interest rate is based on the zero-coupon U.S. Treasury yield at the date of grant for a term equivalent to the expected term of the option.

For stock options granted as consideration for services rendered by non-employees, the Company recognizes expense in accordance with the requirements of ASC 505-50, *Equity Based Payments to Non-Employees*. Non-employee option grants that do not vest immediately upon grant are recorded as an expense over the vesting period of the underlying stock options. At the end of each financial reporting period prior to vesting, the value of these options, as calculated using the Black-Scholes option-pricing model, will be re-measured using the fair value of the Company s common stock and the non-cash expense recognized during the period will be adjusted accordingly. Since the fair value of options granted to non-employees is subject to change in the future, the amount of the future expense will include fair value re-measurements until the stock options are fully vested.

Share-based compensation expense includes stock options and restricted stock units granted to employees and non-employees, and has been reported in the Company s Statements of Operations as follows:

	Years ended December 31,		
	2014	**2013**	**2012**
Research and development	$ 7,594	$ 2,062	$ 412
General and administrative	5,446	809	228
Total	$ 13,040	$ 2,871	$ 640

JOBS Act

Prior to January 1, 2015, as an emerging growth company under the Jumpstart Our Business Startups Act of 2012, the Company was able to take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. Prior to January 1, 2015, the Company elected to delay its adoption of such new or revised accounting standards. As a result of this election, the Company s financial statements for the periods prior to January 1, 2015 may not be comparable to the financial statements of other public companies. Commencing January 1, 2015, the Company no longer qualifies for such status and, as such, it will comply with all new or revised accounting standards applicable to other public companies. The Company does not expect this change in status to have a material impact on its 2015 financial position or results of operations.

Recent Accounting Pronouncements

In June 2014, the FASB issued Accounting Standards Update No. 2014-10, *Development Stage Entities (Topic 915): Elimination of Certain Financial Reporting Requirements, including an Amendment to Variable Interest Entities Guidance in Topic 810 Consolidation* (ASU 2014-10). The objective of the amendments in ASU 2014-10 is to improve financial reporting by reducing the cost and complexity associated with the incremental reporting requirements for development stage entities. The amendments in ASU 2014-10 will be effective prospectively for annual reporting periods beginning after December 15, 2014, and interim periods within those annual periods, however early adoption is permitted. The Company evaluated and elected early adoption of ASU 2014-10 for its quarterly filing on Form 10-Q for the three and six months ended June 30, 2014. The adoption of ASU 2014-10 did not have a material impact on the Company s financial statements.

In May 2014, the FASB issued Accounting Stands Update No. 2014-09, *Revenue from Contracts with Customers (Topic 606),* (ASU 2014-09). ASU 2014-09 outlines a new, single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. This new revenue recognition model provides a five-step analysis in determining when and how revenue is recognized. The new model will require revenue recognition to depict the transfer of promised goods or services to customers in an amount that reflects the consideration a company expects to receive in exchange for those goods or services. ASU 2014-09 is effective for public entities for annual reporting periods beginning after December 15, 2016 and interim

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periods within those periods. Early adoption is not permitted. Companies may use either a full retrospective or a modified retrospective approach to adopt ASU 2014-09. The Company is currently assessing the impact that adopting this new accounting guidance will have on its financial statements and footnote disclosures.

In February 2013, the FASB issued Accounting Standards Update No. 2013-02, *Comprehensive Income: Reporting of Amounts Reclassified Out of Accumulated Other Comprehensive Income* (ASU 2013-02). ASU 2013-02 requires an entity to present the effect of certain significant reclassifications out of accumulated other comprehensive income on the respective line items in net income. The amendments in ASU 2013-02 do not change the items that must be reported in other comprehensive income or when an item of other comprehensive income must be reclassified to net income. ASU 2013-02 is effective for public companies on a prospective basis for fiscal years beginning after December 15, 2012 and for new public companies and for non-public companies for reporting periods beginning after December 15, 2013. The Company adopted this pronouncement on January 1, 2014. The Company has not reclassified any components of comprehensive income into net income for the periods presented. ASU 2013-02 requires only additional presentation and as such, there was no impact to the Company s results of operations or financial position upon adoption.

3. Capitalization

On September 30, 2013, the Company closed its initial public offering of 8,740,000 shares of common stock at a price of $22.00 per share. The net proceeds to the Company were $175.6 million, after deducting underwriters discounts and commissions and other offering expenses. In connection with the closing of the IPO, all of the Company s shares of redeemable convertible preferred stock outstanding at the time of the offering were automatically converted into 21,038,477 shares of common stock.

On February 18, 2014, the Company closed a follow-on public offering of 2,628,571 shares of common stock at a public offering price of $31.50 per share of common stock. The Company sold 1,900,000 shares and 728,571 shares were sold by selling stockholders, 342,857 of which were sold by the selling stockholders upon the full exercise by the underwriters of their option to purchase additional shares in the follow-on public offering. The net proceeds to the Company were $55.4 million, after deducting underwriters discounts and commissions and other offering expenses. The Company did not receive any proceeds from the sale of shares by the selling stockholders in the follow-on public offering.

4. Net Loss Per Common Share

Basic and diluted net loss per common share is determined by dividing net loss applicable to common stockholders by the weighted average common shares outstanding during the period. For the periods where there is a net loss attributable to common shareholders, the outstanding shares of preferred stock, stock options, and warrants have been excluded from the calculation of diluted loss per common share because their effect would be anti-dilutive. Therefore, the weighted average shares used to calculate both basic and diluted loss per share would be the same. The following table sets forth the computation of basic and diluted net loss per share for the periods indicated:

	Year ended December 31,		
	2014 (Restated)	**2013**	**2012**
Basic and diluted net loss per common share calculation:			

Net loss	$	(116,772)	$	(51,145)	$	(14,562)
Accretion of preferred stock dividends				(5,891)		(7,063)
Net loss attributable to common stockholders	$	(116,772)	$	(57,036)	$	(21,625)
Weighted average common shares outstanding		33,258		9,003		1,452
Net loss per share of common stock - basic and diluted	$	(3.51)	$	(6.34)	$	(14.89)

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The following potentially dilutive securities have been excluded from the computations of diluted weighted average shares outstanding for the periods presented, as they would be anti-dilutive:

	Years ended December 31,		
	2014	**2013**	**2012**
Redeemable convertible preferred stock			16,663
Options outstanding	3,680	2,708	1,344
Warrants	14	88	94
Restricted stock units	37		
Total	3,731	2,796	18,101

5. Cash, Cash Equivalents and Available for Sale Securities

The Company considers all highly liquid investments purchased with original maturities at the date of purchase of 90 days or less to be cash equivalents. Cash and cash equivalents included cash of $4.7 million and $6.8 million at December 31, 2014 and 2013, respectively. Cash and cash equivalents at December 31, 2014 and December 31, 2013 also included investments of $35.1 million and $203.8 million, respectively, in U.S. Treasury securities with original maturities of less than 90 days and investments in money market funds that invest in U.S. Treasury Securities.

At December 31, 2014, the Company held available for sale securities with a fair value totaling $423.7 million. These available for sale securities consisted of U.S. Treasury securities and had maturities less than one year. The Company evaluates securities with unrealized losses, if any, to determine whether such losses are other than temporary. The Company has determined that there were no other than temporary declines in fair values of its investments as of December 31, 2014. The Company classifies these securities as available for sale, however, the Company does not currently intend to sell its investments and it is more likely than not that the Company will recover the carrying value of these investments. Unrealized gains (losses) are reported as a component of accumulated other comprehensive loss in stockholders equity. The unrealized loss, net of tax, was $0.1 million as of December 31, 2014. As of December 31, 2013, the Company did not hold any available for sale securities.

