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**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

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**FORM 10-Q**

(Mark One)

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

For the quarterly period ended September 30, 2014

OR

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

Commission file number: 001-36080

**Ophthotech Corporation**

(Exact name of registrant as specified in its charter)

**Delaware**

(State or other jurisdiction of incorporation or organization)

**20-8185347**

(I.R.S. Employer Identification Number)

**One Penn Plaza, 19<sup>th</sup> Floor**

**New York, NY**

(Address of principal executive offices)

**10119**

(Zip Code)

**(212) 845-8200**

(Registrant's telephone number, including area code)

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, 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). Yes  No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer

Accelerated filer

Non-accelerated filer

Smaller reporting company

(Do not check if a smaller reporting company)

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

As of November 5, 2014 there were 33,651,188 shares of Common Stock, \$0.001 par value per share, outstanding.

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This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our product development plans, 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,” “potential,” “will,” “would,” “could,” “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 Quarterly Report on Form 10-Q 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 both the treatment of patients with geographic atrophy, a severe 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 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 successful collaborative relationship with Novartis Pharma AG, including the ability to achieve remaining potential milestone payments;
- 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;
- the impact of 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 you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, 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 Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

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**PART I—FINANCIAL INFORMATION**

**Item 1. Financial Statements.**

**OPHTHOTECH CORPORATION**  
**Unaudited Balance Sheets**  
(in thousands, except share and per share data)

	September 30, 2014	December 31, 2013
<b>Assets</b>		
Current assets		
Cash and cash equivalents	\$ 94,804	\$ 210,596
Accounts receivable	50,000	—
Available for sale securities	313,978	—
Prepaid expenses and other current assets	2,793	6,804
Deferred tax assets	1,069	—
Total current assets	462,644	217,400
Property and equipment, net	1,289	27
Deferred tax assets, non-current	26,187	—
Security deposits	282	255
Other assets	84	—
Total assets	\$ 490,486	\$ 217,682
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities		
Accrued clinical drug supplies and trial costs	\$ 6,342	\$ 2,485
Accounts payable and accrued expenses	6,706	3,810
Deferred revenue	3,206	—
Income tax payable	7,785	—
Total current liabilities	24,039	6,295
Deferred revenue, long-term	207,219	—
Royalty purchase liability	83,333	41,667
Total liabilities	314,591	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, 33,586,609 and 31,413,208 shares issued and outstanding at September 30, 2014 and December 31, 2013, respectively	34	31
Additional paid-in capital	421,193	352,739
Accumulated deficit	(245,367)	(183,050)
Accumulated other comprehensive income	35	—
Total stockholders' equity	175,895	169,720
Total liabilities and stockholders' equity	\$ 490,486	\$ 217,682

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

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**OPHTHOTECH CORPORATION**  
**Unaudited Statements of Operations**  
(in thousands, except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Revenue	\$ 39,575	\$ —	\$ 39,575	\$ —
<b>Costs and expenses:</b>				
Research and development	17,105	11,101	66,189	17,836
General and administrative	8,812	4,166	22,731	9,145
<b>Total costs and expenses</b>	<b>25,917</b>	<b>15,267</b>	<b>88,920</b>	<b>26,981</b>
<b>Income (loss) from operations</b>	<b>13,658</b>	<b>(15,267)</b>	<b>(49,345)</b>	<b>(26,981)</b>
Interest income (expense)	73	—	189	(1,454)
Gain (loss) on extinguishment of debt	—	105	—	(1,091)
Other loss	—	(970)	—	(1,231)

Income (loss) before income tax provision	13,731	(16,132)	(49,156)	(30,757)
<b>Income tax provision</b>	2,867	—	13,161	—
Net income (loss)	10,864	(16,132)	(62,317)	(30,757)
Add: accretion of preferred stock dividends	—	(2,292)	—	(5,891)
Net income (loss) attributable to common stockholders	\$ 10,864	\$ (18,424)	\$ (62,317)	\$ (36,648)
Net income (loss) attributable to common stockholders per share				
:				
Basic	\$ 0.32	\$ (10.26)	\$ (1.88)	\$ (23.21)
Diluted	\$ 0.31	\$ (10.26)	\$ (1.88)	\$ (23.21)
Weighted average common shares outstanding:				
Basic	33,531	1,795	33,074	1,579
Diluted	34,859	1,795	33,074	1,579

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

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**OPHTHOTECH CORPORATION**  
**Unaudited Statements Comprehensive Income (Loss)**  
**(in thousands)**

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2014</u>	<u>2013</u>	<u>2014</u>	<u>2013</u>
Net income (loss)	\$ 10,864	\$ (16,132)	\$ (62,317)	\$ (30,757)
Other comprehensive income:				
Unrealized gain on available for sale securities, net of taxes	10	—	35	—
Other comprehensive income	10	—	35	—
Comprehensive Income (loss)	<u>\$ 10,874</u>	<u>\$ (16,132)</u>	<u>\$ (62,282)</u>	<u>\$ (30,757)</u>

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

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**OPHTHOTECH CORPORATION**  
**Unaudited Statements of Cash Flows**  
**(in thousands)**

	<u>Nine months ended September 30,</u>	
	<u>2014</u>	<u>2013</u>
<b>Operating Activities</b>		
Net loss	\$ (62,317)	\$ (30,757)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation	80	14
Amortization of debt issuance costs	—	88
Accretion of debt discount	—	87
Amortization of premium and discounts on investment securities	1,338	—
Non-cash change in fair value of warrant liability	—	1,231
Loss on extinguishment of debt	—	1,091
Deferred income taxes	(24,947)	—
Share-based compensation	9,492	1,607
Excess tax benefits from stock-based compensation	(2,335)	—
Changes in operating assets and liabilities:		
Accounts receivable	(50,000)	—
Prepaid expense and other current assets	4,011	(2,052)
Accrued interest receivable	148	—
Security deposits	(28)	(11)
Other assets	(84)	—
Accrued clinical drug supplies and trial costs	3,857	2,464
Accounts payable and accrued expenses	2,896	3,040
Income tax payable	7,785	—
Deferred revenue	210,425	—
Net cash provide by (used in) operating activities	<u>100,321</u>	<u>(23,198)</u>
<b>Investing Activities</b>		
Purchase of marketable securities	(385,403)	—
Maturities of marketable securities	70,000	—
Purchase of property and equipment	(1,342)	(5)
Net cash used in investing activities	<u>(316,745)</u>	<u>(5)</u>
<b>Financing Activities</b>		

Payment of debt issuance costs	—	(43)
Proceeds from stock option/warrant exercises	1,221	—
Proceeds from follow-on public offering, net	55,409	—
Proceeds from initial public offering, net	—	175,555
Excess tax benefits from share-based compensation	2,335	—
Repayment of venture debt facility, net	—	(11,900)
Proceeds from issuance of preferred stock, net	—	49,699
Proceeds from royalty purchase agreement	41,667	41,667
Net cash provided by financing activities	100,632	254,978
Net change in cash and cash equivalents	(115,792)	231,775
<b>Cash and cash equivalents</b>		
Beginning of period	210,596	4,304
End of period	\$ 94,804	\$ 236,079
<b>Supplemental disclosure of cash paid</b>		
Interest	\$ —	\$ 1,524
Income Taxes	\$ 30,112	\$ —
<b>Supplemental disclosures of non-cash information related to investing activities</b>		
Change in unrealized gains in marketable securities, net of tax	\$ 35	\$ —
<b>Supplemental disclosures of cash flow information</b>		
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

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

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**OPHTHOTECH CORPORATION**  
**Notes to Unaudited 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 severe 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.

The Company has devoted substantially all of its efforts to research and development, including clinical trials. The Company has not generated product revenue to date and is subject to a number of risks similar to those of other early stage companies, including dependence on key individuals, the difficulties inherent in the development of commercially usable products, the potential need to obtain additional capital necessary to fund the development of its products, and competition from other companies. As of September 30, 2014, the Company had an accumulated deficit of approximately \$245.4 million. To date, the Company has financed its operations primarily through private placements of its preferred stock, venture debt borrowings, its royalty purchase and sale agreement with Novo A/S (the “Novo Agreement”), its initial public offering (“IPO”), which closed on September 30, 2013, a follow-on public offering, which closed on February 18, 2014, and its license and commercialization agreement with Novartis Pharma AG (the “Novartis Agreement”), which it entered into on May 19, 2014.

**2. Summary of Significant Accounting Policies**

**Basis of Presentation**

The accompanying unaudited financial information as of September 30, 2014 and for the three and nine months ended September 30, 2014 and 2013 has been prepared by the Company pursuant to the rules and regulations of the Securities and Exchange Commission. Certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. generally accepted accounting principles (“GAAP”) have been condensed or omitted pursuant to such rules and regulations. The December 31, 2013 balance sheet was derived from the Company’s audited financial statements. These interim financial statements should be read in conjunction with the notes to the financial statements contained in the Company’s Annual Report on Form 10-K (“Annual Report”) for 2013, as filed with the Securities and Exchange Commission on March 11, 2014.

In the opinion of management, the unaudited financial information as of September 30, 2014 and for the three and nine months ended September 30, 2014 and 2013, reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results or operations and cash flows. The results of operations for the three and nine months ended September 30, 2014 and 2013 are not necessarily indicative of the operating results for the full fiscal year or any future period.

**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.

## Cash and Cash Equivalents

The Company considers all highly liquid investments with an original maturity of three months 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.

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### Available for Sale Securities

The Company considers securities with original maturities of greater than three months to be available for sale securities. Securities under this classification are recorded at fair value and unrealized gains and losses 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

Prior to the third quarter of 2014, the Company had not generated any revenue. In May 2014, the Company received an up-front, non-refundable 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, the Company achieved a \$50.0 million enrollment-based milestone under the Novartis Agreement, for which the Company recognized revenue of approximately \$39.6 million during the three and nine months ended September 30, 2014, and recorded approximately \$10.4 million as deferred revenue relating to research and development activities that the Company expects to perform in the future. 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 and \$1.2 million to research and development activities the Company has performed under the Novartis Agreement.

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 stand-alone 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.

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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.

### **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.

### **Property and Equipment**

Property and equipment, which consists mainly of 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 five to ten years, using the straight-line method.

### **Research and Development**

Research and development expenses consist of costs associated with the development and clinical testing of Fovista, an anti-platelet derived growth factor ("PDGF") aptamer 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 the Company is developing with a focus on treatment of patients with geographic atrophy, a severe 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. Research and development expenses consist of:

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- 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 early 2015, and in late 2014 or early 2015 a Phase 2 clinical trial to evaluate the safety and efficacy of Zimura, in combination with anti-VEGF therapy and, potentially, Fovista, for the treatment of patients with wet AMD who are anti-VEGF resistant and 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.

