Table of Contents

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

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2016

OR

o 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	**20-8185347**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)
One Penn Plaza, 19th Floor **New York, NY**	**10119**
(Address of principal executive offices)	(Zip Code)

(212) 845-8200
(Registrant's telephone number, including area code)

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 x No o

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 x No o

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

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

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

As of May 2, 2016 there were 35,370,681 shares of Common Stock, $0.001 par value per share, outstanding.

Table of Contents

TABLE OF CONTENTS

PART II—OTHER INFORMATION

Table of Contents

FORWARD-LOOKING STATEMENTS

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 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® (pegpleranib) 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 of the initiation of and completion of enrollment in such trials, 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 clinical trials for Zimura® (avacincaptad pegol) for the treatment of patients with geographic atrophy, or GA, a form of dry AMD and, in combination with anti-VEGF drugs, for the treatment of wet AMD, including statements regarding the timing of the initiation of and completion of enrollment in such trials, and the costs to obtain and timing of receipt of initial results from, and the completion of, such trials;
- the timing, costs, conduct and outcome of our preclinical work for an ophthalmic formulation of tivozanib, including statements regarding the timing of the initiation of, and the costs to obtain and timing of receipt of results from, such work;
- the timing of and our ability to obtain marketing approval of Fovista, Zimura and other product candidates we may develop, and the ability of Fovista, Zimura and other product candidates we may develop to meet existing or future regulatory standards;
- our ability to maintain a productive collaborative relationship with Novartis Pharma AG, including our ability to achieve remaining potential milestone payments under our agreement;
- the potential advantages of Fovista and Zimura;
- the rate and degree of potential market acceptance and clinical utility of Fovista and Zimura;
- our estimates regarding the potential market opportunity for Fovista and Zimura;
- the potential receipt of revenues from future sales of Fovista and Zimura;
- our sales, marketing and distribution capabilities and strategy;
- our ability to establish and maintain arrangements for the 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 existing and new governmental laws and regulations; and
- our competitive position.

Table of Contents

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and our stockholders should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this 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 and our other periodic reports completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Quarterly Report on Form 10-Q are made as of the date of this Quarterly Report on Form 10-Q, and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

Table of Contents

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

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

	March 31, 2016	December 31, 2015
Assets		
Current assets		
Cash and cash equivalents	$ 183,822	$ 221,861
Available for sale securities	109,104	74,731
Due from Novartis Pharma AG	10,062	4,389
Income tax receivable	26,497	3,421
Prepaid expenses and other current assets	2,299	2,083
Total current assets	331,784	306,485
Available for sale securities	63,211	95,298
Property and equipment, net	3,350	3,466
Deferred tax assets	—	23,113
Other assets	500	489
Total assets	$ 398,845	$ 428,851
Liabilities and Stockholders' Equity		
Current liabilities		
Accrued research and development expenses	$ 21,727	$ 18,820
Accounts payable and accrued expenses	7,287	12,018
Deferred revenue	5,212	6,667
Total current liabilities	34,226	37,505
Deferred revenue, long-term	206,396	206,399
Royalty purchase liability	125,000	125,000
Total liabilities	365,622	368,904
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, 35,290,135 and 35,196,567 shares issued and outstanding at March 31, 2016 and December 31, 2015, respectively	35	35
Additional paid-in capital	475,024	465,924
Accumulated deficit	(441,840)	(405,539)
Accumulated other comprehensive income (loss)	4	(473)
Total stockholders' equity	33,223	59,947
Total liabilities and stockholders' equity	$ 398,845	$ 428,851

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

Table of Contents

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

	Three Months Ended March 31, 2016	2015
Collaboration revenue	$ 15,721	$ 41,678
Operating expenses:		
Research and development	37,770	24,557
General and administrative	14,696	9,584
Total operating expenses	52,466	34,141
Income (loss) from operations	(36,745)	7,537
Interest income	446	125

Other income (loss)	30	(52)
Income (loss) before income tax provision	(36,269)	7,610
Income tax provision	32	974
Net income (loss)	$ (36,301)	$ 6,636
Net income (loss) per common share:		
Basic	$ (1.03)	$ 0.19
Diluted	$ (1.03)	$ 0.19
Weighted average common shares outstanding:		
Basic	35,256	34,154
Diluted	35,256	35,239

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

Table of Contents

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

	Three Months Ended March 31, 2016	2015
Net income (loss)	$ (36,301)	$ 6,636
Other comprehensive income:		
Unrealized gain on available for sale securities, net of tax	477	48
Other comprehensive income:	477	48
Comprehensive income (loss)	$ (35,824)	$ 6,684

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

Table of Contents

OPHTHOTECH CORPORATION
Unaudited Statements of Cash Flows
(in thousands)

	Three months ended March 31, 2016	2015
Operating Activities		
Net income (loss)	$ (36,301)	$ 6,636
Adjustments to reconcile net income (loss) to net cash used in operating activities		
Depreciation	178	56
Amortization of premium and discounts on investment securities	196	991
Deferred income taxes	23,113	—
Share-based compensation	8,332	5,054
Changes in operating assets and liabilities:		
Due from Novartis Pharma AG	(5,673)	(49,289)
Income tax receivable	(23,076)	—
Prepaid expense and other current assets	(216)	(88)
Accrued interest receivable	—	(447)
Other assets	(11)	21
Accrued research and development expenses	2,907	185
Accounts payable and accrued expenses	(4,731)	(2,950)
Deferred revenue	(1,458)	8,322
Net cash used in operating activities	(36,740)	(31,509)
Investing Activities		
Purchase of marketable securities	(12,005)	(144,433)
Maturities of marketable securities	10,000	174,000
Purchase of property and equipment	(62)	(64)
Net cash (used in) provided by investing activities	(2,067)	29,503
Financing Activities		
Proceeds from stock option/warrant exercises	768	1,760
Net cash provided by financing activities	768	1,760
Net change in cash and cash equivalents	(38,039)	(246)
Cash and cash equivalents		
Beginning of period	221,861	39,814
End of period	$ 183,822	$ 39,568
Supplemental disclosures of non-cash information related to investing activities		
Change in unrealized gain on available for sale securities, net of tax	$ 477	$ 48