Available for sale securities, including carrying value and estimated fair values, are summarized as follows:

	As of December 31, 2014			
	Cost	**Fair Value**	**Carrying Value**	**Unrealized Loss**
U.S. Treasury securities maturities < 1 year	$ 423,859	$ 423,746	$ 423,746	$ (113)
U.S. Treasury securities maturities > 1 year	$	$	$	$
Total	$ 423,859	$ 423,746	$ 423,746	$ (113)

6. Licensing and Commercialization Agreement with Novartis Pharma AG

On May 19, 2014, the Company entered into a licensing and commercialization agreement with Novartis Pharma AG. Under the Novartis Agreement, the Company granted Novartis exclusive rights under specified patent rights, know-how and trademarks controlled by the Company to manufacture, from bulk API supplied by the Company, standalone Fovista products and products combining Fovista with an anti-VEGF product to which Novartis has rights in a co-formulated product, for the treatment, prevention, cure or control of any human disease, disorder or condition of the eye, and to develop and commercialize those licensed products in all countries outside of the United States (the Novartis Territory). The Company has agreed to use commercially reasonable efforts to complete its ongoing pivotal Phase 3 clinical program for Fovista and Novartis has agreed to use commercially reasonable efforts to develop a standalone Fovista product and a co-formulated product containing Fovista and an anti-VEGF product to which Novartis has rights, as well as a pre- filled syringe presentation of such products and to use commercially reasonable efforts, subject to obtaining marketing approval, to commercialize licensed products in the Novartis Territory in accordance with agreed development and marketing plans. Novartis has also granted the Company options, subject to specified limitations, and to the extent such rights are controlled by Novartis, to obtain exclusive rights from Novartis to develop and commercialize in the United States the co-formulated and pre-filled syringe products developed by Novartis. The Company and Novartis have each granted the other options, subject to specified limitations, to obtain access to study data from certain clinical trials of licensed products that the Company or Novartis may conduct, including for use by the other in regulatory filings in its territory. The Company has agreed to exclusively supply Novartis, and Novartis has agreed to exclusively purchase from the Company, its clinical and commercial requirements for the bulk API in Fovista for use in

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licensed products in the Novartis Territory. The Company has agreed not to commercialize any product comprising Fovista or any other anti-PDGF product in the ophthalmic field in the Novartis Territory.

Novartis paid the Company a $200.0 million upfront fee upon execution of the Novartis Agreement. Novartis is also obligated to pay the Company up to an aggregate of $130.0 million if the Company achieves specified patient enrollment milestones for its Phase 3 clinical program for Fovista, $50.0 million of which was achieved in September 2014 and received by the Company in October 2014, and up to an aggregate of an additional $300.0 million upon achievement of specified regulatory approval milestones, including reimbursement approval, in certain countries in the Novartis Territory. In addition, Novartis has agreed to pay the Company up to an aggregate of an additional $400.0 million if Novartis achieves specified sales milestones in the Novartis Territory. Novartis also is obligated to pay the Company royalties with respect to standalone Fovista products and combination Fovista products that Novartis successfully commercializes. The Company will receive royalties at a mid-thirties percentage of net sales of standalone Fovista products and a royalty of approximately equal value for sales of combination Fovista products. Such royalties are subject to customary deductions, credits, and reductions for lack of patent coverage or market exclusivity. Novartis s obligation to pay such royalties will continue on a licensed product-by-licensed product and country-by-country basis until Novartis s last actual commercial sale of such licensed product in such country. The Company will continue to be responsible for royalties it owes to third parties on sales of Fovista products.

Novartis has agreed to pay the Company s manufacturing costs plus a specified percentage margin for supplies of the bulk API in Fovista that the Company supplies to Novartis. If the Company or Novartis exercises each of their respective rights to obtain access to study data from clinical trials conducted by the other party, the party exercising the option will be obligated to pay the other party s associated past development costs and share with such other party any future associated development costs. If the Company exercises its option to obtain Novartis-controlled rights to develop, manufacture and commercialize any co-formulated Fovista product in the United States, the Company will be obligated to pay a specified percentage of Novartis s associated past development costs and share with Novartis any future associated development costs. The Company and Novartis will also need to negotiate and agree on financial and other terms that would apply to such rights. If the Company exercises its option to obtain Novartis-controlled rights to develop and commercialize a pre-filled syringe product in the United States, the Company will be obligated to either enter into a supply agreement with Novartis under which the Company will pay Novartis its manufacturing cost plus a specified percentage margin for supplies of Fovista products in pre-filled syringes that Novartis supplies to the Company, or obtain supplies of products in pre-filled syringes from a third party manufacturer and pay Novartis a low single-digit percentage of the Company s net sales of such products.

The Company has retained control over the design and execution of its pivotal Phase 3 clinical program for Fovista and remains responsible for funding the costs of that program, subject to Novartis s responsibility to provide Lucentis, an anti-VEGF agent to which Novartis has rights in the Novartis Territory, for use in the Phase 3 trials in the Novartis Territory following the effective date of the Novartis Agreement. Novartis will have control over, and will be responsible for the costs of, all other clinical trials that may be required to obtain marketing approvals in the Novartis Territory for licensed products under the agreement. Novartis is also responsible for costs associated with co-formulation development, pre-filled syringe development and other development costs in the Novartis Territory, excluding regulatory filing fees in the European Union for the standalone Fovista product, for which the Company will be responsible.

The Novartis Agreement, unless earlier terminated by the Company or Novartis, will expire upon the expiration of Novartis s obligation to pay the Company royalties on net sales of licensed products. The Company and Novartis each may terminate the Novartis Agreement if the other party materially breaches the agreement and does not cure such breach within a specified cure period, if the other party experiences any specified insolvency event, if the other party challenges or assists a third party in challenging the validity or enforceability of certain patent rights controlled by the terminating party, or if the parties are prevented in any manner that materially adversely affects the progression of the development or commercialization of licensed products for a specified period as a result of specified governmental actions. Novartis may terminate the Novartis Agreement at any time without cause, or within a specified period after a change in control of the Company, as defined in the Novartis Agreement, or for specified safety reasons, effective at the end of a specified period following Novartis s written notice to the Company of Novartis s election to terminate the agreement. The Company may also terminate the agreement if Novartis determines to seek marketing approval of an alternative anti-PDGF product in the Novartis Territory as more fully described below. If the Company elects to terminate the Novartis Agreement because specified governmental actions prevent the parties from materially progressing the development or commercialization of licensed products as described above, the Company will be required to pay a substantial termination fee, with the specific amount of such fee determined based on the effective date of the termination. Following any termination, all rights to Fovista that the Company granted to Novartis, including, without limitation, the right to commercialize standalone Fovista products in the Novartis Territory, will revert to the Company, Novartis will perform specified activities in connection with transitioning to the Company the rights and responsibilities for the continued development, manufacture and commercialization of the standalone Fovista product for

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countries in the Novartis Territory, and the parties will cooperate on an orderly wind down of development and commercialization activities for other licensed products in the Novartis Territory.

Novartis has agreed to specified limitations on its ability to in- license, acquire or commercialize any anti-PDGF product that does not contain Fovista (an Alternative Anti-PDGF Product) in the Novartis Territory and, to the extent Novartis develops, in-licenses or acquires such a product, to make such product available to the Company in the United States under specified option conditions. If the Company exercises its option, the Company will be obligated to make certain payments to Novartis, including specified milestone and royalty payments. The amounts of such payments will vary based on the product s stage of clinical development at the time the Company exercise its option, whether the product is a standalone or combination product and whether Novartis exercises an option to co-promote such product in the United States. If Novartis determines to seek marketing approval of an Alternative Anti-PDGF Product in the Novartis Territory, the Company will, subject to specified limitations, have the option to terminate the Novartis Agreement, convert Novartis s exclusive licenses into non-exclusive licenses, or elect to receive a royalty on sales of such product by Novartis. If the Company elects to terminate the Novartis Agreement, Novartis will, subject to specified limitations, be required to pay to the Company, certain payments based on achievement, with respect to such product, of the milestones that would have otherwise applied to licensed products under the Novartis Agreement.