### **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 the 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.

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:

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	Three months ended September 30,		Nine months ended September 30,	
	2014	2013	2014	2013
Research and development	\$ 1,928	\$ 752	\$ 5,535	\$ 1,052
General and administrative	1,652	395	3,957	555
Total	\$ 3,580	\$ 1,147	\$ 9,492	\$ 1,607

## JOBS Act

As an "emerging growth company" under the Jumpstart Our Business Startups Act of 2012, the Company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. The Company has elected to delay the adoption of such new or revised accounting standards. As a result of this election, the Company's financial statements may not be comparable to the financial statements of other public companies. Commencing January 1, 2015, the Company will no longer qualify for such status and will need to comply with all new or revised accounting standards applicable to other public companies. The Company does not expect this prospective change in status to have a material impact on the Company's 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 No. 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 10Q for the three and six months ended June 30, 2014.

In May 2014, the FASB issued Accounting Standards 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 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 consolidated 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 9, 2013, the Company effected a one-for-5.9 reverse stock split of its common stock. All share and per share data (except par value) related to common stock, options and warrants included in these financial statements and accompanying notes have been adjusted to reflect the reverse stock split for all periods presented.

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

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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 Income (Loss) Per Common Share

Basic and diluted net income (loss) per common share is determined by dividing net income (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:

	Three months ended September 30,		Nine months ended September 30,	
	2014	2013	2014	2013
Basic and diluted net income (loss) per common share calculation:				
Net income (loss)	\$ 10,864	\$ (16,132)	\$ (62,317)	\$ (30,757)
Accretion of preferred stock dividends	—	(2,292)	—	(5,891)
Net income (loss) attributable to common share	\$ 10,864	\$ (18,424)	\$ (62,317)	\$ (36,648)
Weighted average common shares outstanding - basic	33,531	1,795	33,074	1,579
Plus: net effect of dilutive stock options, warrants and unvested restricted stock units	1,328	—	—	—
Weighted average common shares outstanding - dilutive	34,859	1,795	33,074	1,579
Net income (loss) per share of common stock - basic	\$ 0.32	\$ (10.26)	\$ (1.88)	\$ (23.21)
Net income (loss) per share of common stock - diluted	\$ 0.31	\$ (10.26)	\$ (1.88)	\$ (23.21)

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:

	Three months ended September 30,		Nine months ended September 30,	
	2014	2013	2014	2013
Options outstanding	1,503	2,620	2,244	2,620
Warrants	—	101	14	101
Restricted stock units	4	—	17	—
Total	1,507	2,721	2,275	2,721

### 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 three months or less to be cash equivalents. Cash and cash equivalents included cash of \$5.8 million and \$6.8 million at September 30, 2014 and December 31, 2013, respectively. Cash and cash equivalents at September 30, 2014 and December 31, 2013 also included investments of \$89.0 million and \$203.8 million, respectively, in U.S. Treasury securities with original maturities of less than three months and in money market funds that invest in U.S. Treasury Securities.

At September 30, 2014, the Company held available for sale securities with a fair value totaling \$314.0 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 September 30, 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:

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	As of September 30, 2014			
	Cost	Fair Value	Carrying Value	Unrealized Gain (Loss)
U.S. Treasury securities - maturities < 1 year	\$ 313,917	\$ 313,978	\$ 313,978	\$ 61
U.S. Treasury securities - maturities > 1 year	\$ —	\$ —	\$ —	\$ —
Total	\$ 313,917	\$ 313,978	\$ 313,978	\$ 61

## 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 (the “Novartis Agreement”). 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 active pharmaceutical ingredient 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 active pharmaceutical ingredient in Fovista for use in 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 active pharmaceutical ingredient 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 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"); and (3) 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 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

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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.

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 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, for which the Company recognized revenue of approximately \$39.6 million during the three and nine months ended September 30, 2014, and recorded approximately \$10.4 million as deferred revenue relating primarily to research and development activities that the Company expects to perform in the future. 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 and \$1.2 million to research and development activities the Company has performed under the Novartis Agreement.

## 7. Royalty 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 and which provides for the Company to sell, and Novo A/S to purchase, the right, title, and interest in a portion of the revenues from the sale 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 provides 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. If all royalty interests under the Novo Agreement are purchased, Novo A/S will have a right to receive royalties on net sales at a mid-single digit percentage.

In each of May 2013 and January 2014, the Company received cash payments of \$41.7 million, \$83.3 million in the aggregate, for the royalty entitlement related to each of the first and second tranche closings under the Novo Agreement. On November 7, 2014, the Company provided notice to Novo A/S of its achievement of the enrollment-based trigger for the third and final \$41.7 million tranche of financing, which the Company expects to receive within 5 business days of the date of such notice, as per the terms of the Novo Agreement.

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 proceeds from the first and second financing tranches under the Novo Agreement were recorded as a liability on the Company's Balance Sheet as of September 30, 2014, in accordance with ASC Topic 730, *Research and Development*. The Company also expects to increase the amount of such liability on its Balance Sheet in connection with the expected receipt of the third and final \$41.7 million tranche of financing in November 2014. 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

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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 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. 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 September 30, 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 and the fact the Company has reported tax losses since inception.

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 impacted by the Company's ability to carryback losses to previous years in which the Company had taxable income. In connection with the \$41.7 million the Company received from Novo A/S in January 2014, the \$200.0 million the Company received from Novartis in May 2014, a portion of which has been deferred for income tax purposes, the \$50.0 million enrollment-based milestone payment the Company earned in September 2014 under the Novartis Agreement, and the \$41.7 million financing tranche the Company triggered in November 2014 under the Novo Agreement, the Company is currently projecting taxable income in 2014, after taking into account the utilization of its federal net operating losses from prior years. As such, the Company made income tax payments of \$30.1 million in September 2014 and expects to make additional income tax payments during the fourth quarter of 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.

Deferred tax assets relating to employee share-based compensation deductions were reduced to reflect exercises of non-qualified stock option grants and vesting of restricted stock. 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 has unrealized excess tax benefits related to stock based compensation costs of \$2.3 million that it expects to credit to stockholder's equity in future periods.

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.

## **9. 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:

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- 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.
- 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 September 30, 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)
<b>Assets</b>			
Investments in U.S. Treasury money market funds*	\$ 69,005	\$ —	\$ —
Investments in U.S. Treasury securities maturities < three months*	\$ 20,000	\$ —	\$ —
Investments in U.S. Treasury securities maturities < 1 year	\$ 313,978	\$ —	\$ —
Investments in U.S. Treasury securities maturities > 1 year	\$ —	\$ —	\$ —

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)
<b>Assets</b>			
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.

## 10. 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 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

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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 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 September 30, 2014, the Company had approximately 402,000 shares available for grant under the 2013 Plan.

Cash proceeds from, and the aggregate intrinsic value of, stock options exercised during the three and nine months ended September 30, 2014 and 2013, respectively, were as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2014	2013	2014	2013
Cash Proceeds from options exercised	\$ 921	\$ —	\$ 1,221	\$ —
Aggregate intrinsic value of options exercised	\$ 3,785	\$ —	\$ 6,884	\$ —

A summary of the stock options outstanding and exercisable as of September 30, 2014 is as follows:

Range of Exercise Prices	As of September 30, 2014				
	Total Options Outstanding	Options Outstanding		Options Exercisable	
		Weighted Average Remaining Life (Years)	Weighted Average Exercise Price	Number Exercisable	Weighted Average Exercise Price
\$0.12-\$10.03	1,622	7.1	\$ 4.96	1,058	\$ 3.09
\$10.04-\$20.00	569	8.7	\$ 13.37	133	\$ 13.34
\$20.01-\$30.00	185	9.1	\$ 25.75	8	\$ 27.63
\$30.00-\$43.90	1,639	9.4	\$ 33.22	31	\$ 35.10
	<u>4,015</u>			<u>1,230</u>	
Aggregate Intrinsic Value	\$ 81,594			\$ 41,517	

In connection with stock option awards granted to employees, the Company recognized share-based compensation expense of approximately \$3.1 million and \$0.8 million for the three months ended September 30, 2014 and 2013, respectively. In connection with stock option awards granted to employees, the Company recognized share-based compensation expense of approximately \$8.3 million and \$1.1 million for the nine months ended September 30, 2014 and 2013, respectively. As of September 30, 2014, there was approximately \$39.9 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 3.2 years.

In connection with stock option awards granted to consultants, the Company recognized approximately \$0.4 million in share-based compensation expense during the each of the three month periods ended September 30, 2014 and 2013. In connection with stock options awards granted to consultants, the Company recognized approximately \$1.0 million and \$0.5 million, in share-based compensation expense during the nine months ended September 30, 2014 and 2013, respectively. As of September 30, 2014, there was approximately \$4.0 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 3.1 years.

As of September 30, 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 \$0.1 million and

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\$0.2 million in the three and nine month periods ended September 30, 2014, respectively. The Company did not recognize any share-based compensation expense related to restricted stock units during the three and nine month periods ended September 30, 2013. As of September 30, 2014, there was approximately \$1.1 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 2.6 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).

## 11. Property and Equipment

Property and equipment at September 30, 2014 and December 31, 2013 were as follows:

	Useful Life (Years)	September 30, 2014		December 31, 2013	
		\$		\$	
Manufacturing equipment	7-10	\$ 586		\$ —	
Computer and other office equipment	5	292		85	
Furniture and fixtures	7	517		117	
Leasehold improvements	3-5	149		—	
		<u>1,544</u>		<u>202</u>	
Accumulated depreciation and amortization		(255)		(175)	
Property and equipment, net		<u>\$ 1,289</u>		<u>\$ 27</u>	

For the three months ended September 30, 2014 and 2013, depreciation expense was \$36,000 and \$4,000, respectively. For the nine months ended September 30, 2014 and 2013, depreciation expense was \$80,000 and \$14,000, respectively.

## 12. Subsequent Events

On November 7, 2014, the Company provided notice to Novo A/S of its achievement of the enrollment-based trigger for the third and final \$41.7 million tranche of financing, which the Company expects to receive within 5 business days of the date of such notice, as per the terms of the Novo Agreement.

On November 10, 2014, the Company entered into an exclusive research and option agreement with AVEO Pharmaceuticals, Inc. under which the Company has been granted (a) exclusive rights to investigate the potential of tivozanib, a small molecule vascular endothelial growth factor (VEGF) tyrosine kinase inhibitor, for the treatment of non-oncologic conditions of the eye, and (b) an exclusive option for a license to further develop and commercialize tivozanib worldwide (except Asia) for the treatment of non-oncologic conditions of the eye. Under the terms of the agreement, Ophthotech will pay an upfront fee of \$500,000 within 10 days of execution of the agreement.

## Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

*The following discussion of our financial condition and results of operations should be read in conjunction with our financial statements and the notes to those financial statements appearing elsewhere in this Quarterly Report on Form 10-Q and the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the year ended December 31, 2013 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 11, 2014. This discussion contains forward-looking statements that involve significant risks and uncertainties. As a result of many factors, such as those set forth in Part II, Item 1A. (Risk Factors) of this Quarterly Report on Form 10-Q, our actual results may differ materially from those anticipated in these forward-looking statements.*

### 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 severe 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

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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 have also initiated a Phase 2a open label study designed to investigate the potential effect of administration of Fovista in combination with anti-VEGF therapy in reducing the formation of subretinal fibrosis in wet AMD patients, and we are planning to initiate one or more Phase 2 clinical trials in the fourth quarter of 2014, 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.