Table of Contents

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® (pegpleranib), which is an anti-platelet derived growth factor ("PDGF") aptamer that is in Phase 3 clinical development for use in combination with anti-vascular endothelial growth factor ("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® (ranibizumab), has completed patient enrollment for two Phase 3 clinical trials of Fovista administered in combination with Lucentis and expects to complete enrollment in a third Phase 3 clinical trial evaluating Fovista in combination with Eylea® (aflibercept) or Avastin® (bevacizumab) in 2016. The Company is also developing its product candidate Zimura® (avacincaptad pegol), an inhibitor of complement factor C5, as a monotherapy for the treatment of patients with geographic atrophy ("GA"), a form of dry AMD, as well as in combination with anti-VEGF drugs for the treatment of wet AMD, and for the treatment of polypoidal choroidal vasculopathy ("PCV"), a specific type of wet AMD, in patients who do not respond adequately to treatment with anti-VEGF monotherapy or for whom anti-VEGF monotherapy fails. The Company is also investigating the potential of an ophthalmic formulation for tivozanib, a small molecule VEGF tyrosine kinase inhibitor for which the Company has an option for a license.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited financial information as of March 31, 2016 and for the three months ended March 31, 2016 and 2015 has been prepared by the Company pursuant to the rules and regulations of the Securities and Exchange Commission ("SEC"). 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, 2015 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 2015, as filed with the SEC on February 26, 2016.

In the opinion of management, the unaudited financial information as of March 31, 2016 and for the three months ended March 31, 2016 and 2015, reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations and cash flows. The results of operations for the three months ended March 31, 2016 and 2015 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 research and development costs, revenue recognition, accounting for share-based compensation 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 90 days or less when purchased to be cash equivalents. The carrying amounts reported in the Balance Sheets for cash and cash equivalents are valued at cost, which approximates their fair value.

Available for Sale Securities

The Company considers securities with original maturities of greater than 90 days when purchased to be available for sale securities. Available for sale securities with original maturities of greater than one year are recorded as non-current assets. Available for sale securities are recorded at fair value and unrealized gains and losses are recorded within accumulated other comprehensive income

Table of Contents

(loss). The estimated fair value of the available for sale securities is determined based on quoted market prices or rates for similar instruments. In addition, the cost of debt securities in this category is adjusted for amortization of premium and accretion of discount to maturity. The Company evaluates securities with unrealized losses to determine whether such losses, if any, are other than temporary.

Revenue Recognition

Collaboration Revenue

Prior to 2014, the Company had not generated any revenue. In May 2014, the Company received an upfront payment of $200.0 million in connection with its licensing and commercialization agreement with Novartis Pharma AG, (the "Novartis Agreement"), which has not been recorded as revenue due to certain contingencies associated with the payment. In each of September 2014 and March 2015, the Company achieved a $50.0 million enrollment-based milestone, or $100.0 million in the aggregate, under the Novartis Agreement. The Company uses the relative selling price method to allocate arrangement consideration to the Company's performance obligations under the Novartis Agreement. Below is a summary of the components of the Company's collaboration revenue for the three months ended March 31, 2016 and 2015:

	Three months ended March 31,	
	2016	2015
License revenue	$ —	$ 38,083
Research and development activity revenue	1,275	3,585
API transfer revenue	14,443	—
Joint operating committee revenue	3	10
Total collaboration revenue	$ 15,721	$ 41,678

In the future, the Company may generate additional revenues from a combination of product sales and license fees, milestone payments, research and development activity-related payments, payments for manufactured material 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 preclinical, clinical and commercial material. Payments to the Company under these arrangements typically include one or more of the following: non-refundable, upfront license fees; option exercise fees; funding of research and/or development efforts; milestone payments; payments for manufactured material; and royalties on future product sales.

When evaluating multiple element arrangements, the Company considers whether the deliverables under the arrangement represent separate units of accounting. This evaluation requires subjective determinations and requires management to make judgments about the individual deliverables and whether such deliverables are separable from the other aspects of the contractual relationship. In determining the units of accounting, management evaluates certain criteria, including whether the deliverables have standalone value, based on the 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 that is subject to the license. In validating the Company's BESP, the Company evaluates whether changes in the key assumptions used to determine the BESP will have a significant effect on the allocation of arrangement consideration among multiple deliverables.

Table of Contents

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

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

The Company aggregates its milestones into three categories: (i) clinical and development milestones, (ii) regulatory milestones, and (iii) commercial milestones. Clinical and development milestones are typically achieved when a product candidate advances into a defined phase of clinical research or completes such phase or when a contractually specified clinical trial enrollment target is attained. Regulatory milestones are typically achieved upon acceptance of the submission of an application for marketing approval for a product candidate or upon approval to market the product candidate by the U.S. Food and Drug Administration (the "FDA") or other regulatory authorities. 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 reimbursement milestones are not substantive and that the marketing approval milestones are substantive. Milestone 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 generally exceed federally insured limits. The Company maintains its cash equivalents in investments in money market funds and, at times, in U.S. Treasury securities with original maturities of 90 days or less.

The Company's available for sale securities are also invested in U.S. Treasury securities and investment-grade corporate debt securities. 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 denominated in foreign currencies 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.

Table of Contents

Financial Instruments

Cash equivalents and available for sale securities are reflected in the accompanying financial statements at fair value. The carrying amount of accounts payable and accrued expenses, including accrued research and development expenses, approximates fair value due to the short-term nature of those instruments.

Property and Equipment

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

Research and Development

Research and development expenses primarily consist of costs associated with the manufacturing, development and clinical testing of Fovista and Zimura as well as costs associated with the preclinical development of other product candidates and formulations. Research and development expenses consist of:

- external research and development expenses incurred under arrangements with third parties, such as contract research organizations ("CROs") and other vendors and contract manufacturing organizations ("CMOs") for the production of drug substance and drug product; and
- employee-related expenses, including salaries, benefits and share-based compensation expense.

Research and development expenses 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 expenses are charged to operations as incurred in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic, or ASC, 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.

Income Taxes

The Company utilizes the liability method of accounting for deferred income taxes, as set forth in ASC 740, *Income Taxes*. Under this method, deferred tax assets and liabilities 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 incurred U.S. federal net operating losses ("NOLs") in each year from its inception in 2007 through 2013 and utilized these NOLs in 2014. In addition, the Company generated a taxable loss in 2015 that it intends to carry back to the 2014 tax year. As such, all prior tax years since 2007 remain subject to potential tax examination as the utilization of NOLs from prior years opens the relevant year to potential audit.