Activities under the licensing and commercialization Novartis Agreement were evaluated under ASC 605-25, *Revenue Recognition Multiple Element Arrangements* (ASC 605-25) (as amended by ASU 2009-13, *Revenue Recognition* (ASU 2009-13)) to determine if they represented a multiple element revenue arrangement. The Novartis Agreement includes the following deliverables: (1) an exclusive license to commercialize Fovista outside the United States (the License Deliverable); (2) the performance obligation to conduct research and development activities related to the Phase 3 Fovista clinical trials and certain Phase 2 trials for Fovista (the R&D Activity Deliverable); (3) the performance obligation to supply API to Novartis for development and manufacturing purposes (the Manufacturing Deliverable) and (4) the Company s obligation to participate on the joint operating committee established under the terms of the Novartis Agreement and related subcommittees (the Joint Operating Committee Deliverable). Novartis has the right, subject to the certain approval rights of the Company, to sublicense the exclusive royalty-bearing license to commercialize Fovista in the Novartis Territory. The Company s obligation to provide access to clinical and regulatory information as part of the License Deliverable includes the obligation to provide access to all clinical data, regulatory filings, safety data and manufacturing data to Novartis which is necessary for commercialization of Fovista in the Novartis Territory. The R&D Activity Deliverable includes the right and responsibility for the Company to conduct the Phase 3 Fovista clinical program and other studies of Fovista in the Novartis Territory which are necessary or desirable for regulatory approval or commercialization of Fovista. The Manufacturing Deliverable includes the obligation for the Company to supply API to Novartis for development and manufacturing purposes, for which Novartis has agreed to pay the Company s manufacturing costs, plus a specified margin. The Joint Operating Committee Deliverable includes the obligation to participate in the Joint Operating Committee and related subcommittees at least through the first anniversary of regulatory approval in the European Union. All of these deliverables were deemed to have stand-alone value and to meet the criteria to be accounted for as separate units of accounting under ASC 605-25. Factors considered in this determination included, among other things, the subject of the licenses and the research and development and commercial capabilities of Novartis. Accordingly, each unit will be accounted for separately.

Options are considered substantive if, at the inception of the arrangement, the Company is at risk as to whether the collaboration partner will choose to exercise the option. Factors that the Company considers in evaluating whether an option is substantive include the overall objective of the arrangement, the benefit the collaborator might obtain from the arrangement without exercising the option, the cost to exercise the option and the likelihood that the option will be exercised. For arrangements under which an option is considered substantive, the Company does not consider the item underlying the option to be a deliverable at the inception of the arrangement and the associated option fees are not included in allocable arrangement consideration, assuming the option is not priced at a significant and incremental discount. Conversely, for arrangements under which an option is not considered substantive or if an option is priced at a significant and incremental discount, the Company would consider the item underlying the option to be a deliverable at the inception of the arrangement and a corresponding amount would be included in allocable arrangement consideration. All of the options included in the Company s collaboration arrangement have been determined to be substantive, and none of the options are priced at a significant and incremental discount.

The Novartis Agreement provides that, if the Company elects to terminate the Novartis Agreement because specified governmental actions prevent the parties from materially progressing the development or commercialization of licensed products as described above, the Company will be required to pay a substantial termination fee, with the specific amount of such fee determined based on the effective date of the termination. The Company has concluded that this termination provision

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constitutes a contingent event that is unknown at the inception of the agreement. As such, the Company has recorded the $200.0 million upfront payment in deferred revenue, long-term until such time that the contingency related to this termination provision is resolved. The Company believes the enrollment milestones and certain of the regulatory milestones that may be achieved under the Novartis Agreement do not meet the recognition criteria within the definition of a milestone included in ASU 2010-17, *Revenue Recognition Milestone Method*, and therefore, payments received for the achievement of the enrollment milestones in excess of the termination fee will be included in the allocable arrangement consideration and allocated to the deliverables based upon BESP using the relative selling price method.

The Company believes the remaining regulatory approval milestones that may be achieved under the Novartis Agreement are consistent with the definition of a milestone included in ASU 2010-17, *Revenue Recognition Milestone Method*, and, accordingly, the Company will recognize payments related to the achievement of such milestones, if any, when the applicable milestone is achieved. Factors considered in this determination included scientific and regulatory risks that must be overcome to achieve each milestone, the level of effort and investment required to achieve each milestone, and the monetary value attributed to each milestone.

In September 2014, the Company achieved a $50.0 million enrollment-based milestone under the Novartis Agreement. The Company recognized revenue of approximately $41.3 million during the year ended December 31, 2014. Using the relative selling price method, the Company allocated revenue of $38.4 million to the license it delivered to Novartis under the Novartis Agreement, $2.0 million to research and development activities the Company has performed under the Novartis Agreement, and $0.9 million to the transfer of API to Novartis during the year ended December 31, 2014.

7. Financing Agreement with Novo A/S

In May 2013, the Company entered into a Purchase and Sale Agreement with Novo A/S, which is referred to as the Novo Agreement, pursuant to which the Company had the ability to obtain financing in three tranches in an amount of up to $125.0 million in return for the sale to Novo A/S of aggregate royalties of worldwide sales of (a) Fovista, (b) Fovista-Related Products, and (c) Other Products (each as defined in the Novo Agreement), calculated as low to mid-single digit percentages of net sales, with the royalty percentage determined by the amount of funding provided by Novo A/S.

The Novo Agreement provided for up to three separate purchases for a purchase price of $41.7 million each, at a first, second and third closing, for an aggregate purchase price of $125.0 million. In each purchase, Novo A/S acquires rights to a low single digit percentage of net sales. In each of May 2013, January 2014 and November 2014, the Company received cash payments of $41.7 million, $125.0 million in the aggregate, and Novo A/S received a right to receive royalties on net sales of Fovista at a mid-single digit percentage in the aggregate.

The royalty payment period covered by the Novo Agreement begins on commercial launch and ends, on a product-by-product and country-by-country basis, on the latest to occur of (i) the 12th anniversary of the commercial launch, (ii) the expiration of certain patent rights and (iii) the expiration of the regulatory exclusivity for each product in each country.

Under the terms of the Novo Agreement, the Company is not required to reimburse or otherwise compensate Novo A/S through any means other than the agreed royalty entitlement. In addition, the Company does not, under the terms of the Novo Agreement, have the right or obligation to prepay Novo A/S in connection with a change of control of the Company or otherwise.

The $125.0 million in aggregate proceeds from the three financing tranches under the Novo Agreement represents the full funding available under the Novo Agreement, and has been recorded as a liability on the Company s Balance Sheet as of December 31, 2014, in accordance with ASC Topic 730, *Research and Development*. Because there is a significant related party relationship between the Company and Novo A/S, the Company is treating its obligation to make royalty payments under the Novo Agreement as an implicit obligation to repay the funds advanced by Novo A/S. As the Company makes royalty payments in accordance with the Novo Agreement, it will reduce the liability balance. At the time that such royalty payments become probable and estimable, and if such amounts exceed the liability balance, the Company will impute interest accordingly on a prospective basis based on such estimates, which would result in a corresponding increase in the liability balance.

The Novo Agreement requires the establishment of a Joint Oversight Committee in the event that Novo A/S does not continue to have a representative on the Company s board of directors. The Joint Oversight Committee would have responsibilities that include discussion and review of all matters related to Fovista research, development, regulatory

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approval and commercialization, but there is no provision either implicit or explicit that gives the Joint Oversight Committee or its members decision-making authority.