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 active pharmaceutical ingredient 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 was achieved in September 2014 and paid to us 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 also plan to initiate a Phase 2/3 clinical trial to evaluate the safety and efficacy of Zimura monotherapy in patients with geographic atrophy in early 2015. We are also planning a clinical trial to be initiated in late 2014 or early 2015 investigating Zimura administered in combination with anti-VEGF therapy

and, potentially, Fovista, for the treatment of a subpopulation of wet AMD patients who do not respond adequately to treatment with anti-VEGF monotherapy or for whom anti-VEGF monotherapy fails and who are believed to have complement mediated inflammation.

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 \$62.3 million for the nine months ended September 30, 2014, and \$51.1 million for the year ended December 31, 2013. As of September 30, 2014, we had an accumulated deficit of \$245.4 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, our initial public offering, which we closed in September 2013, our follow-on public offering of

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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 \$83.3 million of royalty funding to date under our royalty purchase and sale agreement with Novo A/S, which we refer to as the Novo Agreement. Further, we expect to receive the third and final \$41.7 million tranche of financing under the Novo Agreement in November 2014. 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 of \$30.1 million during the three months ended September 30, 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, \$41.7 million of which we received in January 2014 and \$41.7 million we expect to receive in November 2014 in connection with the third and final tranche of financing under the Novo Agreement. We expect to make additional income tax payments during the fourth quarter of 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, most of which we expect to enroll in 2014 and 2015. 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 severe 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. 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 September 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 \$39.6 million as revenue during the three and nine months ended September 30, 2014 and recorded the remaining \$10.4 million as deferred revenue, which relates primarily to research and development related activities that we expect to recognize on a proportional performance basis through 2017. 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.

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### **Research and Development Expenses**

Research and development expenses 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
- 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 three and nine months ended September 30, 2014 and 2013:

	Three months ended September 30,		Nine months ended September 30,	
	2014	2013	2014	2013
	(in thousands)			
Fovista	\$ 11,842	\$ 9,219	\$ 52,520	\$ 14,073
Zimura	800	6	1,799	13
Personnel related	2,335	1,104	6,093	2,672
Share-based compensation	1,928	752	5,535	1,052
Other	200	20	242	26
	<u>\$ 17,105</u>	<u>\$ 11,101</u>	<u>\$ 66,189</u>	<u>\$ 17,836</u>

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 planned 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. This is due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

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- the scope, rate of progress and expense of our research and development activities;
- the potential benefits of our product candidates over other therapies;
- 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 29 of this Quarterly Report on Form 10-Q 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 and commercialization activities and as a result of increased headcount, 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 Quarterly Report on Form 10-Q. 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.

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### **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.

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**

Prior to third quarter of 2014, we had not generated any revenue. In May 2014, we received an up-front, non-refundable payment of \$200.0 million upon execution of 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, for which we recognized revenue of approximately \$39.6 million during the three and nine months ended September 30, 2014, and recorded approximately \$10.4 million as deferred revenue relating primarily to research and development activities that we expect to perform in the future. Using the relative selling price method, we allocated revenue of \$38.4 million to the license we delivered to Novartis under the Novartis Agreement and \$1.2 million to research and development activities we have performed under the Novartis Agreement.

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

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pursuant to the license. In validating our best estimate of selling price, we 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 first and second financing tranches under the Novo Agreement have been recorded as a liability on our balance sheet in accordance with ASC Topic 730, *Research and Development*. We also expect to increase the amount of this liability on our balance sheet in connection with the expected receipt of the third and final \$41.7 million tranche of financing in November 2014. 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 the straight-line single

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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, 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 three and nine months ended September 30, 2014 and 2013:

	Three months ended September 30,		Nine months ended September 30,	
	2014	2013	2014	2013
Expected common stock price volatility	77%	82%	83%	82%
Risk-free interest rate	1.84%-2.48%	1.66%-2.69%	1.61%-2.48%	0.89%-2.69%
Expected term of options (years)	6.3	6.5	6.3	6.2
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 September 30, 2014, actual forfeitures have not been material.

Share-based compensation expense for equity grants to employees and non-employees was \$3.6 million for the three months ended September 30, 2014 and \$1.1 million for the three months ended September 30, 2013. Share-based compensation expense for equity grants to employees and non-employees was \$9.5 million for the nine months ended September 30, 2014 and \$1.6 million for the nine months ended September 30, 2013. As of September 30, 2014, we had \$45.0 million of total unrecognized share-based compensation expense, which we expect to recognize over a weighted-average remaining vesting period of approximately 3.2 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 three and nine months ended September 30, 2014 and 2013, we allocated share-based compensation as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2014	2013	2014	2013
Research and development	\$ 1,928	\$ 752	\$ 5,535	\$ 1,052
General and administrative	1,652	395	3,957	555
Total	\$ 3,580	\$ 1,147	\$ 9,492	\$ 1,607

[Table of Contents](#)**Income Taxes**

As of December 31, 2013, we had approximately \$91.3 million of federal net operating loss carry-forwards. We also had federal research and development tax credit carry-forwards of approximately \$3.0 million available to offset future taxable income.

In January 2014, we received \$41.7 million from Novo A/S under the Novo Agreement, 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. On November 7, 2014, we provided notice to Novo A/S of our achievement of the enrollment-based trigger for the third and final \$41.7 million tranche of financing, which we expect to receive within five business days of the date of such notice, as per the terms of the Novo Agreement, and 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, we are projecting taxable income for the 2014 tax year. These projections are based upon estimates, which could change in the future. We made income tax payments of \$30.1 million during the three months ended September 30, 2014. These payments relate to estimated taxable income that resulted from the payments we received and expect to receive from Novo and Novartis in 2014. We expect to make additional income tax payments during the fourth quarter of 2014. In addition, we expect that the valuation allowance on certain of our deferred tax assets will be released, where appropriate. See

Note 8 to our financial statements in Part I-Item 1 of this Quarterly Report on Form 10-Q for further information regarding our expectations with respect to our income tax provision.

## JOBS Act

As an “emerging growth company” under the Jumpstart Our Business Startups Act of 2012, we have been 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. We have elected to delay our adoption of such new or revised accounting standards. As a result of this election, our financial statements may not be comparable to the financial statements of other public companies. Commencing in January 1, 2015, we will no longer qualify for such status and we will need to comply with all new or revised accounting standards applicable to other public companies. We do not expect this prospective change in status to have a material impact on our 2015 financial position or results of operations.

## Results of Operations

### Comparison of Three Month Periods Ended September 30, 2014 and 2013

	Three months ended September 30,		Increase (Decrease)
	2014	2013	
	(in thousands)		
<b>Statement of Operations Data:</b>			
Revenue	\$ 39,575	\$ —	\$ 39,575
Operating Expenses:			
Research and development	17,105	11,101	6,004
General and administrative	8,812	4,166	4,646
Total operating expenses	25,917	15,267	10,650
Income (loss) from operations	13,658	(15,267)	28,925
Interest income (expense)	73	—	73
Gain on extinguishment of debt	—	105	(105)
Other loss	—	(970)	970
Income (loss) before income tax provision	13,731	(16,132)	29,863
Income tax provision	2,867	—	2,867
Net income (loss)	10,864	(16,132)	26,996
Add: accretion of preferred stock dividends	—	(2,292)	2,292
Net income (loss) attributable to common stockholders	\$ 10,864	\$ (18,424)	\$ 29,288

### Revenue

Revenues for the three months ended September 30, 2014 were approximately \$39.6 million. Using the relative selling price method, we allocated \$38.4 million to the license delivered to Novartis under the Novartis Agreement and \$1.2 million to research and development activities we performed under the Novartis Agreement.

We did not recognize any revenue for the three months ended September 30, 2013.

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### Research and Development Expenses

Our research and development expenses were \$17.1 million for the three months ended September 30, 2014, an increase of \$6.0 million compared to \$11.1 million for the three months ended September 30, 2013. The increase was primarily due to 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 three months ended September 30, 2014 were \$8.8 million, an increase of \$4.6 million compared to \$4.2 million for the three months ended September 30, 2013. The increase was primarily due to an increase in costs to support our public company infrastructure, including additional management, 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 three months ended September 30, 2014 was \$0.1 million. Net interest income was de minimis for the three months ended September 30, 2013. Net interest income earned during the three months ended September 30, 2014 was a result of a significant increase in our cash, cash equivalents and marketable securities average balances during the three months ended September 30, 2014 as compared to the three months ended September 30, 2013.

### Other Loss

There was no other loss recorded for the three months ended September 30, 2014 compared to other loss of \$1.0 million for the three months ended September 30, 2013. Amounts recorded as other loss were due to the change in fair value of the preferred stock warrant liability recorded in the third quarter

of 2013. Upon completion of our initial public offering in 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 three months ended September 30, 2014 was \$2.9 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 projected taxable income for 2014. For the three months ended September 30, 2013, we did not record a provision for income taxes due to our significant operating losses.

### **Comparison of Nine Month Periods Ended September 30, 2014 and 2013**

	<u>Nine months ended September 30,</u>		<u>Increase (Decrease)</u>
	<u>2014</u>	<u>2013</u>	
	(in thousands)		
<b>Statement of Operations Data:</b>			
Revenue	\$ 39,575	\$ —	\$ 39,575
Operating Expenses:			
Research and development	66,189	17,836	48,353
General and administrative	22,731	9,145	13,586
Total operating expenses	88,920	26,981	61,939
Loss from operations	(49,345)	(26,981)	(22,364)
Interest income (expense)	189	(1,454)	1,643
Loss on extinguishment of debt	—	(1,091)	1,091
Other loss	—	(1,231)	1,231
Income (loss) before income tax provision	(49,156)	(30,757)	(18,399)
Income tax provision	13,161	—	13,161
Net loss	(62,317)	(30,757)	(31,560)
Add: accretion of preferred stock dividends	—	(5,891)	5,891
Net loss attributable to common stockholders	\$ (62,317)	\$ (36,648)	\$ (25,669)

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#### **Revenue**

Revenues for the nine months ended September 30, 2014 were approximately \$39.6 million. Using the relative selling price method, we allocated \$38.4 million to the license delivered to Novartis under the Novartis Agreement and \$1.2 million to research and development activities we performed under the Novartis Agreement.

We did not recognize any revenue for the nine months ended September 30, 2013.

#### **Research and Development Expenses**

Our research and development expenses were \$66.2 million for the nine months ended September 30, 2014, an increase of \$48.4 million compared to \$17.8 million for the nine months ended September 30, 2013. The increase was primarily due to a milestone payment of \$19.8 million that we made in June 2014 in connection with 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 nine months ended September 30, 2014 were \$22.7 million, an increase of \$13.6 million compared to \$9.1 million for the nine months ended September 30, 2013. The increase was primarily due to an increase in costs to support our public company infrastructure, including additional management, 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 nine months ended September 30, 2014 was \$0.2 million compared to net interest expense of \$1.5 million for the nine months ended September 30, 2013. Net interest income earned during the nine months ended September 30, 2014 was a result of a significant increase in our cash, cash equivalents and marketable securities average balances during the nine months ended September 30, 2014 as compared to the nine months ended September 30, 2013. The amounts recorded in the nine months ended September 30, 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 nine months ended September 30, 2014.