Share-Based Compensation

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

Stock Options

The Company estimates the fair value of stock options granted to employees and non-employee directors 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 utilizes a dividend yield of zero based on the fact that the Company has never paid cash dividends

Table of Contents

to stockholders and has no current intentions to pay cash dividends. 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 consultants, the Company recognizes expense in accordance with the requirements of ASC 505-50, *Equity Based Payments to Non-Employees*. Consultant stock option grants 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 consultants 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.

RSUs

The Company estimates the fair value of RSUs granted to employees using the closing market price of the Company's common stock on the date of grant.

Share-based compensation expense includes expenses related to stock options and RSUs granted to employees, non-employee directors and consultants, and has been reported in the Company's Statements of Operations as follows:

	Three months ended March 31,	
	2016	2015
Research and development	$ 4,685	$ 3,115
General and administrative	3,647	1,939
Total	$ 8,332	$ 5,054

Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board, or FASB, issued Accounting Standards Update, or ASU, 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. The FASB subsequently issued additional clarifying standards to address issues arising from implementation of the new revenue standard, including a one-year deferral of the effective date for the new revenue standard. Public companies should now apply the guidance in ASU 2014-09 to annual reporting periods beginning after December 15, 2017 and interim periods within those annual periods. Earlier application is permitted only as of annual reporting periods beginning after December 15, 2016, including interim periods within that annual period. Companies may use either a full retrospective or a modified retrospective approach to adopt ASU 2014-09. The Company is currently assessing the impact that adopting this new accounting guidance will have on its financial statements and footnote disclosures.

In August 2014, the FASB issued ASU 2014-15, *Presentation of Financial Statements—Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*, which defines management's responsibility to assess an entity's ability to continue as a going concern, and to provide related footnote disclosures if there is substantial doubt about its ability to continue as a going concern. The pronouncement is effective for annual reporting periods ending after December 15, 2016 with early adoption permitted. The adoption of this guidance is not expected to have a significant impact on the Company's financial statements.

In November 2015, the FASB issued ASU 2015-17, *Income Taxes (Topic 740)*: *Balance Sheet Classification of Deferred Taxes*, which updated and simplified the presentation of deferred income taxes. Current GAAP requires an entity to separate deferred income tax assets and liabilities into current and non-current amounts in a classified statement of financial position. The requirement results in little or no benefit to users of financial statements because the classification does not generally align with the time period in which the recognized deferred tax amounts are expected to be recovered or settled. To simplify the presentation of deferred income taxes, the amendments in this update require that deferred tax assets and liabilities be classified as noncurrent in a classified statement of financial position. The current requirement that deferred tax assets and liabilities of a tax-paying component of an entity be offset and presented as a single amount is not affected by the amendments in this update. The amendments in this update are effective for

Table of Contents

financial statements issued for annual periods beginning after December 15, 2016 and interim periods within those annual periods. Earlier application is permitted as of the beginning of an interim or annual reporting period. The Company has elected to adopt this standard retrospectively, effective December 31, 2015.

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)*. Under the new guidance, lessees will be required to recognize the following for all leases (with the exception of short-term leases) at the commencement date: (1) A lease liability, which is a lessee's obligation to make lease payments arising from a lease, measured on a discounted basis; and (2) A right-of-use asset, which is an asset that represents the lessee's right to use, or control the use of, a specified asset for the lease term. Under the new guidance, lessor accounting is largely unchanged. Certain targeted improvements were made to align, where necessary, lessor accounting with the lessee accounting model and Topic 606, *Revenue from Contracts with Customers*. The new lease

guidance simplified the accounting for sale and leaseback transactions primarily because lessees must recognize lease assets and lease liabilities. Lessees will no longer be provided with a source of off-balance sheet financing. Public business entities should apply the amendments in ASU 2016-02 for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years (i.e., January 1, 2019, for a calendar year entity). Early application is permitted for all public business entities and all nonpublic business entities upon issuance. Lessees (for capital and operating leases) and lessors (for sales-type, direct financing, and operating leases) must apply a modified retrospective transition approach for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements. The modified retrospective approach would not require any transition accounting for leases that expired before the earliest comparative period presented. Lessees and lessors may not apply a full retrospective transition approach. The Company is currently assessing the impact that adopting this new accounting guidance will have on its financial statements and footnote disclosures.

In March 2016, the FASB issued ASU 2016-09, *Compensation - Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting*. The amendments are intended to improve the accounting for employee share-based payments and affect all organizations that issue share-based payment awards to their employees. Several aspects of the accounting for share-based payment award transactions are simplified, including: (*a*) income tax consequences; (*b*) classification of awards as either equity or liabilities; and (*c*) classification on the statement of cash flows. For public companies, the amendments are effective for annual periods beginning after December 15, 2016, and interim periods within those annual periods. Early adoption is permitted in any interim or annual period. The Company is currently assessing the impact that adopting this new accounting guidance will have on its financial statements and footnote disclosures.

3. Net Income (Loss) Per Common Share

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

	Three months ended March 31,	
	2016	2015
Basic and diluted net income (loss) per common share calculation:		
Net income (loss)	$ (36,301)	$ 6,636
Weighted average common shares outstanding - basic	35,256	34,154
Plus: effect of dilutive stock options, warrants and unvested restricted stock units	—	1,085
Weighted average common shares outstanding - dilutive	35,256	35,239
Net income (loss) per common share - basic	$ (1.03)	$ 0.19
Net income (loss) per common share - diluted	$ (1.03)	$ 0.19

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

Table of Contents

	Three months ended March 31,	
	2016	2015
Stock options outstanding	3,495	929
Restricted stock units	404	—
Total	3,899	929

4. Cash, Cash Equivalents and Available for Sale Securities

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at the date of purchase to be cash equivalents. Cash and cash equivalents included cash of $1.3 million and $5.5 million at March 31, 2016 and December 31, 2015, respectively. Cash and cash equivalents at March 31, 2016 and December 31, 2015 also included $182.5 million and $216.4 million, respectively, of investments in money market funds and U.S. Treasury securities with original maturities of 90 days or less.

The Company considers securities with original maturities of greater than 90 days at the date of purchase to be available for sale securities. The Company held available for sale securities with a fair value totaling $172.3 million and $170.0 million at March 31, 2016 and December 31, 2015, respectively. These available for sale securities consisted of U.S. Treasury securities and investment-grade corporate debt securities. At March 31, 2016, the Company held available for sale securities of $109.1 million with maturities of less than one year, and $63.2 million with maturities of greater 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 losses in fair value of its investments as of March 31, 2016. The Company classifies these securities as available for sale, however, the Company does not currently intend to sell its investments and the Company believes it is more likely than not that the Company will recover the carrying value of these investments.