8. Product and Technology Agreements

Transferred Technology and Assumed Agreements

Under an agreement dated July 27, 2007, the Company assumed the rights and obligations related to certain patents and know-how (the Transferred Technology) and under certain agreements (the Assumed Agreements) owned and/or controlled by OSI (Eyetech), Inc. (the Transferor) for use in the Company s activities in the research, development and commercial production of a product as defined in the agreement (the Divestiture Agreement). In consideration for the Transferred Technology and the Assumed Agreements, the Company made an upfront payment of $4.0 million to the Transferor. In addition, on August 9, 2007, the Company issued to the Transferor 3,000,000 shares of Junior Series A Preferred Stock which was valued at $1.00 per share based upon the Original Issue Price.

The Divestiture Agreement also entitles the Transferor to significant payments from the Company upon achievement of certain milestones, and to royalties on the Company s net sales of Products, as defined, and on terms set forth in the Divestiture Agreement.

The Divestiture Agreement may be terminated by either party in the event of the other party s insolvency or material breach (following a specified cure period). Unless terminated earlier by the Company or the Transferor, the Divestiture Agreement will remain in effect until the Company no longer has any financial obligations to the Transferor, after which the rights granted to the Company under the Divestiture Agreement will become perpetual and fully paid-up.

If the Company fails to satisfy its diligence obligations under the Divestiture Agreement, the Transferor may terminate the Divestiture Agreement as to particular countries with respect to which such failure has occurred, and upon such termination the Company will be obligated to transfer to the Transferor specified rights and licenses related to the product covered by the Divestiture Agreement and other related assets, and if the Company is then manufacturing such product or products, at the time of such termination, the Company may be obligated to provide transitional supply of the covered products to the Transferor, for the applicable countries.

The Assumed Agreements include a license, manufacturing and supply agreement (the Supply Agreement) with Nektar Therapeutics, AL (the Supplier) for a reagent linked with the active ingredient in the Company s lead product candidate. Prior to the Company s assumption of the Supply Agreement in 2007, the Transferor paid the Supplier approximately $0.3 million under the Supply Agreement. The Company has paid the Supplier an aggregate of approximately $21.5 million in milestone payments under the Supply Agreement, $19.8 million of which was paid and charged to research and development expense during the year ended December 31, 2014. Under the Supply Agreement, the Company is obligated to make certain milestone payments to the Supplier, as well as tiered royalties based on certain percentages of net sales. See Note 13 Commitments and Contingencies below.

The Supply Agreement, unless earlier terminated by either party, will expire upon the expiration of the Company s obligation to pay royalties to the Supplier on net sales of licensed products. The Supply Agreement may be terminated by either party in the event of the other party s material breach (following a specified cure period). The Company may terminate the Supply Agreement, without cause, effective at the end of a specified period following written notice to the Supplier, in which event the Company will be obligated to pay the Supplier specified termination fees and reimburse the Supplier for certain costs.

License Agreements

The Assumed Agreements also included an agreement with Archemix Corp. (the Licensor) for the Company s acquisition of an exclusive royalty-bearing license over certain patent rights and technology owned and/or controlled by the Licensor (the PDGF License) for use in the Company s activities in the research, development and commercial production of pharmaceutical products related to anti-PDGF aptamers (the PDGF Licensed Products) as contemplated in the agreement (the PDGF Agreement). In addition, on July 31, 2007, the Company also entered into an agreement with the Licensor for the Company s acquisition of an exclusive royalty-bearing license over certain patent rights and technology owned and/or controlled by the Licensor (the C5 License and together with the PDGF License, the Licenses) for use in the Company s activities in the research, development and commercial production of pharmaceutical products related to Zimura, formerly known as ARC1905 (the C5 Licensed Product), as contemplated in the agreement (the C5 License Agreement and together

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with the PDGF License Agreement, the License Agreements). In consideration of the Licenses, the Company paid the Licensor aggregate upfront fees of $1.0 million and, on August 9, 2007, issued to the Licensor an aggregate of 2,000,000 shares of Series A-1 Preferred Stock which was valued at $1.00 per share based on the cash price paid by the Series A Investors for similar shares on the same date.

The Licensor is also entitled to certain regulatory milestone payments and sales milestone payments under the License Agreements.

On September 12, 2011, the License Agreements, were amended to cover expanded licenses for all indications outside of the ophthalmic field (as defined in the amended license agreements (the Amended License Agreements)). Upon the execution of the Amended License Agreements, the Company issued 500,000 shares of Series B-1 Preferred Stock to the Licensor. The Series B-1 Preferred Stock was valued at $1.00 per share based upon the Original Issue Price, which was deemed to be fair value as of the date of this transaction.

Unless earlier terminated, the amended PDGF Agreement will expire upon the later of 10 years after the first commercial sale in any country of the last PDGF Licensed Product and the expiration of the last-to-expire valid claim of the PDGF licensed patents that covers a PDGF Licensed Product. Unless earlier terminated, the amended C5 Agreement will expire upon the later of 12 years after the first commercial sale in any country of the last C5 Licensed Product, the expiration of the last-to-expire valid claim of the C5 licensed patents, and the date on which no further payments of sublicensing income, if any, are to be received by the Company.

Either of the Amended License Agreements may be terminated by either party in the event of the other party s material breach (following a specified cure period). The Licensor may also terminate each of the Amended License Agreements, or may convert the Company s exclusive licenses to non-exclusive licenses, if the Company challenges or assists a third party in challenging the validity or enforceability of any of the patents licensed under the applicable Amended License Agreement. The Company may terminate each of the Amended License Agreements at any time and for any or no reason effective at the end of a specified period following written notice to the Licensor.

9. Property, Plant and Equipment

Property and equipment at December 31, 2014 and 2013 were as follows:

	Useful Life (Years)	December 31, 2014	December 31, 2013
Manufacturing and clinical equipment	7 - 10	$ 617	$
Computer and other office equipment	5	292	$ 85
Furniture and fixtures	7	591	117
Leasehold improvements	3 - 5	357	
		1,857	202
Accumulated depreciation		(302)	(175)
Property and equipment, net		$ 1,555	$ 27

For the years ended December 31, 2014, 2013 and 2012, depreciation expense was $127 thousand, $20 thousand, $31 thousand, respectively.

10. Income Taxes

The Company utilizes the liability method of accounting for deferred income taxes. Under this method, deferred tax liabilities and assets are recognized for the expected future tax consequences of temporary differences between the carrying amounts and the tax basis of assets and liabilities. A valuation allowance is established against deferred tax assets when, based on the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. The Company s policy is to record interest and penalties on uncertain tax positions as income tax expense. As of December 31, 2014 and December 31, 2013, the Company does not believe any material uncertain tax positions were present. Accordingly, interest and penalties have not been accrued due to an uncertain tax position.

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Deferred income taxes reflect the net effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. A reconciliation of the statutory U.S. federal rate to the Company s effective tax rate is as follows:

	Years ended December 31, 2014 (Restated)	2013	2012
Percent of pre-tax income:			
U.S. federal statutory income tax rate	35.0%	35.0%	35.0%
State taxes, net of federal benefit	6.8%		0.0%
Permanent items	2.3%	(2.2)%	(1.0)%
Research and development credit		2.7%	1.0%
Change in valuation allowance	(89.5)%	(35.5)%	(35.0)%
Effective income tax rate	(45.4)%	0.0%	0.0%

The components of income tax expense are as follows:

	Years ended December 31, 2014 (Restated)	2013	2012
Current:			
Federal	$ 29,505	$	$
State	11,440		
Deferred:			
Federal	(4,469)		
State			
Income tax expense	$ 36,476	$	$

Significant components of the Company s deferred tax assets (liabilities) for 2014 and 2013 consist of the following:

	As of December 31, 2014 (Restated)	2013
Deferred tax assets (liabilities)		
Deferred revenue	$ 120,611	$ 16,667
License and technology payments	14,251	6,407
Share-based compensation	5,679	1,396
Accrued Expenses	442	
Depreciation	(328)	(7)
Federal net operating loss carryforwards		30,084
State and local net operating loss carryforwards		5,026
Federal research and development credit carryforwards		2,966
State research and development credit carryforwards		1,483
Deferred income tax assets	140,655	64,022
Valuation allowance	(136,138)	(64,022)