#### **Other Loss**

There was no other loss recorded for the nine months ended September 30, 2014 compared to other loss of \$1.2 million for the nine months ended September 30, 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 in 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 nine months ended September 30, 2014 was \$13.2 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 projected taxable income for 2014. For the nine months ended September 30, 2013, we did not record a provision for income taxes due to our significant operating losses.

## 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. 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 \$83.3 million of this royalty financing in separate tranches in May 2013 and January 2014 and we expect to receive the third and final \$41.7 million tranche of

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financing under the Novo Agreement in November 2014. 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 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 net proceeds of \$55.4 million from the follow-on offering.

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 was achieved in September 2014 and 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 \$30.1 million during the three months ended September 30, 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, \$41.7 million in proceeds from the Novo Agreement in January 2014 and \$41.7 million we expect to receive under the Novo Agreement in November 2014. We expect to make additional income tax payments during the fourth quarter of 2014.

### Cash Flows

As of September 30, 2014, we had cash, cash equivalents and marketable securities totaling \$408.8 million. We also had \$314.6 million in total liabilities as of September 30, 2014, including liabilities of \$293.8 million relating to the Novo Agreement and deferred revenue associated with Novartis Agreement. We primarily invest our cash, cash equivalents and marketable securities 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 nine months ended September, 2014 and 2013:

	<b>Nine months ended September 30,</b>	
	<b>2014</b>	<b>2013</b>
	(in thousands)	
Net cash (used in) provided by:		
Operating Activities	\$ 100,321	\$ (23,198)
Investing Activities	(316,745)	(5)
Financing Activities	100,632	254,978
Net change in cash and cash equivalents	<u>\$ (115,792)</u>	<u>\$ 231,775</u>

#### Cash Flows from Operating Activities

Net cash provided by operating activities of \$100.3 million for the nine months ended September 30, 2014 relates primarily to the upfront payment of \$200.0 million upon the execution of the Novartis Agreement, offset by (i) estimated tax payments made in September 2014 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 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 that consists of three separate clinical trials. Two of these trials were initiated in the third quarter of 2013 and the third trial was initiated in the first quarter of 2014. We expect significant cash outflows to continue for the foreseeable future, particularly as our patient enrollment increases in our Phase 3 clinical program and as we continue the development of and seek marketing approval for Fovista, Zimura and, possibly, other product candidates we may develop.

#### Cash Flows from Investing Activities

Net cash used in investing activities for the nine months ended September 30, 2014 was \$316.7 million and relates primarily to the purchase of marketable securities totaling \$385.4 million offset by market security maturities of \$70.0 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 nine months ended September 30, 2013.

### ***Cash Flows from Financing Activities***

Net cash provided by financing activities was \$100.6 million for the nine months ended September 30, 2014 and \$255.0 million for the nine months ended September 30, 2013. Net cash provided by financing activities for the nine months ended September 30, 2014 consisted primarily of proceeds of \$55.4 million from our follow-on public offering in February 2014, and proceeds of \$41.7 million from the Novo Agreement in January 2014. Net cash provided by financing activities for the nine months ended September 30, 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, most of which we expect to enroll in 2014 and 2015. 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 severe 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 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. Also, if we obtain marketing approval for Zimura or any other product candidate that we develop, we 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 (Eyetechnology), 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 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 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 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, quality control and scientific personnel; and

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

As of September 30, 2014, we had cash, cash equivalents, and marketable securities of \$408.8 million, which includes the \$200.0 million upfront payment that we received upon the execution of the Novartis Agreement. In September 2014, we achieved a \$50.0 million enrollment-based milestone under the Novartis Agreement, which was received in October 2014. On November 7, 2014, we provided notice to Novo A/S of our achievement of the enrollment-based trigger for the third and final \$41.7 million tranche of financing, which we expect to receive within five business days of the date of such notice, as per the terms of the Novo Agreement. As of September 30, 2014, we had \$314.6 million in total liabilities, including liabilities of \$293.8 million relating to the Novo Agreement and deferred revenue associated with the Novartis Agreement. We expect that the amount of our long-term liabilities will increase in connection with the expected receipt of the third and final tranche of financing under the Novo Agreement in November 2014.

We believe that our cash, cash equivalents and marketable securities, together with the receipt of the third and final \$41.7 million tranche of financing under the Novo Agreement and 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 or 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.

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 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, costs and results of our planned Phase 2/3 clinical trial evaluating Zimura for the treatment of geographic atrophy and whether and to what extent additional clinical trials may be required by regulatory authorities for us to seek marketing approval in this indication and our Phase 2 clinical trial evaluating 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;
- 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;
- 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;

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

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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 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.

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 active pharmaceutical ingredient in Fovista. The agreement has an initial five

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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 active pharmaceutical ingredient 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.

### Royalty Financing Agreement with Novo A/S

In May 2013, we entered into the Novo Agreement, pursuant to which we had the ability to obtain royalty 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 low to 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 first and second tranches of the royalty financing, in which Novo A/S purchased two low single-digit royalty interests and paid us \$83.3 million in the aggregate, closed in May 2013 and January 2014. On November 7, 2014, we provided notice to Novo A/S of our achievement of the enrollment-based trigger for the third and final \$41.7 million tranche of financing, which we expect to receive within five business days of the date of such notice, as per the terms of the Novo Agreement. In connection with the closing of the third and final tranche, Novo A/S will purchase a third low single-digit royalty interest and will have a right to receive royalties on worldwide sales of Fovista at a mid-single-digit percentage.

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. The Novo Agreement provides that we will use 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, to the extent applicable, other specified products we may develop pursuant to the terms of the Novo Agreement, and for general corporate expenses. We intend to use the proceeds from the second tranche of financing that we received in January 2014 and the proceeds from the third tranche of financing that we expect to receive in November 2014 to support clinical development and regulatory activities for Fovista.

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.

### Contractual Obligations and Commitments

The following table summarizes our contractual obligations as of September 30, 2014:

	Total	Less than 1 year	Payments Due by Period		
			1-3 years	3-5 years	More than 5 years
	(\$ in thousands)				
Operating Leases (1)	\$ 5,719	\$ 1,185	\$ 2,155	\$ 2,038	\$ 341
Purchase Obligations (2)	5,488	5,488	—	—	—
Total (3)	\$ 11,207	\$ 6,673	\$ 2,155	\$ 2,038	\$ 341

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- (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 \$83.3 million as of September 30, 2014 (which liability we expect will increase in connection with the expected receipt of the third and final \$41.7 million tranche of financing in November 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. Our agreement with Nektar also provides that we pay double-digit percentage of other specified amounts, such as milestone payments, we receive in connection with any such commercialization agreement, including the Novartis Agreement, subject to agreed minimum and maximum amounts
- Under the Novo Agreement, with respect to Fovista, following the expected closing of the third and final tranche of financing in November 2014, we will be obligated to pay Novo A/S a mid-single-digit percentage royalty based on worldwide sales of Fovista. See “—Royalty Financing” 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 active pharmaceutical ingredient 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.

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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 3. Quantitative and Qualitative Disclosures About Market Risk.**

We are exposed to market risk related to changes in interest rates. We had cash, cash equivalents and marketable securities of \$408.8 million as of September 30, 2014, consisting of cash, money market funds that invest in U.S. Treasury securities, and direct investment in U.S. Treasury securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because a significant portion of our investments are in short-term securities. Our available for sale securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

We contract with CROs and contract manufacturers globally. We may be subject to fluctuations in foreign currency rates in connection with certain of these agreements. Transactions denominated in currencies other than the U.S. dollar are recorded based on exchange rates at the time such transactions arise. As of September 30, 2014, substantially all of our total liabilities were denominated in the U.S. dollar.

**Item 4. 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 September 30, 2014. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or 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 SEC’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. Based on the evaluation of our disclosure controls and procedures as of September 30, 2014, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

### Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(d) and 15d-15(d) under the Exchange Act) occurred during the three months ended September 30, 2014 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

## PART II—OTHER INFORMATION

### Item 1. Legal Proceedings.

We are not currently subject to any material legal proceedings.

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### Item 1A. Risk Factors.

*The following risk factors and other information included in this Quarterly Report on Form 10-Q should be carefully considered. The risks and uncertainties described below are not the only ones 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 Quarterly Report on Form 10-Q 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

***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 \$62.3 million for the nine months ended September 30, 2014 and \$30.8 million for the nine months ended September 30, 2013. As of September 30, 2014, we had a deficit accumulated of \$245.4 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 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. We received net proceeds from our 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 our follow-on public offering of \$55.4 million, after deducting underwriting discounts and commissions and other offering expenses payable by us. We have received \$83.3 million of royalty funding to date under our royalty purchase and sale agreement with Novo A/S, which we refer to as the Novo Agreement. Further, we expect to receive the third and final \$41.7 million tranche of financing under the Novo Agreement in November 2014. In May 2014, we received \$200.0 million from Novartis, which constituted the upfront payment under the Novartis Agreement. 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. In September 2014, we achieved a \$50.0 million enrollment-based milestone under the Novartis Agreement, which was received in October 2014. We made income tax payments of \$30.1 million during the three months ended September 30, 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, \$41.7 million in proceeds from the Novo Agreement in January 2014 and \$41.7 million we expect to receive under the Novo Agreement in November 2014. We expect to make additional income tax payments during the fourth quarter of 2014.

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, most of which we expect to enroll in 2014 and 2015. 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 severe 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 (Eyeteq), 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. See “Business—Acquisition and License Agreements” in our Annual Report on

Our expenses also will increase if and as we:

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- 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 for us to seek marketing approval of 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 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 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.

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;
- if initiated, 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' 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

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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.

***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 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, most of which we expect to enroll in 2014 and 2015. 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 severe 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 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 September 30, 2014, we had cash, cash equivalents, and marketable securities of \$408.8 million, which includes the \$200.0 million upfront payment that we received upon the execution of the Novartis Agreement. In September 2014, we achieved a \$50.0 million enrollment-based milestone payment under the Novartis Agreement, which was received in October 2014. On November 7, 2014, we provided notice to Novo A/S of our achievement of the enrollment-based trigger for the third and final \$41.7 million tranche of financing, which we expect to receive within five business days of the date of such notice, as per the terms of the Novo Agreement. As of September 30, 2014, we had \$314.6 million in total liabilities, including liabilities of \$293.8 million relating to the Novo Agreement and deferred revenue associated with the Novartis Agreement. We expect that the amount of our long-term liabilities will increase in connection with the expected receipt of the third and final tranche of financing under the Novo Agreement in November 2014.