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

	As of March 31, 2016			
	Cost	Fair Value	Carrying Value	Unrealized Gain
U.S. Treasury securities	$ 132,386	$ 132,390	$ 132,390	$ 4
Corporate debt securities	39,925	39,925	39,925	—
Total	$ 172,311	$ 172,315	$ 172,315	$ 4

	As of December 31, 2015

	Cost	Fair Value	Carrying Value	Unrealized Loss
U.S. Treasury securities	$ 130,507	$ 130,196	$ 130,196	$ (311)
Corporate debt securities	39,995	39,833	39,833	(162)
Total	$ 170,502	$ 170,029	$ 170,029	$ (473)

The Company's available for sale securities are reported at fair value on the Company's balance sheet. Unrealized gains (losses) are reported within accumulated other comprehensive income (loss) in the statements of comprehensive income (loss). The cost of securities sold and any realized gains/losses from the sale of available for sale securities are based on the specific identification method. The changes in accumulated other comprehensive income (loss) associated with the unrealized gain on available for sale securities during the three months ended March 31, 2016 and 2015 were as follows:

Table of Contents

	Three months ended March 31, 2016	2015
Beginning balance	$ (473)	$ (65)
Current period changes in fair value before reclassifications, net of tax	477	48
Amounts reclassified from accumulated other comprehensive income, net of tax	—	—
Total other comprehensive income, net of tax	477	48
Ending balance	$ 4	$ (17)

5. Licensing and Commercialization Agreement with Novartis Pharma AG

In May 2014, the Company entered into a licensing and commercialization agreement with Novartis Pharma AG ("Novartis", and such agreement, 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, or API, supplied by the Company, standalone Fovista products and products combining Fovista with an anti-VEGF drug 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 drug to which Novartis has rights, as well as a pre-filled syringe presentation of such products and to use commercially reasonable efforts, subject to obtaining marketing approval, to commercialize licensed products in the Novartis Territory in accordance with agreed development and marketing plans. Novartis has also granted the Company options, subject to specified limitations, and to the extent such rights are controlled by Novartis, to obtain exclusive rights from Novartis to develop and commercialize in the United States the co-formulated and pre-filled syringe products developed by Novartis. The Company and Novartis have each granted the other options, subject to specified limitations, to obtain access to study data from certain clinical trials of licensed products that the Company or Novartis may conduct, including for use by the other in regulatory filings in its territory. The Company has agreed to exclusively supply Novartis, and Novartis has agreed to exclusively purchase from the Company, its clinical and commercial requirements for the bulk API for 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. Under the terms 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-based milestones for its Phase 3 clinical program for Fovista, of which $50.0 million was achieved in September 2014 and received by the Company in October 2014 and $50.0 million was achieved in March 2015 and received in April 2015, and up to an aggregate of an additional $300.0 million upon achievement of specified regulatory milestones, including marketing approval and 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 is also 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 for clinical supplies and the Company's manufacturing costs plus a specified percentage margin for commercial supplies of the bulk API for 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

Table of Contents

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 drug to which Novartis has rights in the Novartis Territory, for use in the Phase 3 trials already initiated and in other Phase 2 and Phase 3 clinical trials in the Novartis Territory initiated 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. 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 exercises 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 Novartis Agreement were evaluated under ASC 605-25, *Revenue Recognition—Multiple Element Arrangements* ("ASC 605-25") (as amended by ASU 2009-13, *Revenue Recognition* ("ASU 2009-13")) to determine if they represented a multiple element revenue arrangement. The Novartis Agreement includes the following deliverables: (1) an exclusive license to commercialize Fovista outside the United States (the "License Deliverable"); (2) the performance obligation to conduct research and development activities related to the Phase 3 Fovista clinical trials and certain Phase 2 trials for Fovista (the "R&D Activity Deliverable"); (3) the performance obligation to supply API to Novartis for development and manufacturing purposes (the "Manufacturing Deliverable") and (4) the Company's obligation to participate on the joint operating committee established under the terms of the Novartis Agreement and related subcommittees (the "Joint Operating Committee Deliverable"). Novartis has the right, subject to 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 the commercialization of Fovista in the Novartis Territory. The R&D Activity Deliverable includes the right and responsibility for the Company to conduct the Phase 3 Fovista clinical program and other studies of Fovista in the Novartis Territory which are necessary or desirable for regulatory approval or commercialization of Fovista. The Manufacturing Deliverable includes the obligation for the Company to supply API to Novartis for clinical purposes, for which Novartis has agreed to pay the Company's manufacturing costs, and for commercial purposes, for which Novartis has agreed to pay the Company's manufacturing costs plus a specified margin. The Joint Operating Committee Deliverable includes the obligation to participate in the Joint Operating Committee and related subcommittees at least through the first anniversary of regulatory approval in the European Union. All of these deliverables were deemed to have stand-alone value and to meet the criteria

Table of Contents

to be accounted for as separate units of accounting under ASC 605-25. Factors considered in this determination included, among other things, the subject of the licenses and the research and development and commercial capabilities of Novartis. Accordingly, each unit will be accounted for separately.

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

The Novartis Agreement provides that, if the Company elects to terminate the Novartis Agreement because specified governmental actions prevent the parties from materially progressing the development or commercialization of licensed products as described above, the Company will be required to pay a substantial termination fee. The Company has concluded that this termination provision constitutes a contingent event that was 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 reimbursement 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 marketing 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 May 2014, the Company received an upfront payment of $200.0 million in connection with its entry into the Novartis Agreement, which has not been recorded as revenue due to certain contingencies associated with the payment. In each of September 2014 and March 2015, the Company achieved a $50.0 million enrollment-based milestone, or $100.0 million in aggregate, under the Novartis Agreement. The Company uses the relative selling price method to allocate arrangement consideration to the Company's performance obligations under the Novartis Agreement. Below is a summary of the components of the Company's collaboration revenue for the three months ended March 31, 2016 and 2015:

	Three months ended March 31,	
	2016	2015
License revenue	$ —	$ 38,083
Research and development activity revenue	1,275	3,585
API transfer revenue	14,443	—
Joint operating committee revenue	3	10
Total collaboration revenue	$ 15,721	$ 41,678

6. Financing Agreement with Novo A/S

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

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

$41.7 million, or $125.0 million in the aggregate, and Novo A/S received, in the aggregate, a right to receive royalties on net sales of Fovista at a mid-single digit percentage.