Net deferred tax assets	$	4,517	$

In assessing the realizability of deferred tax assets, the Company considers whether it is more likely than not that some portion or all of the deferred tax assets will not be realized. The ultimate realization of deferred tax assets is dependent upon the generation of taxable income during the periods in which the temporary differences representing net future deductible amounts become deductible, and is impacted by the Company s ability to carryback losses to previous years in which the Company had taxable income. In connection with the $83.3 million the Company received from Novo A/S in 2014 under the Novo Agreement, the $200.0 million the Company received from Novartis in May 2014, a portion of which has been deferred for income tax purposes, and the $50.0 million enrollment-based milestone payment the Company earned in September 2014

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under the Novartis Agreement, the Company expects to have taxable income in 2014, after taking into account the utilization of its federal net operating losses from prior years and the utilization of its research and development tax credits. As such, the Company made income tax payments of $40.2 million during the year ended December 31, 2014. Due to the Company s history of losses and lack of other positive evidence to support taxable income after the 2014 tax year, the Company has recorded a valuation allowance against those deferred tax assets that are not expected to be realized. The valuation allowance was approximately $136.1 million and $64.0 million as of December 31, 2014 and 2013, respectively, representing an increase of $72.1 million.

Actual tax benefits result from a stock plan award transaction that exceeds the tax benefit associated with the grant date fair value of the related stock award. The Company recognizes these excess tax benefits in additional paid in capital only if an incremental tax benefit would be realized after considering all other tax benefits presently available to the Company. In 2013, deferred tax assets relating to employee share-based compensation deductions were reduced to reflect exercises of non-qualified stock option grants. Although certain of these deductions were reported on the corporate tax returns and increased net operating losses, these related tax benefits were not recognized for financial reporting purposes.

The Company s federal, state, and local net operating loss carryforwards of approximately $91.3 million are expected to be utilized in 2014. Utilization of the net operating losses and general business tax credits carryforwards may be subject to a substantial limitation under Sections 382 and 383 of the Internal Revenue Code of 1986 due to changes in ownership of the Company that have occurred previously or that could occur in the future. These ownership changes may limit the amount of net operating losses and general business tax credits carryforwards that can be utilized annually to offset future taxable income and tax, respectively. In general, an ownership change, as defined by Section 382, results from transactions increasing the ownership of certain stockholders or public groups in the stock of a corporation by more than 50 percentage points over a three-year period. The Company has completed a study to determine whether it had undergone an ownership change since the Company s inception. The Company concluded that it had not undergone an ownership change and the Company expects that it will have the ability to utilize its net operating loss carryforwards against taxable income in 2014.

As further described in Note 18, the Company restated its balance sheets and statements of operations as of and for the quarters ended June 30, 2014, September 30, 2014 and December 31, 2014 and as of and for the year ended December 31, 2014 to reflect the correction of its accounting for valuation allowances related to deferred tax assets. See Note 18 for a summary of the effect of the restatement by major financial statement line items as of these dates and for these periods. The restatement has no effect on any amount reported in the periods prior to June 30, 2014.

11. Operating Leases

The Company leases office spaces located in Princeton, New Jersey and New York, New York under operating lease arrangements. The Company s Princeton, New Jersey office space lease expires in January 2019, whereas the Company s New York, New York office space lease expires in February 2020. Future minimum rental commitments under non-cancelable operating leases in effect as of December 31, 2014, are as follows:

2015	$	1,562
2016		1,629
2017		1,669
2018		1,700
2019		1,499
Thereafter		239

Total	$	8,298

Rent expense is calculated on the straight-line basis and amounted to $1.0 million, $0.5 million and $0.4 million for the years ended December 31, 2014, 2013 and 2012, respectively.

12. Security Deposits

Security deposits consist of amounts required to secure the Company s performance of its obligations under the operating leases for its New Jersey and New York offices. Such amounts were approximately $0.3 million as of December 31, 2014 and 2013, respectively.

13. Commitments and Contingencies

Under various agreements, the Company may be required to pay royalties and make milestone payments. These agreements include the following:

- Under the Company s acquisition agreement with OSI (Eyetech), Inc., which agreement is now held by OSI Pharmaceuticals, LLC., or OSI Pharmaceuticals, a subsidiary of Astellas US, LLC, for rights to particular

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anti-PDGF aptamers, including Fovista, the Company is obligated to pay to OSI Pharmaceuticals future one-time payments of $12.0 million in the aggregate upon marketing approval in the United States and the European Union of a covered anti-PDGF product. The Company is also obligated to pay to OSI Pharmaceuticals a royalty at a low single-digit percentage of net sales of any covered anti-PDGF product the Company successfully commercializes.

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- Under a license agreement with Archemix Corp., or Archemix, with respect to pharmaceutical products comprised of or derived from any anti-PDGF aptamer, the Company is obligated to make future payments to Archemix of up to an aggregate of $14.0 million if the Company achieves specified clinical and regulatory milestones with respect to Fovista, up to an aggregate of $3.0 million if the Company achieves specified commercial milestones with respect to Fovista and, for each other anti-PDGF aptamer product that it may develop under the agreement, up to an aggregate of approximately $18.8 million if the Company achieves specified clinical and regulatory milestones and up to an aggregate of $3.0 million if the Company achieves specified commercial milestones. No royalties are payable to Archemix under this license agreement.

- Under a license agreement with Archemix with respect to pharmaceutical products comprised of or derived from anti-C5 aptamers, for each anti-C5 aptamer product that the Company may develop under the agreement, including Zimura, the Company is obligated to make future payments to Archemix of up to an aggregate of $57.5 million if the Company achieves specified development, clinical and regulatory milestones and, as to all anti-C5 products under the agreement collectively, up to an aggregate of $22.5 million if the Company achieves specified commercial milestones. The Company is also obligated to pay Archemix a double-digit percentage of specified non-royalty payments the Company may receive from any sublicensee of its rights under this license agreement. No royalties are payable to Archemix under this license agreement.

- Under a license, manufacturing and supply agreement with Nektar Therapeutics, or Nektar, for specified pegylation reagents used to manufacture Fovista, the Company is obligated to make future payments to Nektar of up to an aggregate of $6.5 million if the Company achieves specified clinical and regulatory milestones, and an additional payment of $3.0 million if the Company achieves a specified commercial milestone with respect to Fovista. The Company is obligated to pay Nektar tiered royalties at low to mid-single-digit percentages of net sales of any licensed product the Company successfully commercializes, with the royalty percentage determined by the Company s level of licensed product sales and the extent of patent coverage for the licensed product and whether the Company has granted a third-party commercialization rights to the licensed product. In June 2014, the Company paid Nektar $19.8 million in connection with its entry into the Novartis Agreement.

- Under the Novo Agreement, with respect to Fovista, the Company will be obligated to pay Novo A/S a mid-single-digit percentage royalty based on worldwide sales of Fovista. See Note 7 Financing Agreement with Novo A/S above for further information about Novo Agreement.

- Under the clinical supply agreement with Agilent Technologies, Inc., Agilent has agreed to manufacture and supply to the Company, and the Company has agreed to purchase from Agilent, a specified percentage of its clinical requirements in specified jurisdictions of the active pharmaceutical ingredient in the Company s product candidate Fovista. The Company s agreement with Agilent has an initial five year term, which is subject to automatic renewal absent termination by either party in accordance with the terms of the Agreement. The Agreement provides for pricing structured on a tiered basis with the price reduced as the volume ordered increases. The Company may terminate the agreement or any statement of work thereunder upon 12 months prior written notice to Agilent.

The Company also has employment agreements with certain employees which require the funding of a specific level of payments, if certain events, such as a change in control, termination without cause or retirement, occur.