We believe that our cash, cash equivalents and marketable securities, together with the receipt of the third and final \$41.7 million tranche of financing under the Novo Agreement and the potential remaining enrollment-based milestone payments

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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. We expect to obtain initial, top-line data from our Phase 3 clinical program for Fovista in 2016. 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 to 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. 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 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 our planned Phase 2/3 clinical trial evaluating Zimura for the treatment of geographic atrophy and whether and to what extent additional clinical trials may be required by regulatory authorities for us to seek marketing approval in this indication and our Phase 2 clinical trial evaluating 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;
- 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 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 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

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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 was paid to us 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 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 cash needs through a combination of equity offerings, debt financings, potential milestone payments under collaborations, strategic alliances and marketing, distribution or licensing arrangements. 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



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### 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 severe 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 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 no 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 from our third Phase 3 clinical trial become available, we may experience further delays in our application process. 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. Adverse safety events or negative or inconclusive efficacy results in any of these trials may impact the progress of our Phase 3 clinical program. In addition, adverse results from any of these additional planned clinical trials would be disclosed in and could negatively impact our applications for marketing approval for Fovista administered in combination with anti-VEGF drugs for the treatment of wet AMD. 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

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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 only had preliminary discussions with regulatory authorities regarding our trial designs. As we continue these discussions, our plans may change significantly based on feedback 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;
- 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.

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***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 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 complete preclinical development and then 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 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 no clinical efficacy data on the effects of Fovista when administered in combination with 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 due, in part, to the fact that we have no clinical safety data on patient exposure to Fovista administered in combination with any anti-VEGF drug for longer than 24 weeks and that we have no clinical safety data on the effects of Fovista when administered in combination with Avastin or Eylea.

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

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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 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 two of the most widely used anti-VEGF drugs 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.

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 have submitted the protocols for our Phase 3 clinical trials to the FDA 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. To date, we have obtained approval in many of these countries and have begun dosing patients in certain of those countries. 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 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.

We may not receive clearance from regulatory authorities in jurisdictions outside the United States to initiate our Phase 3 clinical program in those jurisdictions on a timely basis. 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 the planned primary endpoint for each of the Phase 3 clinical trials for Fovista, mean change from baseline in best corrected visual acuity, was acceptable. In addition, the CHMP confirmed that carcinogenicity studies are not needed for our Phase 3 clinical program. The CHMP also 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

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Fovista administered in combination with Avastin or Eylea prior to initiating our corresponding Phase 3 clinical trial. 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 broad label specifying Fovista for use in combination with any anti-VEGF drug. The CHMP further advised us that there would be a requirement for additional data to bridge the results from our Phase 3 clinical trials evaluating Fovista administered in combination with Lucentis as compared to Lucentis monotherapy to the less frequent dosing regimens of Lucentis and Eylea approved in the European Union.

In the first quarter of 2014, we received written confirmation from the CHMP on these issues. The CHMP is in agreement with our plan to use the dosing schedule approved for Eylea in the European Union as the dosing schedule in our Phase 3 clinical trial for Fovista administered in combination with Eylea so that no bridging study will be needed for this combination. The CHMP has also agreed with our plan for monthly dosing in the first year in both of our Phase 3 clinical trials evaluating Fovista administered in combination with Lucentis and, for one of these trials, to slightly modify the dosing regimen in the second year so that it is consistent with the dosing schedule approved for Lucentis in the European Union. The dosing schedule for the second year in the other trial evaluating Fovista administered with Lucentis remains unchanged. Accordingly, no bridging study will be needed and our anticipated timing and overall expense of our Phase 3 clinical plan, including our plan to have initial, top-line data from our Phase 3 clinical program for Fovista available in 2016, remains unchanged.

Although our plans reflect our current expectations regarding the endpoints, duration and number of patients to be included in our planned Phase 2/3 clinical trial evaluating Zimura for the treatment of geographic atrophy, we have only had preliminary discussions with regulatory authorities regarding our trial design. As we continue these discussions, our plans may change significantly based on feedback from such regulatory authorities. We expect that we will be required by regulatory authorities to conduct 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.

***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;

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- 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;
- 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.

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***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. Although Fovista administered in combination with Lucentis was generally well tolerated in our Phase 1 clinical trial and our Phase 2b clinical trial, we have no clinical safety data on patient exposure to Fovista administered in combination with Lucentis for longer than 24 weeks, and we have no clinical safety data on the effects of Fovista when administered in combination with Avastin or Eylea. 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. Our Phase 3 clinical program for Fovista involves the administration of Fovista in combination with anti-VEGF drugs, and the safety results of our trials are dependent, in part, on the safety and tolerability of the anti-VEGF drug administered in combination with Fovista. Avastin is not approved for the treatment of wet AMD, and according to third-party clinical trials, may be associated with a greater risk of serious adverse events or undesirable side effects as compared to Lucentis. Furthermore, we have very limited data regarding the safety and efficacy of Zimura for the treatment of geographic atrophy. In addition, we have no preclinical or clinical data on the effects of Zimura when administered in combination with Fovista and/or an anti-VEGF drug.

***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 on 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;
- 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

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- 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.

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 optical coherence tomography, or 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 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, 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

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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 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 half of 2015, Allergan, Inc., Ohr Pharmaceutical, Inc., Xcovery Vision LLC, Santen, Neurotech Pharmaceuticals, Inc., Avalanche Biotechnologies, Inc., Somalogic, Inc. and others. 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 corticosteroids, 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 Macular.

See "Business—Competition" in our Annual Report on Form 10-K for 2013, filed with the SEC on March 11, 2014, for more information regarding potential competitive products.

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 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 active pharmaceutical ingredients, or API, of Fovista or Zimura at commercial scale. We currently rely on a single third-party manufacturer 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

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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 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 experience 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 focused, 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

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- 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 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 successful 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 meet 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

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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. 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 in early stage research and drug discovery, the future growth of our business will depend in significant part 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.

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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 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 focused, 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, product and product candidate priorities or available funding;
- 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 successful 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 result 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 a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing 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 a 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 on 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 on third parties, such as CROs, clinical data management organizations, medical institutions 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 on 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.

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 on 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 on and expect to continue to rely on 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.

We currently rely exclusively on 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

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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 on 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 on 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 an agreed price. 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.

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

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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 fail to use commercially reasonable efforts to achieve the first commercial sale of Fovista in the United States or one of a specified group of other countries by December 31, 2017, which date Nektar and we may agree in good faith to extend in specified circumstances, 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.

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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 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.

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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.

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.

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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 active pharmaceutical ingredient 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 active pharmaceutical ingredient 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 active pharmaceutical ingredient 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 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.

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***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.

***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.

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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 on 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.

***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 on 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 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 on 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.

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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 on 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.

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.

***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

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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.

***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.

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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.

***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 Health Care Reform Law 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.

***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 PPACA. Among the provisions of PPACA 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 PPACA was enacted. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. 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 PPACA 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.

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***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**

***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 area of clinical development. Between January 1, 2013 and September 30, 2014, we hired more than half of our 59 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 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 September 30, 2014, our executive officers, directors and principal stockholders and their affiliates, in the aggregate, beneficially owned shares representing approximately 34% 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 September 30, 2014, we had outstanding 33,586,609 shares of common stock. Of these shares, approximately 11,777,178 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 11,305,258 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. In October 2013 and January 2014, we filed registration statements registering all shares of common stock that we may issue under our equity compensation plans. As of September 30, 2014, we had outstanding stock options to purchase an aggregate of approximately 4,015,000 shares of our common stock, of which options to purchase approximately 1,230,000

shares were vested. 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;

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- 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;
- 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;

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- 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.

***Commencing January 1, 2015, we will no longer be an “emerging growth company,” and the reduced disclosure requirements applicable to emerging growth companies will no longer apply to us.***

We are currently an “emerging growth company,” as defined in the Jumpstart Our Business Startups Act of 2012. Because as of June 30, 2014, the market value of our common stock that was held by non-affiliates exceeded \$700 million, we will no longer qualify for such status commencing January 1, 2015. As a large-accelerated filer, we will be subject to certain disclosure requirements that are applicable to other public companies that have not been applicable to us as an emerging growth company. These requirements include:

- compliance with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- compliance with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements;
- full disclosure obligations regarding executive compensation; and
- compliance with the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

In addition, we will no longer be able to take advantage of transition periods for complying with new or revised accounting standards that are available to emerging growth companies.

***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, and particularly commencing January 1, 2015 when we will no longer be an “emerging growth company,” we do 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.

Commencing January 1, 2015, we will no longer be an emerging growth company and as such we will not be able to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are emerging growth companies as has been the case to date.

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Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, commencing January 1, 2015, we will be required to furnish with our periodic Exchange Act reports a report by our management on our internal control over financial reporting. Commencing January 1, 2015, because we will no longer be an emerging growth company, we will be 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 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, 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.

***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.

## **Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.**

### **Recent Sales of Unregistered Securities**

On September 30, 2014, we issued to an employee an option to purchase 200,000 shares of our common stock at an exercise price of \$38.93 per share. This option was an inducement grant issued outside our existing equity compensation plan in accordance with NASDAQ listing rule 5635(c)(4). We intend to file a registration statement on a Form S-8 to register the shares of common stock underlying this option prior to the time at which this option becomes exercisable.

### Purchase of Equity Securities

We did not purchase any of our registered equity securities during the period covered by this Quarterly Report on Form 10-Q.

### Use of Proceeds from Registered Securities

On September 30, 2013, we closed our initial public offering of 8,740,000 shares of our common stock, including 1,140,000 shares of our common stock pursuant to the exercise by the underwriters of an over-allotment option, at a public offering price of \$22.00 per share for an aggregate offering price of approximately \$192.3 million. The offer and sale of all of the shares in our initial public offering were registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-190643), which was declared effective by the SEC on September 24, 2013.

We received aggregate net proceeds from our initial public offering of \$175.6 million, after deducting underwriting discounts and commissions and other offering expenses payable by us.

As of September 30, 2014, we have used approximately \$18.4 million of the net proceeds from initial public offering as follows:

- approximately \$11.9 million to fund certain costs of our Phase 3 clinical program for Fovista administered in combination with anti-VEGF drugs for the treatment of wet AMD, which costs consists of external research and development expenses and clinical development related employee expenses; and
- approximately \$6.5 million for working capital and other general corporate purposes.

We have not used any of the net proceeds from our initial public offering to make payments, directly or indirectly, to any director or officer of ours, or any of their associates, to any person owning 10% or more of our common stock or to any affiliate of ours. We have invested the remaining net proceeds from initial public offering in a variety of capital preservation investments, including short-term, investment grade, interest bearing instruments and U.S. government securities. There has been no material change in our planned use of the net proceeds from our initial public offering as described in our final prospectus filed with the SEC pursuant to Rule 424(b) under the Securities Act.

### Item 5. Other Information.

None.

### Item 6. Exhibits.

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index, which Exhibit Index is incorporated herein by reference.

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### SIGNATURES

Pursuant to the requirements 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.