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

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

The $125.0 million in aggregate proceeds from the three financing tranches under the Novo Agreement represents the full funding available under the Novo Agreement, and has been recorded as a liability on the Company's Balance Sheet as of March 31, 2016, in accordance with ASC 730, *Research and Development*. Because there is a significant related party relationship between the Company and Novo A/S, the Company is treating its obligation to make royalty payments under the Novo Agreement as an implicit obligation to repay the funds advanced by Novo A/S. As the Company makes royalty payments in accordance with the Novo Agreement, it will reduce the liability balance. At the time that such royalty payments become probable and estimable, and if such amounts exceed the liability balance, the Company will impute interest accordingly on a prospective basis based on such estimates, which would result in a corresponding increase in the liability balance.

The Novo Agreement requires the establishment of a Joint Oversight Committee in the event that Novo A/S does not continue to have a representative on the Company's board of directors. The Joint Oversight Committee would have responsibilities that include "discussion and review" of all matters related to Fovista research, development, regulatory approval and commercialization, but there is no provision either implicit or explicit that gives the Joint Oversight Committee or its members decision-making authority.

7. Income Taxes

The Company utilizes the liability method of accounting for deferred income taxes. Under this method, deferred tax assets and liabilities 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 March 31, 2016, the Company had accrued approximately $0.3 million in interest and penalties related to the timing of certain tax payments for the 2014 tax year.

In assessing the realizability of deferred tax assets, the Company considers whether it is more likely than not that some portion or all of the deferred tax assets will not be realized. The ultimate realization of deferred tax assets is dependent upon the generation of taxable income during the periods in which the temporary differences representing net future deductible amounts become deductible, and is impacted by the Company's ability to carryback losses to 2014, the only year in which the Company had taxable income. The Company is currently projecting tax losses in 2016. The Company expects to realize its

net deferred tax assets recorded as of December 31, 2015 in 2016 due to the Company's ability to carryback its 2015 federal tax losses to 2014. As such, the Company reclassified these amounts to income tax receivable on the Company's Balance Sheet as of March 31, 2016. The Company expects to carry forward its 2015 state tax losses due to various state restrictions on the use of carryback claims. The state NOLs are expected to begin to expire in 2027. Because of 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 remaining 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 RSUs. Although certain of these deductions will be reported on the corporate tax returns and increase the Company's NOLs, these related tax benefits are not recognized for financial reporting purposes.

8. Fair Value Measurements

ASC 820, *Fair Value Measurements and Disclosures* ("ASC 820"), 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 Company reviews investments on a periodic basis for other than temporary impairments. This review is subjective as it requires management to evaluate whether an event or change in circumstances has occurred in the period that may have a significant

Table of Contents

adverse effect on the fair value of the investment. The Company uses the market approach to measure fair value for its financial assets. The market approach uses prices and other relevant information generated by market transactions involving identical or comparable assets. The Company classifies its corporate debt securities within the fair value hierarchy as Level 2 assets, as it primarily utilizes quoted market prices or rates for similar instruments to value these securities.

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

- Level 1—inputs to the valuation methodology are quoted prices (unadjusted) for an identical asset or liability in an active market. The Company's Level 1 assets consist of investments in 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. The Company's Level 2 assets consist of investments in investment-grade corporate debt securities.

- Level 3—inputs to the valuation methodology are unobservable and significant to the fair value measurement of the asset or liability. The Company does not hold any assets that are measured using Level 3 inputs.

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 March 31, 2016:

	Fair Value Measurement Using		
	Quoted prices in active markets for identical assets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets			
Investments in money market funds*	$ 182,496	$ —	$ —
Investments in U.S. Treasury securities	$ 132,390	$ —	$ —
Investments in Corporate debt securities	$ —	$ 39,925	$ —

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, 2015:

	Fair Value Measurement Using		
	Quoted prices in active markets for identical assets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets			
Investments in money market funds*	$ 196,188	$ —	$ —
Investments in U.S. Treasury securities*	$ 150,387	$ —	$ —
Investments in Corporate debt securities	$ —	$ 39,833	$ —

* Investments in money market funds and U.S. Treasury securities with maturities less than 90 days are reflected in cash and cash equivalents in the accompanying Balance Sheets.

No transfer of assets between Level 1 and Level 2 of the fair value hierarchy occurred during the three months ended March 31, 2016.

9. Stock Option and Compensation Plans

The Company adopted its 2007 Stock Incentive Plan (the "2007 Plan") for employees, non-employee directors and consultants for the purpose of advancing the interests of the Company's 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

Table of Contents

option awards, RSU 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. In June 2015, the Company's board of directors adopted a first amendment to the 2013 Plan. The 2013 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, RSUs, restricted stock unit awards, and other stock-based awards. Upon the effectiveness of the 2013 Plan, the number of shares of the Company's common stock that were reserved for issuance under the 2013 Plan was the sum of (1) such number of shares (up to approximately 3,359,641 shares) as is equal to the sum of 739,317 shares (the number of shares of the common stock then available for issuance under the 2007 Plan), and such number of shares of the Company's common stock that are subject to outstanding awards under the 2007 Plan that expire, terminate or are otherwise surrendered, canceled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right plus (2) an annual increase, to be added the first business day of each fiscal year, beginning with the fiscal year ending December 31, 2014 and continuing until, and including, the fiscal year ending December 31, 2023, equal to the lowest of 2,542,372 shares of the Company's common stock, 4% of the number of shares of the Company's common stock outstanding on the first day of the fiscal year and an amount determined by its board of directors. The Company's employees, officers, directors, consultants and advisors are eligible to receive awards under the 2013 Plan. However, incentive stock options may only be granted to employees of the Company.

In connection with the evergreen provisions of the 2013 Plan, the number of shares available for issuance under the 2013 Plan was increased by approximately 1,257,000 shares, effective as of January 1, 2014, by approximately 1,360,000 shares, effective as of January 1, 2015, and by approximately 1,408,000 shares, effective as of January 1, 2016. As of March 31, 2016, the Company had approximately 1,753,000 shares available for grant under the 2013 Plan.