In addition, in the course of normal business operations, the Company has agreements with contract service providers to assist in the performance of the Company s research and development and manufacturing activities. Expenditures to CROs represent a significant cost in clinical development. The Company can elect to discontinue the work under these agreements at any time. The Company could also enter into additional collaborative research, contract research, manufacturing, and supplier agreements in the future, which may require upfront payments and even long-term commitments of cash.

14. Stock Option and Compensation Plans

The Company adopted its 2007 Stock Incentive Plan (the 2007 Plan) for employees, directors and consultants for the purpose of advancing the interests of the Company stockholders by enhancing its ability to attract, retain and motivate persons who are expected to make important contributions to the Company. The 2007 Plan provided for the granting of stock option awards, restricted stock awards, and other stock-based and cash-based awards. Following the effectiveness of the 2013

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Stock Incentive Plan described below in connection with the closing of the Company s initial public offering, the Company is no longer granting additional awards under the 2007 Plan.

In August 2013, the Company s board of directors adopted and the Company s stockholders approved the 2013 stock incentive plan (the 2013 Plan), which became effective immediately prior to the closing of the Company s initial public offering. The 2013 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, and other stock-based awards. Upon effectiveness of the 2013 Plan, the number of shares of the Company s common stock that were reserved for issuance under the 2013 Plan was the sum of (1) such number of shares (up to approximately 3,359,641 shares) as is equal to the sum of 739,317 shares (the number of shares of the common stock then available for issuance under the 2007 Plan), and such number of shares of the Company s common stock that are subject to outstanding awards under the 2007 Plan that expire, terminate or are otherwise surrendered, canceled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right plus (2) an annual increase, to be added the first business day of each fiscal year, beginning with the fiscal year ending December 31, 2014 and continuing until, and including, the fiscal year ending December 31, 2023, equal to the lowest of 2,542,372 shares of the Company s common stock, 4% of the number of shares of the Company s common stock outstanding on the first day of the fiscal year and an amount determined by its board of directors. The Company s employees, officers, directors, consultants and advisors are eligible to receive awards under the 2013 Plan. However, incentive stock options may only be granted to employees of the Company.

In connection with the evergreen provisions of the 2013 Plan, the number of shares available for issuance under the 2013 Plan was increased by approximately 1,257,000 shares, effective as of January 1, 2014. As of December 31, 2014, the Company had approximately 479,000 shares available for grant under the 2013 Plan. In connection with the evergreen provisions of the 2013 Plan, the number of shares available for issuance under the 2013 Plan was further increased by approximately 1,360,000 shares, effective as of January 1, 2015.

A summary of the stock option activity, weighted average exercise prices, options outstanding and exercisable as of December 31, 2014, 2013 and 2012 is as follows:

	Year ended December 31,					
	2014		2013		2012	
	Common Stock Options	Weighted Average Exercise Price	Common Stock Options	Weighted Average Exercise Price	Common Stock Options	Weighted Average Exercise Price
Outstanding at beginning of year:	2,708	$ 9.41	1,344	$ 1.65	1,113	$ 1.30
Granted	1,744	$ 33.92	1,583	$ 14.82	250	$ 3.12
Exercised	(621)	$ 4.75	(151)	$ 0.61	(19)	$ 0.12
Expired or forfeited	(151)	$ 28.50	(68)	$ 1.53		
Outstanding at end of year:	3,680	$ 21.03	2,708	$ 9.41	1,344	$ 1.65

	Year ended December 31,		
	2014	2013	2012
Options exercisable at end of year	993	984	823
Weighted average grant date fair value (per share) of options granted during the period	$ 24.41	$ 10.48	$ 1.65

	As of December 31, 2014				
	Options Outstanding			Options Exercisable	
Range of Exercise Prices	Total Options Outstanding	Weighted Average Remaining Life (Years)	Weighted Average Exercise Price	Number Exercisable	Weighted Average Exercise Price
$0.12 - $10.03	1,243	7.1	$ 5.67	761	$ 3.66
$10.04 - $20.00	534	8.5	$ 13.38	132	$ 13.39
$20.01 - $30.00	179	8.8	$ 25.64	46	$ 25.43
$30.00 - $43.90	1,724	9.2	$ 34.00	54	$ 34.79
	3,680		$ 21.03	993	$ 7.65
Aggregate Intrinsic Value	$ 87,728			$ 36,965	

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Cash proceeds from, and the aggregate intrinsic value of, stock options exercised during the years ended December 31, 2014, 2013 and 2012, respectively, were as follows:

	Year ended December 31, 2014	2013	2012
Cash Proceeds from options exercised	$ 2,949	$ 94	$ 2
Aggregate intrinsic value of options exercised	$ 21,646	$ 4,545	$ 28

In connection with stock option awards granted to employees, the Company recognized share-based compensation expense approximately $11.3 million, $2.2 million, and $0.5 million, for the years ended December 31, 2014, 2013, and 2012, respectively, net of expected forfeitures. As of December 31, 2014, there was approximately $37.8 million of unrecognized compensation costs, net of estimated forfeitures, related to stock option awards to employees, which are expected to be recognized over a remaining weighted average period of 3.1 years.

In connection with stock options awards granted to consultants, the Company recognized approximately $1.4 million, $0.7 million and $0.1 million in share-based compensation expense during the years ended December 31, 2014, 2013 and 2012, respectively, net of expected forfeitures. As of December 31, 2014, there was approximately $4.1 million of unrecognized compensation costs, net of estimated forfeitures, related to stock option award granted to consultants which are expected to be recognized over a remaining weighted average period of 2.9 years.

As of December 31, 2014, the Company had approximately 37,000 restricted stock units outstanding. In connection with restricted stock units granted to employees, the Company recognized share-based compensation of approximately $0.3 million during the year ended December 31, 2014, net of expected forfeitures. The Company did not recognize any share-based compensation expense related to restricted stock units during year ended December 31, 2013. As of December 31, 2014, there was approximately $0.9 million of unrecognized compensation costs, net of estimated forfeitures, related to restricted stock units granted to employees to be recognized over a remaining weighted average period of 2.4 years.

On September 30, 2014, the Company issued to an employee an option to purchase 200,000 shares of its common stock at an exercise price of $38.93 per share. This option grant was an inducement grant issued outside the Company s existing equity compensation plan in accordance with NASDAQ listing rule 5635(c)(4).

On October 3, 2014, the Company issued to an employee an option to purchase 150,000 shares of its common stock at an exercise price of $38.41 per share. This option grant was an inducement grant issued outside the Company s existing equity compensation plan in accordance with NASDAQ listing rule 5635(c)(4).

15. Employee Benefit Plan

The Company maintains a defined contribution 401(k) plan available to employees. Employee contributions are voluntary and are determined on an individual basis, limited by the maximum amounts allowable under federal tax regulations. The Company s matching contributions to employees totaled approximately $0.2 million during the year ended December 31, 2014. The Company did not match any of the employee contributions during the years ended December 31, 2013 and 2012.

16. Fair Value Measurements

ASC 820, *Fair Value Measurements and Disclosures*, defines fair value as the price that would be received to sell an asset, or paid to transfer a liability, in the principal or most advantageous market in an orderly transaction between market participants on the measurement date. The fair value standard also establishes a three-level hierarchy, which requires an entity to maximize the use of observable inputs and minimize the use of unobservable inputs when measuring fair value.

The valuation hierarchy is based upon the transparency of inputs to the valuation of an asset or liability on the measurement date. The three levels are defined as follows:

- Level 1 inputs to the valuation methodology are quoted prices (unadjusted) for an identical asset or liability in an active market. The Company s Level 1 assets consist of investments in U.S. Treasury money market funds and U.S. Treasury securities.

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• Level 2 inputs to the valuation methodology include quoted prices for a similar asset or liability in an active market or model-derived valuations in which all significant inputs are observable for substantially the full term of the asset or liability.

• Level 3 inputs to the valuation methodology are unobservable and significant to the fair value measurement of the asset or liability.