#### OPHTHOTECH CORPORATION

Date: November 12, 2014

By: /s/ Michael G. Atieh  
Michael G. Atieh  
Chief Financial and Business Officer  
(Principal Financial and Accounting Officer)

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### EXHIBIT INDEX

<u>Exhibit Number</u>	<u>Description of Exhibit</u>
10.1	Offer of Employment between the Registrant and Michael G. Atieh dated September 20, 2014
10.2	Nonstatutory Stock Option Agreement between the Registrant and Michael G. Atieh, dated September 30, 2014
31.1	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002



31.2	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	XBRL Instance Document*
101.SCH	XBRL Taxonomy Extension Schema Document*
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document*
101.LAB	XBRL Taxonomy Extension Label Linkbase Database*
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document*
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document*

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\* Submitted electronically herewith.

Attached as Exhibit 101 to this report are the following formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheet at December 31, 2013 and September 30, 2014 (unaudited), (ii) Statement of Operations (unaudited) for the three month period and nine month period ended September 30, 2014 and 2013, (iii) Statement of Cash Flows (unaudited) for the nine month period ended September 30, 2014 and 2013 and (iv) Notes to Financial Statements (unaudited).

In accordance with Rule 406T of Regulation S-T, the XBRL related information in Exhibit 101 to this Quarterly Report on Form 10-Q is deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act, is deemed not filed for purposes of Section 18 of the Exchange Act, and otherwise is not subject to liability under these sections.

  
 One Penn Plaza, Suite 19<sup>th</sup> Floor  
 New York, NY 10119  
 (212) 845-8200

September 20, 2014

Mr. Michael G. Atieh

Dear Mike:

It is my pleasure to extend to you this offer of employment with Ophthotech Corporation (the "Company"). On behalf of the Company, I set forth below the terms of your employment:

1. **Employment.** You will be employed to serve on a full-time basis as the Company's Executive Vice President, Chief Financial & Business Officer & Treasurer, effective September 30, 2014 (the "Start Date"). As the Company's Executive Vice President, Chief Financial & Business Officer & Treasurer you will report to the Company's Chief Executive Officer and have the duties and responsibilities that are consistent with your position and such other duties as may from time to time be assigned to you by the Company. The Company reserves the right to change your title and responsibilities at any time, with or without notice. You shall perform and discharge faithfully and diligently your duties and responsibilities hereunder. You agree to devote your full business time, efforts, skill, knowledge, attention and energies to the advancement of the Company's business and interests and to the performance of your duties and responsibilities as an employee of the Company. You agree to abide by the rules, regulations, instructions, personnel practices and policies of the Company and any changes therein that may be adopted from time to time by the Company. Notwithstanding the foregoing, you may continue to serve as a member of the board of directors of the two companies for which you currently serve, may serve on civic, charitable, educational, religious, public interest or public service boards, and may manage your personal and family investments, in each case, to the extent such activities, whether individually or in the aggregate, do not materially interfere or conflict with the performance of your duties and responsibilities for the Company.
2. **Base Salary.** Your base salary will be at the minimum rate of \$18,750 per semi-monthly pay period (which if annualized equals \$450,000), less all applicable taxes and withholdings, to be paid in installments in accordance with the Company's regular payroll practices. Such base salary may be increased, but not decreased, from time to time in accordance with normal business practices and in the sole discretion of the Company.

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3. **Discretionary Bonus.** Following the end of each calendar year and subject to the approval of the Company's Board of Directors (the "Board"), you will be eligible for a performance bonus of up to 50% of your annualized base salary (the "Target Bonus"), based on your personal performance and the Company's performance during the applicable calendar year, as determined by the Company in its sole discretion. In any event, except as provided in Section 6 below, you must be an active employee of the Company on the date the bonus is distributed in order to be eligible for and to earn any bonus award, as it also serves as an incentive to remain employed by the Company. You will be eligible for a prorated discretionary bonus for 2014. Payouts for annual bonuses typically occur in January of the following year.
4. **Equity.** In connection with the commencement of your employment with the Company, you will be eligible to receive an option to purchase 200,000 shares of the Company's common stock (the "Option"), subject to the approval by the Board (acting in its sole discretion) of such option grant. This option grant is also contingent upon your execution of the stock option agreement covering the Option. If the Board approves the grant, the Option would be issued on the Start Date with an exercise price equal to the fair market value of the Company's common stock (as determined by the Board) as of the date of grant and would vest over a four-year period, with 25% of the shares vesting on the first anniversary of the Start Date and the remainder of the shares vesting in equal monthly amounts thereafter until the fourth anniversary of the Start Date, pursuant to the terms of the stock option agreement and subject to your continued employment with the Company. You will be eligible for a prorated annual performance-based option grant in January of 2015. Provided that the Option is assumed or an award substantially similar to the Option is substituted by the Company or its successor in connection with a Change in Control Event, if your employment with the Company, or its successor, is terminated by the Company without Cause (as defined in Section 6 hereof) or by you for Good Reason (as defined in Section 6 hereof) within the one (1) year period following a Change in Control Event (as defined in your option agreement), then, 100% of the portion of the Option that is not then-vested, and which has not been exercised, cancelled or forfeited, shall become vested and exercisable in full as of the date of such termination.
5. **Benefits.** You may participate in any and all benefit programs that the Company establishes and makes generally available to its employees from time to time, provided that you are eligible under (and subject to all provisions of) the plan documents that govern those programs. Benefits are subject to change at any time in the Company's sole discretion.
6. **Severance.** If your employment is terminated at any time by the Company without Cause or by you for Good Reason, the Company will (i) pay you in a lump sum on the Payment Date (as herein defined) (a) an amount equal to twelve (12) months of your then-current base salary, less standard employment-related withholdings and deductions and (b) an amount equal to your Target Bonus, less standard employment-related withholdings and deductions, and (ii) provided you elect to continue your and your eligible dependents' participation in the Company's medical and dental benefit plans pursuant to the Consolidated Omnibus Budget Reconciliation Act of 1986 ("COBRA"), reimburse you for the monthly premium to continue such coverage for the lesser of the

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twelve (12) full calendar months immediately following the month in which the termination of your employment occurs and the end of the calendar month in which you become eligible to receive group health plan coverage under another employee benefit plan. Notwithstanding the foregoing, if the reimbursement of monthly premiums would otherwise violate the nondiscrimination rules or cause the reimbursement of claims to be taxable under the Patient Protection and Affordable Care Act of 2010, together with the Health Care and Education Reconciliation Act of 2010 (collectively,

the "Healthcare Reform Act") or Section 105(h) of the Internal Revenue Code of 1986, as amended, these payments shall be treated as taxable payments to you and you shall be subject to imputed income tax treatment to the extent necessary to eliminate any discriminatory treatment or taxation under the Act or Section 105(h).

Notwithstanding the foregoing, the Company shall not be obligated to pay you the severance payments provided for herein unless you have timely executed (and not revoked) a separation agreement in a form to be provided by the Company. Such separation agreement must be executed and become binding and enforceable within sixty (60) calendar days after the effective date of your termination of employment (such 60th day, the "Payment Date"); provided however, that if the 60th day following the date of termination occurs in the next calendar year following the date of termination, then the Payment Date shall be no earlier than January 1 of such following calendar year.

For purposes hereof, "Cause" shall mean that: (i) you failed to attempt in good faith, refused or willfully neglected to perform and discharge your material duties and responsibilities; (ii) you have been convicted of, or pled nolo contendere to, a felony or other crime involving fraud or moral turpitude; (iii) you breached your fiduciary duty of loyalty to the Company, or acted fraudulently or with material dishonesty in discharging your duties to the Company; (iv) you undertook an intentional act or omission of misconduct that materially harmed or was reasonably likely to materially harm the business, interests, or reputation of the Company; (v) you materially breached any material provision of this letter or any other agreement with the Company; or (vi) you materially breached any material provision of any Company code of conduct or ethics policy. Notwithstanding the foregoing, "Cause" shall not be deemed to have occurred unless: (A) the Company provides you with written notice that it intends to terminate your employment hereunder for one of the grounds set forth in subsections (i), (v) or (vi) within sixty (60) days of such reason(s) occurring, (B) if such ground is capable of being cured, you have failed to cure such ground within a period of thirty (30) days from the date of such written notice, and (C) the Company terminates your employment within six (6) months from the date that Cause first occurs.

For purposes hereof, "Good Reason" shall mean, without your written consent: (i) any change in your position or reporting relationship with the Company that diminishes in any material respect your authority, duties or responsibilities; (ii) any material reduction in your base compensation; (iii) a material change in the primary geographic location at which services are to be performed by you (unless the new location is closer to your primary residence than the prior location); or (iv) a material breach of any provision hereof by the Company or any successor or assign. Notwithstanding the foregoing, "Good Reason" shall not be deemed to have occurred unless: (A) you provide the

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Company with written notice that you intend to terminate your employment hereunder for one of the grounds set forth in subsections (i), (ii), (iii) or (iv) of the immediately preceding sentence within sixty (60) days of such reason(s) occurring, (B) if such ground is capable of being cured, the Company has failed to cure such ground within a period of thirty (30) days from the date of such written notice, and (C) you terminate your employment within six (6) months from the date that Good Reason first occurs. For purposes of clarification, the above-listed conditions shall apply separately to each occurrence of Good Reason and failure to adhere to such conditions in the event of Good Reason shall not disqualify you from asserting Good Reason for any subsequent occurrence of Good Reason.

7. Vacation. You will be eligible for a maximum of four (4) weeks of paid vacation per calendar year to be taken at such times as may be approved in advance by the Company. Vacation days for which you are eligible shall accrue pro rata on a monthly basis during the period that you are employed during each calendar year.
8. Commuting Expenses. You will receive a semi-monthly payment of \$4,000, less applicable taxes, by the Company to assist you in paying for reasonable commuting expenses associated with your business related visits to New York City. The amount of such commuting expenses payable by the Company will be reviewed from time to time to ensure reasonableness given the travel required for the role. In general, we expect that you will take two one-week round-trips per month to the New York/New Jersey area as part of your fulfillment of your duties described herein. In the event that your employment hereunder terminates because of a determination by you that such commuting arrangement is not workable, or a determination by the Company that you are not substantially meeting the two one-week trip per month standard, the provisions of Section 6 shall not apply.
9. Invention, Non-Disclosure, Non-Competition and Non-Solicitation Agreement. As a condition of employment, you will be required to execute the attached Invention, Non-Disclosure, Non-Competition and Non-Solicitation Agreement.
10. No Conflict. You represent that you are not bound by any employment contract, restrictive covenant or other restriction preventing you from entering into employment with or carrying out your responsibilities for the Company, or which is in any way inconsistent with the terms of this offer letter.
11. Proof of Legal Right to Work. You agree to provide to the Company, within three (3) days of your date of hire, documentation proving your eligibility to work in the United States, as required by the Immigration Reform and Control Act of 1986. You may need a work visa in order to be eligible to work in the United States. If that is the case, your employment with the Company will be conditioned upon your obtaining a work visa in a timely manner as determined by the Company.
12. At-Will Employment. This letter shall not be construed as an agreement, either express or implied, to employ you for any stated term, and shall in no way alter the Company's policy of employment at-will, under which both the Company and you remain free to end

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the employment relationship for any reason, at any time, with or without cause or notice. Although your job duties, title, compensation and benefits, as well as the Company's personnel policies and procedures, may change from time to time, the "at-will" nature of your employment may only be changed by a written agreement signed by you and the Company's Chief Executive Officer that expressly states the intention to modify the at-will nature of your employment. Similarly, nothing in this letter shall be construed as an agreement, either express or implied, to pay you any compensation or grant you any benefit beyond the end of your employment with the Company. This letter supersedes all prior understandings, whether written or oral, relating to the terms of your employment.