A summary of the stock option activity, weighted average exercise prices, options outstanding and exercisable as of March 31, 2016 is as follows:

	Common Stock Options		Weighted Average Exercise Price
Outstanding, December 31, 2015	3,009	$	30.43
Granted	549	$	69.56
Exercised	(61)	$	12.53
Expired or forfeited	(2)	$	41.39
Outstanding, March 31, 2016	3,495	$	36.88
Options exercisable at March 31, 2016			1,202
Weighted average grant date fair value (per share) of options granted during the period		$	43.97

As of March 31, 2016, there were approximately 3,249,000 options outstanding, net of estimated forfeitures, that had vested or are expected to vest. The weighted-average exercise price of these options was $36.93 per option; the weighted-average remaining contractual life of these options was 7.8 years; and the aggregate intrinsic value of these options was approximately $34.6 million. A summary of the stock options outstanding and exercisable as of March 31, 2016 is as follows:

Table of Contents

	As of March 31, 2016					
	Options Outstanding			Options Exercisable		
Range of Exercise Prices	Total Options Outstanding	Weighted Average Remaining Life (Years)	Weighted Average Exercise Price	Number Exercisable		Weighted Average Exercise Price
$0.12-$10.03	400	6.3	$ 7.55	208	$	5.39
$10.04-$20.00	333	6.4	$ 13.46	159	$	13.51
$20.01-$30.00	161	7.6	$ 25.61	86	$	25.56
$30.01-$40.00	1,268	7.1	$ 33.27	597	$	33.25
$40.01-$55.00	790	9.0	$ 45.62	148	$	45.84
$55.01-$73.22	543	9.7	$ 71.89	4	$	73.22
	3,495	7.8	$ 36.88	1,202	$	26.95
Aggregate Intrinsic Value	$ 37,739			$ 19,086		

Cash proceeds from, and the aggregate intrinsic value of, stock options exercised during the three months ended March 31, 2016 and 2015, respectively, were as follows:

	Three months ended March 31,	
	2016	2015

Cash proceeds from options exercised	$ 768	$ 1,760
Aggregate intrinsic value of options exercised	$ 2,216	$ 10,368

In connection with stock option awards granted to employees, the Company recognized approximately $6.1 million and $3.9 million in share-based compensation expense during the three months ended March 31, 2016 and 2015, respectively, net of expected forfeitures. As of March 31, 2016, there were approximately $49.8 million of unrecognized compensation costs, net of estimated forfeitures, related to stock option awards granted to employees, which are expected to be recognized over a remaining weighted average period of 2.9 years.

In connection with stock options awards granted to consultants, the Company recognized approximately $0.4 million and $0.7 million in share-based compensation expense during the three months ended March 31, 2016 and 2015, respectively, net of expected forfeitures. As of March 31, 2016, there were approximately $2.1 million of unrecognized compensation costs, net of estimated forfeitures, related to stock option awards granted to consultants, which are expected to be recognized over a remaining weighted average period of 1.7 years.

The following table presents a summary of the Company's outstanding RSU awards granted as of March 31, 2016:

	Restricted Stock Units	Weighted Average Grant-Date Fair Value
Outstanding, December 31, 2015	288	$ 44.54
Awarded	148	$ 73.22
Vested	(33)	$ 45.60
Forfeited	1	$ 47.24
Outstanding, March 31, 2016	404	$ 54.99

As of March 31, 2016, there were approximately 368,000 RSUs outstanding, net of estimated forfeitures, that are expected to vest. The weighted-average fair value of these RSUs was $54.99 and the aggregate intrinsic value of these RSUs was approximately $15.5 million.

In connection with RSUs granted to employees, the Company recognized approximately $1.8 million and $0.5 million in share-based compensation expense during the three months ended March 31, 2016 and 2015, respectively, net of expected forfeitures. As of March 31, 2016, there were approximately $16.7 million of unrecognized compensation costs, net of estimated forfeitures,

Table of Contents

related to RSUs granted to employees, which are expected to be recognized over a remaining weighted average period of 3.4 years. The total fair value of the RSUs that vested during the three months ended March 31, 2016 was $1.5 million.

10. Property and Equipment

Property and equipment as of March 31, 2016 and December 31, 2015 were as follows:

	Useful Life (Years)	March 31, 2016	December 31, 2015
Manufacturing and clinical equipment	7-10	$ 617	$ 617
Computer and other office equipment	5	1,479	944
Furniture and fixtures	7	738	738
Leasehold improvements	3-5	1,551	1,551
Construction-in-progress		42	515
		4,427	4,365
Accumulated depreciation		(1,077)	(899)
Property and equipment, net		$ 3,350	$ 3,466

For the three months ended March 31, 2016 and 2015, depreciation expense was $178 thousand and $56 thousand, respectively.

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

The following discussion and analysis of our financial condition and results of operations should be read 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, 2015 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 26, 2016. 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® (pegpleranib), 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® (ranibizumab), have completed patient enrollment for two Phase 3 clinical trials of Fovista administered in combination with Lucentis and expect to complete enrollment in a third Phase 3 clinical trial evaluating Fovista in combination with Eylea® (aflibercept) or Avastin® (bevacizumab) in 2016. We have completed enrollment in two additional Phase 2a clinical trials of Fovista administered in combination with anti-VEGF drugs (Lucentis, Eylea or Avastin), one of which is studying the

potential of Fovista to reduce subretinal fibrosis in wet AMD patients and the other of which is investigating the optimized regimen of Fovista in combination with anti-VEGF drugs, as well as the potential of Fovista to reduce the treatment burden for wet AMD patients. We are also developing our product candidate Zimura® (avacincaptad pegol) as a monotherapy for the treatment of patients with geographic atrophy, or GA, a form of dry AMD, as well as in combination with anti-VEGF drugs for the treatment of wet AMD and for the treatment of polypoidal choroidal vasculopathy, a specific type of wet AMD, in patients who do not respond adequately to anti-VEGF monotherapy or for whom anti-VEGF monotherapy fails. We are also investigating the potential of an ophthalmic formulation for tivozanib, a small molecule VEGF tyrosine kinase inhibitor for which we have an option for a license.

Fovista Phase 3 Clinical Program

Our pivotal Phase 3 clinical program for Fovista consists of three separate Phase 3 clinical trials to evaluate the safety and efficacy of 1.5 mg of Fovista administered in combination with anti-VEGF drugs for the treatment of wet AMD compared to anti-VEGF monotherapy. Two of these trials, referred to as the Fovista Phase 3 Lucentis Trials, are evaluating Fovista in combination with Lucentis compared to Lucentis monotherapy. The third trial, referred to as the Fovista Phase 3 Eylea/Avastin Trial, is evaluating Fovista in combination with Eylea or Avastin compared to Eylea or Avastin monotherapy. Our development strategy for Fovista is to be agnostic with respect to the choice of the anti-VEGF drug administered in combination with Fovista.