The following table presents, for each of the fair value hierarchy levels required under ASC 820, the Company s assets and liabilities that are measured at fair value on a recurring basis as of December 31, 2014:

	Fair Value Measurement Using		
	Quoted prices in active markets for identical assets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets			
Investments in U.S. Treasury money market funds*	$ 35,111	$	$
Investments in U.S. Treasury securities maturities < three months*	$	$	$
Investments in U.S. Treasury securities maturities < 1 year	$ 423,746	$	$

The following table presents, for each of the fair value hierarchy levels required under ASC 820, the Company s assets and liabilities that are measured at fair value on a recurring basis as of December 31, 2013:

	Fair Value Measurement Using		
	Quoted prices in active markets for identical assets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets			
Investments in U.S. Treasury money market funds*	$ 203,828	$	$

* Investments in U.S. Treasury money market funds and U.S. Treasury securities with maturities less than three months are reflected in cash and cash equivalents in the accompanying Balance Sheets.

17. Selected Quarterly Financial Information (unaudited)

The following is a summary of the quarterly results of operations for the years ended December 31, 2014 and 2013:

	2014			
	March 31	June 30 (Restated)	September 30 (Restated)	December 31 (Restated)
Collaboration revenue	$	$	$ 39,575	$ 1,684
Research and development expenses	14,377	34,707	17,105	22,196
General and administrative expenses	6,349	7,570	8,812	10,656
Income (loss) from operations	(20,726)	(42,277)	13,658	(31,168)
Net income (loss) attributable to common stockholders	$ (20,682)	$ (72,990)	$ 8,552	$ (31,652)
Basic earnings (loss) per common share	$ (0.64)	$ (2.19)	$ 0.26	$ (0.94)
Diluted earnings (loss) per common share	$ (0.64)	$ (2.19)	$ 0.25	$ (0.94)

	2013			
	March 31	June 30	September 30	December 31
Collaboration revenue	$	$	$	$
Research and development expenses	2,390	4,345	11,101	15,379
General and administrative expenses	1,738	3,241	4,166	5,065
Loss from operations	(4,128)	(7,586)	(15,267)	(20,444)
Net loss attributable to common shareholders	$ (6,361)	$ (11,863)	$ (18,424)	$ (20,388)
Basic and diluted earnings per common share	$ (4.33)	$ (8.07)	$ (10.26)	$ (0.65)

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As further described in Note 18, the Company restated its balance sheets and statements of operations as of and for the quarters ended June 30, 2014, September 30, 2014 and December 31, 2014 and as of and for the year ended December 31, 2014 to reflect the correction of its accounting for valuation allowances related to deferred tax assets. See Note 18 for a summary of the effect of the restatement by major financial statement line items as of these dates and for these periods. The restatement has no effect on any amount reported in the periods prior to June 30, 2014.

18. Restatement of Previously Issued Financial Statements

Subsequent to the issuance of the Company s Annual Report on Form 10-K for the year ended December 31, 2014, the Company and the Audit Committee of the Board of Directors concluded that the Company should restate its balance sheet as of December 31, 2014, and its statements of operations, comprehensive loss, cash flows and changes in stockholders equity (deficit) for the year ended December 31, 2014 to correct the following errors:

In the second quarter of 2014, the Company recorded an income tax benefit by reducing a portion of its valuation allowance against its gross deferred tax assets. In determining the amount of the valuation allowance release, the Company considered anticipated 2015 tax losses which would generate a refund of a portion of federal income taxes paid in 2014. The Company has now determined that, as a matter of accounting principle, the net deferred tax asset recorded on its balance sheet was overstated and the income tax provision on its statement of operations was understated as of and for the periods ending June 30, 2014, September 30, 2014 and December 31, 2014. The Company s cash position and operating expenses were not affected. The restatement had no effect on amounts reported in periods prior to the quarter ended June 30, 2014.

The following tables summarize the effect of the restatement by major financial statement line as of June 30, 2014 and for the three and six months ended June 30, 2014, as of September 30, 2014 and for the three and nine months ended September 30, 2014, and as of December 31, 2014 and for the three months and year ended December 31, 2014:

Balance Sheets

	June 30, 2014		September 30, 2014		December 31, 2014	
	As Reported	As Restated	As Reported	As Restated	As Reported	As Restated
	(unaudited)		(unaudited)			
Deferred tax assets	$ 837	$	$ 1,069	$ 49	$ 293	$ 50
Total current assets	$ 442,155	$ 441,318	$ 462,644	$ 461,624	$ 473,625	$ 473,382
Deferred tax assets, non-current	$ 19,635	$	$ 26,187	$ 4,404	$ 22,808	$ 4,467
Total assets	$ 478,126	$ 457,654	$ 490,486	$ 467,683	$ 498,370	$ 479,786
Income taxes payable	$ 29,540	$ 29,559	$ 7,785	$ 7,785	$	$
Total current liabilities	$ 35,362	$ 35,381	$ 24,039	$ 24,039	$ 19,831	$ 19,831
Total liabilities	$ 318,695	$ 318,714	$ 314,591	$ 314,591	$ 351,249	$ 351,249
Accumulated deficit	$ (256,231)	$ (276,722)	$ (245,367)	$ (268,170)	$ (281,238)	$ (299,822)
Total stockholders equity	$ 159,431	$ 138,940	$ 175,895	$ 153,092	$ 147,121	$ 128,537
Total liabilities and stockholders equity	$ 478,126	$ 457,654	$ 490,486	$ 467,683	$ 498,370	$ 479,786

Statement of Operations

	Three Months Ended June 30, 2014		Six Months Ended June 30, 2014	
	As Reported	As Restated	As Reported	As Restated
	(unaudited)		(unaudited)	
Income tax provision	$ 10,294	$ 30,785	$ 10,294	$ 30,785
Net loss	$ (52,499)	$ (72,990)	$ (73,181)	$ (93,672)
Basic loss per common share	$ (1.57)	$ (2.19)	$ (2.23)	$ (2.85)
Diluted loss per common share	$ (1.57)	$ (2.19)	$ (2.23)	$ (2.85)

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	Three Months Ended September 30, 2014		Nine Months Ended September 30, 2014	
	As Reported	As Restated	As Reported	As Restated
	(unaudited)		(unaudited)	
Income tax provision	$ 2,867	$ 5,179	$ 13,161	$ 35,964
Net income (loss)	$ 10,864	$ 8,552	$ (62,317)	$ (85,120)
Basic earnings (loss) per common share	$ 0.32	$ 0.26	$ (1.88)	$ (2.57)
Diluted earnings (loss) per common share	$ 0.31	$ 0.25	$ (1.88)	$ (2.57)

	Three Months Ended December 31, 2014		Year Ended December 31, 2014	
	As Reported	As Restated	As Reported	As Restated
	(unaudited)			
Income tax provision	$ 4,731	$ 512	$ 17,892	$ 36,476
Net loss	$ (35,871)	$ (31,652)	$ (98,188)	$ (116,772)
Basic loss per common share	$ (1.06)	$ (0.94)	$ (2.95)	$ (3.51)
Diluted loss per common share	$ (1.06)	$ (0.94)	$ (2.95)	$ (3.51)

Statement of Comprehensive Income (Loss)

	Three Months Ended June 30, 2014		Three Months Ended September 30, 2014	
	As Reported	As Restated	As Reported	As Restated
	(unaudited)		(unaudited)	
Net income (loss)	$ (52,499)	$ (72,990)	$ 10,864	$ 8,552
Comprehensive income (loss)	$ (52,492)	$ (72,983)	$ 10,874	$ 8,562

	Six Months Ended June 30, 2014		Nine Months Ended September 30, 2014		Year Ended December 31, 2014	
	As Reported	As Restated	As Reported	As Restated	As Reported	As Restated
	(unaudited)		(unaudited)		(unaudited)	
Net loss	$ (73,181)	$ (93,672)	$ (62,317)	$ (85,120)	$ (98,188)	$ (116,772)
Comprehensive loss	$ (73,156)	$ (93,647)	$ (62,282)	$ (85,085)	$ (98,253)	$ (116,837)

Statement of Cash Flows

	Six Months Ended June 30, 2014		Nine Months Ended September 30, 2014		Year Ended December 31, 2014	
	As Reported	As Restated	As Reported	As Restated	As Reported	As Restated
	(unaudited)		(unaudited)		(unaudited)	
Net loss	$ (73,181)	$ (93,672)	$ (62,317)	$ (85,120)	$ (98,188)	$ (116,772)
Deferred income taxes	$ (19,246)	$ 1,226	$ (24,947)	$ (2,144)	$ (18,798)	$ (214)
Income taxes payable	$ 29,540	$ 29,559	$ 7,785	$ 7,785	$	$

The restatement did not have any effect on the previously reported amounts of total operating, investing, or financing cash flows in the Company s statements of cash flows for the six months ended June 30, 2014, the nine months ended September 30, 2014 or the year ended December 31, 2014.