13. Successors and Assigns. The terms of this letter shall be binding upon and inure to the benefit of you and the Company and their respective successors and assigns, including any corporation with which, or into which, the Company may be merged or which may succeed to the Company's assets or business; provided, however, that your obligations are personal and may not be assigned by you. You expressly consent to be bound by the

provisions hereof for the benefit of the Company or any subsidiary or affiliate thereof to whose employ you may be transferred without the necessity that this letter be re-signed at the time of such transfer.

14. Governing Law. This letter shall be governed by and construed in accordance with the laws of the State of New York (without reference to the conflicts of laws provisions thereof). Any action, suit, or other legal proceeding which is commenced to resolve any matter arising under or relating to any provision of this letter shall be commenced only in a court of the State of New York (or, if appropriate, a federal court located within New York), and the Company and you each consents to the jurisdiction of such a court. The Company and you each hereby irrevocably waive any right to a trial by jury in any action, suit or other legal proceeding arising under or relating to any provision hereof.
15. Code Section 409A. The intent of the parties is that payments and benefits under this letter comply with, or be exempt from, Internal Revenue Code Section 409A and the regulations and guidance promulgated thereunder (collectively "Code Section 409A"). Accordingly, if any provision of this letter is ambiguous, such that one interpretation would subject a payment or benefit to the excise tax imposed by Code Section 409A and an alternative interpretation would not so subject the payment or benefit, the parties intend the interpretation that would not so subject the payment or benefit to apply. With regard to any provision herein, including, without limitation, Section 8 hereof, that provides for reimbursement of costs and expenses or in-kind benefits, except as permitted by Code Section 409A, (i) the right to reimbursement or in-kind benefits shall not be subject to liquidation or exchange for another benefit, (ii) the amount of expenses eligible for reimbursement, or in-kind benefits, provided during any taxable year shall not affect the expenses eligible for reimbursement, or in-kind benefits to be provided, in any other taxable year, provided that this clause (ii) shall not be violated with regard to expenses reimbursed under any arrangement covered by Internal Revenue Code Section 105(a) solely because such expenses are subject to a limit related to the period the arrangement is in effect, and (iii) such payments shall be made on or before the last day of your taxable year following the taxable year in which the expense occurred, provided that any tax gross-ups may be reimbursed by the end of the calendar year following the calendar

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year in which such taxes are remitted to the taxing authorities. For purposes of Code Section 409A, each payment hereunder shall be treated as a separate payment and your right to receive any installment payments pursuant to this Agreement shall be treated as a right to receive a series of separate and distinct payments. In no event may you, directly or indirectly, designate the calendar year of any payment to be made under this letter that is considered nonqualified deferred compensation. References to the "termination" of your employment as used herein shall mean a "separation from service" within the meaning of Code Section 409A. In the event at the time of any separation from service you are a "specified employee" within the meaning of Code Section 409A, any deferred compensation subject to Code Section 409A payable as a result of such termination shall not be paid prior to the earlier of six (6) months after such termination and your death and shall be paid immediately thereafter.

If this letter correctly sets forth the terms under which you will be employed by the Company, please sign the enclosed duplicate of this letter in the space provided below and return it to me, along with a signed copy of the Invention, Non-Disclosure, Non-Competition and Non-Solicitation Agreement. If you do not accept this offer by Monday, September 22, 2014, the offer will be deemed withdrawn.

Sincerely,

By: /s/ Amy R. Sheehan  
Amy R. Sheehan  
Executive Director, Human Resources

The foregoing correctly sets forth the terms of my at-will employment with Ophthotech Corporation. I am not relying on any representations other than those set forth above.

/s/ Michael G. Atieh  
Michael G. Atieh

9/22/2014  
Date

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## OPHTHOTECH CORPORATION

## Nonstatutory Stock Option Agreement

1. Grant of Option.

This agreement evidences the grant by Ophthotech Corporation, a Delaware corporation (the "Company"), on September 30, 2014 (the "Grant Date") to Michael G. Atieh, an employee of the Company (the "Participant"), of an option to purchase, in whole or in part, on the terms provided herein, a total of 200,000 shares (the "Shares") of common stock, \$0.001 par value per share, of the Company ("Common Stock") at \$38.93 per Share. Unless earlier terminated, this option shall expire at 5:00 p.m., Eastern time, on September 29, 2024 (the "Final Exercise Date").

The option evidenced by this agreement was granted to the Participant pursuant to the inducement grant exception under NASDAQ Stock Market Rule 5635(c)(4), and not pursuant to the Company's 2013 Stock Incentive Plan (the "Plan") or any equity incentive plan of the Company, as an inducement that is material to the Participant's employment with the Company.

It is intended that the option evidenced by this agreement shall not be an incentive stock option as defined in Section 422 of the Internal Revenue Code of 1986, as amended, and any regulations promulgated thereunder (the "Code"). Except as otherwise indicated by the context, the term "Participant", as used in this option, shall be deemed to include any person who acquires the right to exercise this option validly under its terms.

2. Vesting Schedule.

Except as otherwise provided herein, this option will become exercisable ("vest") as to 25% of the original number of Shares on one-year anniversary of the Grant Date and as to an additional 2.0833% of the original number of Shares at the end of each successive month following the one-year anniversary of the Grant Date until the fourth anniversary of the Grant Date.

The right of exercise shall be cumulative so that to the extent the option is not exercised in any period to the maximum extent permissible it shall continue to be exercisable, in whole or in part, with respect to all Shares for which it is vested until the earlier of the Final Exercise Date or the termination of this option under Section 3 hereof.

3. Exercise of Option.

(a) Form of Exercise. Each election to exercise this option shall be in writing, signed by the Participant (or such electronic notice as is approved by the Company), and received by the Company at its principal office, accompanied by this agreement and payment in full as follows:

(1) in cash or by check, payable to the order of the Company;

(2) by (i) delivery of an irrevocable and unconditional undertaking by a creditworthy broker to deliver promptly to the Company sufficient funds to pay the exercise price and any required tax withholding or (ii) delivery by the Participant to the Company of a copy of irrevocable and unconditional instructions to a creditworthy broker to deliver promptly to the Company cash or a check sufficient to pay the exercise price and any required tax withholding;

(3) to the extent approved by the Board of Directors of the Company (the "Board"), in its sole discretion, by delivery (either by actual delivery or attestation) of shares of Common Stock owned by the Participant valued at their fair market value per share as determined by (or in a manner approved by) the Board (the "Fair Market Value"), provided (i) such method of payment is then permitted under applicable law, (ii) such Common Stock, if acquired directly from the Company, was owned by the Participant for such minimum period of time, if any, as may be established by the Board in its discretion and (iii) such Common Stock is not subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements;

(4) to the extent approved by the Board, in its sole discretion, by delivery of a notice of "net exercise" to the Company, as a result of which the Participant would receive (i) the number of shares underlying the portion of this being exercised, less (ii) such number of shares as is equal to (A) the aggregate exercise price for the portion of this option being exercised divided by (B) the Fair Market Value on the date of exercise;

(5) to the extent permitted by applicable law or approved by the Board, in its sole discretion, by payment of such other lawful consideration as the Board may determine; or

(6) by any combination of the above permitted forms of payment.

The Participant may purchase less than the number of shares covered hereby, provided that no partial exercise of this option may be for any fractional share or for fewer than ten whole shares.

(b) Continuous Relationship with the Company Required. Except as otherwise provided in this Section 3, this option may not be exercised unless the Participant, at the time he exercises this option, is, and has been at all times since the Grant Date, an employee, officer or a director of, or consultant or advisor to, the Company or any other entity the employees, officers, directors, consultants, or advisors of which are eligible to receive option grants under the Plan (an "Eligible Participant").

(c) Termination of Relationship with the Company. If the Participant ceases to be an Eligible Participant for any reason, then, except as provided in paragraphs (d) and (e) below, the right to exercise this option shall terminate three months after such cessation (but in no event after the Final Exercise Date), provided that this option shall be exercisable only to the extent that the Participant was entitled to exercise this option on the date of such cessation. Notwithstanding the foregoing, if the Participant, prior to the Final Exercise Date, violates the non-competition or confidentiality provisions of any employment contract, confidentiality and nondisclosure agreement or other agreement between the Participant and the Company, the right

to exercise this option shall terminate immediately upon written notice to the Participant from the Company describing such violation.

(d) Exercise Period Upon Death or Disability. If the Participant dies or becomes disabled (within the meaning of Section 22(e)(3) of the Code) prior to the Final Exercise Date while he is an Eligible Participant and the Company has not terminated such relationship for "cause" as specified in paragraph (e) below, this option shall be exercisable, within the period of one year following the date of death or disability of the Participant, by the Participant (or in the case of death by an authorized transferee), provided that this option shall be exercisable only to the extent that this option was exercisable by the Participant on the date of his death or disability, and further provided that this option shall not be exercisable after the Final Exercise Date.

(e) Termination for Cause. If the Participant, prior to the Final Exercise Date, is terminated by the Company for Cause (as defined in the offer letter, dated as of September 20, 2014, between the Participant and the Company, or any successor agreement thereto (the "Offer Letter")), the right to exercise this option shall terminate immediately upon the effective date of such termination.

(f) Offer Letter. Notwithstanding anything to the contrary in this Section 3 or in Section 7, this option shall be subject to any applicable vesting terms set forth in the Offer Letter, including the accelerated vesting provisions set forth in Section 4 of the Offer Letter applicable in connection with certain terminations within the one year following a Change in Control Event (as defined in the Company's 2013 Stock Incentive Plan).

#### 4. Agreement in Connection with Public Offering.

The Participant agrees, in connection with an underwritten public offering of the Common Stock pursuant to a registration statement under the Securities Act, (i) not to (a) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any other securities of the Company or (b) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of shares of Common Stock or other securities of the Company, whether any transaction described in clause (a) or (b) is to be settled by delivery of securities, in cash or otherwise, during the period beginning on the date of the filing of such registration statement with the Securities and Exchange Commission and ending 180 days after the date of the final prospectus relating to the offering (plus up to an additional 34 days to the extent requested by the managing underwriters for such offering in order to address Rule 2711(f) of the Financial Industry Regulatory Authority or any similar successor provision), and (ii) to execute any agreement reflecting clause (i) above as may be requested by the Company or the managing underwriters at the time of such offering. The Company may impose stop-transfer instructions with respect to the shares of Common Stock or other securities subject to the foregoing restriction until the end of the "lock-up" period.

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#### 5. Withholding.