Table of Contents

We completed patient enrollment in one of the Fovista Phase 3 Lucentis Trials in May 2015 and in the other Fovista Phase 3 Lucentis Trial in November 2015. The Fovista Phase 3 Lucentis Trials are investigating Fovista in combination with Lucentis compared to Lucentis monotherapy and are identical with respect to the trial design in the first year. Therefore, the databases from both of the Fovista Phase 3 Lucentis Trials will be locked and analyzed together, which will allow for the pooled analysis of certain relevant endpoints in accordance with the statistical analysis plan. We expect initial, top-line data from both of the Fovista Phase 3 Lucentis Trials to be available during the fourth quarter of 2016.

We are continuing to actively enroll patients in the Fovista Phase 3 Eylea/Avastin Trial and expect to complete enrollment in 2016, with initial, top-line data from this trial to be available in 2017 based on current enrollment estimates. This trial is investigating Fovista in combination with either Eylea or Avastin compared to Eylea or Avastin monotherapy. Our Phase 2b trial utilized Lucentis as the only anti-VEGF drug because Eylea was not yet approved and Avastin's non-inferiority status compared to Lucentis was not yet established at the time the Phase 2b clinical trial commenced. Therefore, in order to gain more experience with Fovista when administered in combination with Eylea or Avastin prior to starting a pivotal Phase 3 clinical trial, the Fovista Phase 3 Eylea/Avastin Trial started later (May 2014) than the Fovista Phase 3 Lucentis Trials (August 2013). This time period of approximately nine months allowed us to perform initial preclinical and clinical assessments and ensure compatibility of Eylea or Avastin when administered in combination with Fovista.

Our key objective and plan is to make Fovista commercially available to physicians to treat their patients with wet AMD as quickly as possible, subject to a favorable data outcome from the Phase 3 clinical program. We are continuing to explore various regulatory filing options. We plan to initially submit a New Drug Application, or NDA, to the U.S. Food and Drug Administration, or FDA, for Fovista in combination with Lucentis based upon data from the two Fovista Phase 3 Lucentis Trials and subsequently submit an amendment to the NDA with data from the Fovista Phase 3 Eylea/Avastin Trial, subject to a favorable data outcome from these trials. Alternatively, we may choose to file a supplemental NDA for Fovista in combination with Eylea or Avastin following FDA review of the NDA for Fovista in combination with Lucentis.

Fovista Expansion Studies

In addition to our ongoing Phase 3 clinical program for Fovista, we have initiated additional clinical trials to evaluate the potential additional benefits of Fovista administered in combination with anti-VEGF drugs in wet AMD patients. We refer to these trials collectively as the Fovista Expansion Studies. They include:

- *OPH1005 Fovista Anti-Fibrosis Study.* During the third quarter of 2014, we initiated an open-label Phase 2a clinical trial of 1.5 mg of Fovista administered in combination with anti-VEGF drugs (Lucentis, Eylea or Avastin), to study subretinal fibrosis in wet AMD patients. We completed enrollment in this trial in May 2015 with a total of 101 patients enrolled. Patients in this trial are followed over a 24-month period.

- *OPH1006 Fovista Treatment Burden Reduction Study.* During the fourth quarter of 2014, we initiated an open-label Phase 2a clinical trial of 1.5 mg of Fovista administered in combination with anti-VEGF drugs (Lucentis, Eylea or Avastin) to investigate the potential of Fovista to reduce the treatment burden for wet AMD patients. We completed enrollment in this trial in October 2015 with a total of 64 patients enrolled. Patients in this trial are followed over an 18-month period.

- *OPH1007 Fovista in Combination with Avastin Discontinuous Regimen Study.* During the fourth quarter of 2015, we initiated a randomized, double-masked, controlled Phase 2b clinical trial to evaluate the safety and efficacy of a discontinuous, bi-monthly regimen of 1.5 mg of Fovista administered in combination with Avastin during the maintenance phase of wet AMD treatment compared to a discontinuous, bi-monthly regimen of Avastin monotherapy.

- *OPH1008 Fovista Imaging Study.* During the fourth quarter of 2015, we initiated an open-label Phase 2a clinical trial to investigate the role of multi-modal imaging in assessing anatomic responses to various wet AMD treatment regimens of Fovista administered in combination with anti-VEGF drugs (Lucentis, Eylea or Avastin).

We may in the future seek to pursue additional clinical trials to assess the potential therapeutic benefit of Fovista in wet AMD as well as other ophthalmic conditions.

Novartis Agreement

In May 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, or API, supplied by us,

standalone Fovista products and products combining Fovista with an anti-VEGF drug 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 drug 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. Under the terms 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-based milestones for our Phase 3 clinical program for Fovista, $50.0 million of which we achieved in September 2014 and received in October 2014 and $50.0 million of which we achieved in March 2015 and received in April 2015, and up to an aggregate of an additional $300.0 million upon achievement of specified regulatory milestones, including marketing approval and 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.

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 drug to which Novartis has rights in the Novartis Territory, for use in the Phase 3 trials already initiated and in other Phase 2 and Phase 3 clinical trials in the Novartis Territory initiated 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.

In November 2015, we were informed by Novartis that Genentech, Inc., a Roche wholly-owned subsidiary, elected to exercise its option to participate in the financial arrangements relating to Novartis' rights under the Novartis Agreement. Roche's option originated from a pre-existing agreement between Roche and Novartis. The ex-U.S. commercialization agreement between Ophthotech and Novartis and its financial terms remained unchanged as a result of the exercise of the opt-in right. We continue to retain sole rights to Fovista in the United States.

Zimura Clinical Development

Currently, we have the following ongoing clinical trials for Zimura:

- *Zimura Phase 2/3 GA Study.* During the fourth quarter of 2015, we initiated, a randomized, double-masked, controlled Phase 2/3 clinical trial to evaluate the safety and efficacy of Zimura monotherapy in patients with GA. We plan to enroll approximately 300 patients in the initial stage of the trial. During this stage, patients will be randomized into three groups, and will receive monthly injections of 1.0 mg of Zimura per eye, monthly injections of 2.0 mg of Zimura per eye or monthly sham injections as the control arm. At month 18, we plan to conduct an interim analysis to assess the safety and efficacy of Zimura compared to sham. Upon review of this interim analysis, a determination will be made whether to continue the trial and whether to expand the trial by enrolling additional patients. Patients in the trial will receive monthly injections for 24 months.