Financial information included in the accompanying financial statements and the notes thereto reflect the effects of the corrections described above.

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EXHIBIT INDEX

Exhibit Number	Description of Exhibit	Incorporated by Reference Form	File Number	Date of Filing	Exhibit Number	Filed Herewith
3.1	Restated Certificate of Incorporation of the Registrant	S-1/A	333-190643	9/9/2013	3.3	
3.2	Amended and Restated Bylaws of the Registrant	S-1/A	333-190643	9/9/2013	3.4	
4.1	Specimen Stock Certificate evidencing the shares of common stock	S-1/A	333-190643	9/9/2013	4.1	
10.1	Amended and Restated 2007 Stock Incentive Plan, as amended	S-1	333-190643	8/15/2013	10.1	
10.2	Form of Incentive Stock Option Agreement under Amended and Restated 2007 Stock Incentive Plan	S-1	333-190643	8/15/2013	10.2	
10.3	Form of Nonstatutory Stock Option Agreement under 2007	S-1	333-190643	8/15/2013	10.3	
10.4	2013 Stock Incentive Plan	10-K		3/2/2015	10.4	
10.5	Form of Incentive Stock Option Agreement under 2013 Stock Incentive Plan	S-1/A	333-190643	9/9/2013	10.5	
10.6	Form of Nonqualified Stock Option Agreement under 2013 Stock Incentive Plan	S-1/A	333-190643	9/9/2013	10.6	
10.7	Form of Restricted Stock Unit Agreement under 2013 Stock Incentive Plan	10-K		3/2/2015	10.7	
10.8	Lease Agreement, dated as of September 30, 2007, between the Registrant and One Penn Plaza LLC, as the same has been supplemented by agreement dated March 12, 2013 and amended by the Amendment of Lease, dated as of August 30, 2013, Second Amendment to Lease, entered into on January 7, 2014, Third Amendment of Lease, dated as of April 18, 2014, and the Fourth Amendment of Lease, dated as of December 22, 2014	10-K		3/2/2015	10.8	
10.9	Lease Agreement with Carnegie 214 Associates Limited Partnership, dated as of October 25, 2013	S-1	333-193681	1/31/2014	10.8	
10.10	Divestiture Agreement, dated as of July 27, 2007, by and between the Registrant and (OSI) Eyetech, Inc.	S-1	333-190643	8/15/2013	10.9	
10.11	License, Manufacturing and Supply Agreement, dated as of September 30, 2006, by and between Nektar Therapeutics AL, Corporation and (OSI) Eyetech, Inc., as the same was assigned to the Registrant on July 27, 2007 and amended by Amendment No. 1 thereto, dated as of April 5,	S-1	333-190643	8/15/2013	10.10	

2012, and supplemented by a letter agreement, dated as of June 20, 2013

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Exhibit Number	Description of Exhibit	Incorporated by Reference Form	File Number	Date of Filing	Exhibit Number	Filed Herewith
10.12	Amended and Restated Exclusive License Agreement, dated as of September 12, 2011, by and between the Registrant and Archemix Corp., as amended by Amendment No. 1 thereto dated December 20, 2011 and supplemented by a letter agreement, dated as of April 30, 2012	S-1	333-190643	8/15/2013	10.11	
10.13	Amended and Restated Exclusive License Agreement, dated as of September 12, 2011, by and between the Registrant and Archemix Corp., as amended by Amendment No. 1 thereto, dated as of December 20, 2011	S-1	333-190643	8/15/2013	10.12	
10.14	Purchase and Sale Agreement, dated as of May 23, 2013, by and between the Registrant and Novo A/S	S-1	333-190643	8/15/2013	10.13	
10.15+	Offer of Employment between the Registrant and David Guyer	S-1/A	333-190643	9/9/2013	10.14	
10.16+	Second Amended and Restated Employment Agreement between the Registrant and Samir Patel	S-1/A	333-190643	9/9/2013	10.15	
10.17	Office Lease Agreement, dated as of August 22, 2013, by and between the Registrant and PSN Partners, L.P.	S-1/A	333-190643	9/9/2013	10.17	
10.18	Clinical Manufacturing and Supply Agreement by and between the Registrant and Agilent Technologies, Inc. dated May 2, 2014	10-Q		8/6/2014	10.1	
10.19	Licensing and Commercialization Agreement by and between the Registrant and Novartis Pharma AG dated May 19, 2014	10-Q		8/6/2014	10.2	
10.20+	Offer of Employment between the Registrant and Michael G. Atieh dated September 20, 2014	10-Q		11/12/2014	10.1	
10.21+	Nonstatutory Stock Option Agreement between the Registrant and Michael G. Atieh, dated September 30, 2014	10-Q		11/12/2014	10.2	
10.22+	Offer of Employment between the Registrant and Todd N. Smith dated September 29, 2014	10-K		3/2/2015	10.22	
10.23+	Nonstatutory Stock Option Agreement between the Registrant and Todd N. Smith, dated October 3, 2014	10-K		3/2/2015	10.23	
10.24	Amendment No. 1 to the Purchase and Sale Agreement, dated as of May 23, 2013, by and between the Registrant and Novo A/S	10-K		3/2/2015	10.24	
23.1	Consent of Ernst & Young LLP	10-K		3/2/2015	23.1	
23.2	Consent of Ernst & Young LLP				23.2	Yes

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		Incorporated by Reference				
Exhibit Number	Description of Exhibit	Form	File Number	Date of Filing	Exhibit Number	Filed Herewith
31.1	Certification of principal executive officer pursuant to Rule 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.	10-K		3/2/2015	31.1	
31.2	Certification of principal financial officer pursuant to Rule 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.	10-K		3/2/2015	31.2	
31.3	Certification of principal executive officer pursuant to Rule 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.					Yes
31.4	Certification of principal financial officer pursuant to Rule 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.					Yes
32.1	Certification of principal executive officer pursuant to 18 U.S.C. §1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.	10-K		3/2/2015	32.2	
32.2	Certification of principal financial officer pursuant to 18 U.S.C. §1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.	10-K		3/2/2015	32.2	
32.3	Certification of principal executive officer pursuant to 18 U.S.C. §1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					Yes
32.4	Certification of principal financial officer pursuant to 18 U.S.C. §1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					Yes
101.INS	XBRL Instance Document.					Yes
101.SCH	XBRL Taxonomy Extension Schema Document.					Yes
101.CAL	XBRL Taxonomy Calculation Linkbase Document.					Yes
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.					Yes
101.LAB	XBRL Taxonomy Label Linkbase Document.					Yes
101.PRE	XBRL Taxonomy Presentation Linkbase Document.					Yes

Confidential treatment has been granted as to certain portions, which portions have been omitted and separately filed with the Securities and Exchange Commission.

\+ Management contract or compensatory plan or arrangement filed in response to Item 15(a)(3) of the Instructions to the Annual Report on Form 10-K.