No Shares will be issued pursuant to the exercise of this option unless and until the Participant pays to the Company, or makes provision satisfactory to the Company for payment of, any federal, state or local withholding taxes required by law to be withheld in respect of this option. The Participant must satisfy all applicable federal, state, and local or other income and employment tax withholding obligations before the Company will deliver stock certificates or otherwise recognize ownership of Common Stock under this option. The Company may decide to satisfy the withholding obligations through additional withholding on salary or wages. If the Company elects not to or cannot withhold from other compensation, the Participant must pay the Company the full amount, if any, required for withholding or have a broker tender to the Company cash equal to the withholding obligations. Payment of withholding obligations is due before the Company will issue any shares on exercise of this option or at the same time as payment of the exercise price, unless the Company determines otherwise. If approved by the Board, in its sole discretion, a Participant may satisfy such tax obligations in whole or in part by delivery (either by actual delivery or attestation) of shares of Common Stock underlying this option valued at their Fair Market Value; *provided, however*, except as otherwise provided by the Board, that the total tax withholding where stock is being used to satisfy such tax obligations cannot exceed the Company's minimum statutory withholding obligations (based on minimum statutory withholding rates for federal and state tax purposes, including payroll taxes, that are applicable to such supplemental taxable income). Shares used to satisfy tax withholding requirements cannot be subject to any forfeiture, unfulfilled vesting or other similar requirements.

#### 6. Transfer Restrictions.

(a) This option may not be sold, assigned, transferred, pledged or otherwise encumbered by the Participant, either voluntarily or by operation of law, except by will or the laws of descent and distribution, and, during the lifetime of the Participant, this option shall be exercisable only by the Participant.

(b) The Participant agrees that he will not transfer any Shares issued pursuant to the exercise of this option unless the transferee, as a condition to such transfer, delivers to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of Section 4; provided that such a written confirmation shall not be required with respect to Section 4 after the completion of the lock-up period in connection with the Company's underwritten public offering.

#### 7. Adjustments for Changes in Common Stock and Certain Other Events.

(a) Changes in Capitalization. In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of Common Stock other than an ordinary cash dividend, the number and class of securities and exercise price per share of this option shall be equitably adjusted by the Company in the manner determined by the Board. Without limiting the generality of the foregoing, in the event the Company effects a split of the Common Stock by means of a stock dividend and the exercise price of and the number of shares subject to this option are adjusted as of the date of the

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distribution of the dividend (rather than as of the record date for such dividend), then the Participant, if he exercises this option between the record date and the distribution date for such stock dividend, shall be entitled to receive, on the distribution date, the stock dividend with respect to the shares of Common

Stock acquired upon exercise of this option, notwithstanding the fact that such shares were not outstanding as of the close of business on the record date for such stock dividend.

(b) Reorganization Events. A “Reorganization Event” shall mean: (a) any merger or consolidation of the Company with or into another entity as a result of which all of the Common Stock of the Company is converted into or exchanged for the right to receive cash, securities or other property or is cancelled, (b) any transfer or disposition of all of the Common Stock of the Company for cash, securities or other property pursuant to a share exchange or other transaction or (c) any liquidation or dissolution of the Company. In connection with a Reorganization Event, the Board may take any one or more of the following actions with respect to this option (or any portion thereof) on such terms as the Board determines: (i) provide that this option shall be assumed, or substantially equivalent option shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof), (ii) upon written notice to the Participant, provide that the unexercised portion of this option will terminate immediately prior to the consummation of such Reorganization Event unless exercised by the Participant within a specified period following the date of such notice, (iii) provide that this option shall become exercisable, realizable, or deliverable, or restrictions applicable to this option shall lapse, in whole or in part prior to or upon such Reorganization Event, (iv) in the event of a Reorganization Event under the terms of which holders of Common Stock will receive upon consummation thereof a cash payment for each share surrendered in the Reorganization Event (the “Acquisition Price”), make or provide for a cash payment to the Participant with respect to this option equal to (A) the number of shares of Common Stock subject to the vested portion of this option (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such Reorganization Event) multiplied by (B) the excess, if any, of (I) the Acquisition Price over (II) the exercise price of this option and any applicable tax withholdings, in exchange for the termination of this option, (v) provide that, in connection with a liquidation or dissolution of the Company, this option shall convert into the right to receive liquidation proceeds (if applicable, net of the exercise, measurement or purchase price thereof and any applicable tax withholdings) and (vi) any combination of the foregoing.

For purposes of clause (i) above, this option shall be considered assumed if, following consummation of the Reorganization Event, this option confers the right to purchase, for each share of Common Stock subject to this option immediately prior to the consummation of the Reorganization Event, the consideration (whether cash, securities or other property) received as a result of the Reorganization Event by holders of Common Stock for each share of Common Stock held immediately prior to the consummation of the Reorganization Event (and if holders were offered a choice of consideration, the type of consideration chosen by the holders of a majority of the outstanding shares of Common Stock); provided, however, that if the consideration received as a result of the Reorganization Event is not solely common stock of the acquiring or succeeding corporation (or an affiliate thereof), the Company may, with the consent of the acquiring or succeeding corporation, provide for the consideration to be received upon the exercise of this option to consist solely of such number of shares of common stock of the

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acquiring or succeeding corporation (or an affiliate thereof) that the Board determined to be equivalent in value (as of the date of such determination or another date specified by the Board) to the per share consideration received by holders of outstanding shares of Common Stock as a result of the Reorganization Event.

8. Miscellaneous.

(a) No Right To Employment or Other Status. The grant of this option shall not be construed as giving the Participant the right to continued employment or any other relationship with the Company. The Company expressly reserves the right at any time to dismiss or otherwise terminate its relationship with the Participant free from any liability or claim hereunder, except as otherwise expressly provided herein or provided for in the Offer Letter.

(b) No Rights As Stockholder. Subject to the provisions of this option, the Participant shall not have any rights as a stockholder with respect to any shares of Common Stock to be distributed with respect to this option until becoming the record holder of such shares.

(c) Entire Agreement. This Agreement, together with the Offer Letter, constitute the entire agreement between the parties, and supersede all prior agreements and understandings, relating to the subject matter hereof.

(d) Amendment. Except with respect to any vesting terms set forth in the Offer Letter, the Board may amend, modify or terminate this Agreement, including but not limited to, substituting another option of the same or a different type and changing the date of exercise or realization. Notwithstanding the foregoing, the Participant’s consent to such action shall be required unless (i) the Board determines that the action, taking into account any related action, would not materially and adversely affect the Participant, or (ii) the change is permitted under Section 7 and the Offer Letter.

(e) Acceleration. The Board may at any time provide that this option shall become immediately exercisable in whole or in part, free of some or all restrictions or conditions, or otherwise realizable in whole or in part, as the case may be.

(f) Conditions on Delivery of Stock. The Company will not be obligated to deliver any shares of Common Stock pursuant to this Agreement until (i) all conditions of this Agreement have been met to the satisfaction of the Company, (ii) in the opinion of the Company’s counsel, all other legal matters in connection with the issuance and delivery of such shares have been satisfied, including any applicable securities laws and regulations and any applicable stock exchange or stock market rules and regulations, and (iii) the Participant has executed and delivered to the Company such representations or agreements as the Company may consider appropriate to satisfy the requirements of any applicable laws, rules or regulations.

(g) Administration by Board. The Board will administer this Agreement and may construe and interpret the terms hereof Subject to the terms and provisions of the Offer Letter, the Board may correct any defect, supply any omission or reconcile any inconsistency in this

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Agreement in the manner and to the extent it shall deem expedient to carry the Agreement into effect and it shall be the sole and final judge of such expediency. No director or person acting pursuant to the authority delegated by the Board shall be liable for any action or determination relating to or under this Agreement made in good faith.

(h) Appointment of Committees. To the extent permitted by applicable law, the Board may delegate any or all of its powers hereunder to one or more committees or subcommittees of the Board (a “Committee”). All references herein to the “Board” shall mean the Board or a Committee to the extent that the Board’s powers or authority hereunder have been delegated to such Committee.

(i) Severability. The invalidity or unenforceability of any provision hereof shall not affect the validity or enforceability of any other provision hereof, and each such other provision shall be severable and enforceable to the extent permitted by law.

(j) Governing Law. This Agreement shall be governed by and interpreted in accordance with the laws of the State of Delaware, excluding choice-of-law principles of the law of such state that would require the application of the laws of a jurisdiction other than the State of Delaware.

(k) Counterparts. This Agreement may be executed in one or more counterparts, each of which shall be deemed to be an original but all of which together will constitute one in the same instrument.

The Company has caused this option to be executed by its duly authorized officer.

**OPHTHOTECH CORPORATION**

By: /s/ David Guyer  
Name: David Guyer  
Title: Chief Executive Officer

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Agreement in the manner and to the extent it shall deem expedient to carry the Agreement into effect and it shall be the sole and final judge of such expediency. No director or person acting pursuant to the authority delegated by the Board shall be liable for any action or determination relating to or under this Agreement made in good faith.

(h) Appointment of Committees. To the extent permitted by applicable law, the Board may delegate any or all of its powers hereunder to one or more committees or subcommittees of the Board (a "Committee"). All references herein to the "Board" shall mean the Board or a Committee to the extent that the Board's powers or authority hereunder have been delegated to such Committee.

(i) Severability. The invalidity or unenforceability of any provision hereof shall not affect the validity or enforceability of any other provision hereof, and each such other provision shall be severable and enforceable to the extent permitted by law.

(i) Governing Law. This Agreement shall be governed by and interpreted in accordance with the laws of the State of Delaware, excluding choice-of-law principles of the law of such state that would require the application of the laws of a jurisdiction other than the State of Delaware.

(k) Counterparts. This Agreement may be executed in one or more counterparts, each of which shall be deemed to be an original but all of which together will constitute one in the same instrument.

The Company has caused this option to be executed by its duly authorized officer.

**OPHTHOTECH CORPORATION**

By: /s/ David Guyer  
Name: David Guyer  
Title: Chief Executive Officer

Employee Name: /s/ Michael G. Atieh  
Date: 10/8/2014

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## CERTIFICATIONS

I, David R. Guyer, M.D., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Ophthotech Corporation;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 12, 2014

By: /s/ David R. Guyer  
David R. Guyer, M.D.  
Chief Executive Officer  
(Principal Executive Officer)

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## CERTIFICATIONS

I, Michael G. Atieh, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Ophthotech Corporation;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 12, 2014

By: /s/ Michael G. Atieh  
Michael G. Atieh  
Chief Financial and Business Officer  
(Principal Financial Officer)

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**CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,****AS ADOPTED PURSUANT TO****SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Quarterly Report on Form 10-Q of Ophthotech Corporation (the "Company") for the period ended September 30, 2014 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, David R. Guyer, M.D., Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

- (1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 12, 2014

By: /s/ David R. Guyer  
David R. Guyer M.D.  
Chief Executive Officer  
(Principal Executive Officer)

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**CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,****AS ADOPTED PURSUANT TO****SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Quarterly Report on Form 10-Q of Ophthotech Corporation (the "Company") for the period ended September 30, 2014 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Michael G. Atieh, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

- (1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 12, 2014

By: /s/ Michael G. Atieh

Michael G. Atieh  
Chief Financial and Business Officer  
(Principal Financial Officer)

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