- *Zimura Phase 2a Wet AMD Study.* During the fourth quarter of 2015, we initiated an open-label Phase 2a clinical trial to evaluate Zimura's potential role when administered in combination with anti-VEGF drugs (Lucentis, Eylea or Avastin) for the treatment of wet AMD.

- *Zimura PCV Study.* In late 2014, we commenced a very small, open-label Phase 2 clinical trial investigating Zimura's potential role when administered in combination with anti-VEGF drugs for the treatment of polypoidal choroidal vasculopathy, or PCV, a specific type of wet AMD, in patients who do not respond adequately to treatment with

anti-VEGF monotherapy or for whom anti-VEGF monotherapy fails. Our initial, preliminary analysis of the data from this trial has not revealed any safety concerns related to Zimura.

Overview of Funding History and Requirements

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. As of March 31, 2016, we had an accumulated deficit of $441.8 million. Our net loss was $36.3 million for the three months ended March 31, 2016, and $105.7 million for the year ended December 31, 2015, and we expect to continue to incur significant operating losses in 2016 and potentially 2017. We have not generated any revenues from product sales and have financed our operations primarily through private placements of our preferred stock, venture debt borrowings, funding under our royalty purchase and sale agreement with Novo A/S, which we refer to as the Novo Agreement, our initial public offering of common stock, which we closed in September 2013, our follow-on public offering of common stock, which we closed in February 2014, and funds we received under the Novartis Agreement. 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 the follow-on public offering of $55.4 million, after deducting

underwriting discounts and commissions and other offering expenses payable by us. We have received $125.0 million of funding under the Novo Agreement, which constitutes the full amount of funding under that agreement. We also received an upfront payment of $200.0 million from Novartis upon the execution of the Novartis Agreement and enrollment-based milestone payments of $50.0 million in October 2014 and $50.0 million in April 2015.

We expect our expenses to continue to increase, particularly as we continue the development of Fovista in our Phase 3 clinical program, as we further evaluate the potential benefit of Fovista when administered in combination with anti-VEGF drugs in wet AMD patients through the Fovista Expansion Studies, and potentially in other ophthalmic diseases and conditions with unmet medical need, and as we pursue the development of Zimura through our Zimura development programs. We expect our expenses to increase as we initiate additional trials and as patient enrollment increases in trials that have already commenced. We also expect our expenses to increase as we manufacture validation batches of API and drug product for Fovista. In addition, our expenses will increase prior to obtaining marketing approval for Fovista as we expand our commercial infrastructure and build-up our Fovista API supply to support the anticipated launch of Fovista. Furthermore, 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. We are party to agreements, specifically a divestiture agreement with OSI (Eyetech), Inc., which agreement is now held by OSI Pharmaceuticals, LLC, a subsidiary of Astellas US, LLC, and license agreements with Archemix Corp. and Nektar Therapeutics, that impose significant milestone payment obligations on us in connection with our achievement of specific clinical, regulatory and commercial milestones with respect to Fovista and Zimura. We are also exploring the potential of an ophthalmic formulation for tivozanib, a small molecule VEGF tyrosine kinase inhibitor for which we have an option to obtain a license, and expect our expenses to increase as we continue the preclinical development of this compound, including in the event we elect to exercise our option or in the event we trigger certain milestone payment obligations. Furthermore, we are incurring and expect to continue to incur costs associated with hiring additional personnel and expanding our facilities. See "—Liquidity and Capital Resources—Funding Requirements" for a discussion of factors affecting our future capital requirements.

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 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 or technologies. 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 Operations Overview

Revenue

Prior to 2014, we had not generated any revenue. In May 2014, we received an upfront payment of $200.0 million in connection with our entry into the Novartis Agreement, which has not been recorded as revenue due to certain contingencies associated with the payment. In each of September 2014 and March 2015, we achieved a $50.0 million enrollment-based milestone, or

Table of Contents

$100.0 million in the aggregate, under the Novartis Agreement. We use the relative selling price method to allocate arrangement consideration to our performance obligations under the Novartis Agreement. Below is a summary of the components of our collaboration revenue for the three months ended March 31, 2016 and 2015:

	Three months ended March 31,	
	2016	2015
	(in thousands)	
License revenue	$ —	$ 38,083
Research and development activity revenue	1,275	3,585
API transfer revenue	14,443	—
Joint operating committee revenue	3	10
Total collaboration revenue	$ 15,721	$ 41,678

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, payments for manufactured material 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 product sales until the end of 2017 at the earliest. If we fail to complete the development of Fovista, Zimura or other product candidates we may develop in a timely manner or obtain regulatory approval for them, our ability to generate future revenue and our results of operations and financial position, would be materially adversely affected.

Research and Development Expenses

Research and development expenses primarily consist of costs associated with the development and clinical testing and manufacturing of Fovista and Zimura, as well as costs associated with the preclinical development of other product candidates and formulations. 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 and contract manufacturing organizations, or CMOs, for the production of API and drug product; and

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

Research and development expenses 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 expenses are charged to operations as incurred in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic, or ASC, 730, *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 months ended March 31, 2016 and 2015:

Table of Contents

	Three months ended March 31,	
	2016	2015
	(in thousands)	
Fovista	$ 24,611	$ 15,939
Zimura	1,266	1,503
Personnel-related	5,198	3,480
Share-based compensation	4,685	3,115
Other	2,010	520
	$ 37,770	$ 24,557

We anticipate that we will incur significant research and development expenses in connection with conducting our pivotal Phase 3 clinical program for Fovista and, if such trials are successful, seeking marketing approval for Fovista. We also expect that our research and development expenses will increase as we further evaluate the potential benefit of Fovista when administered in combination with anti-VEGF drugs in wet AMD patients through our Fovista Expansion Studies, and potentially, in other ophthalmic diseases and conditions with unmet medical need, and as a result of the pursuit of our Zimura development programs. We expect these research and development expenses to increase as we initiate additional trials and as patient enrollment increases in trials that have already commenced. In addition, we expect that we will incur significant expenses related to manufacturing validation activities associated with Fovista and process development and manufacturing scale-up and validation activities associated with Zimura.

Our expenses may exceed our expectations if we experience delays in enrollment or with the availability of drug supply for our clinical trials. Our expenses may also exceed our expectations if we increase our investigator fees for our clinical trials or if we further expand the scope of our clinical trials and programs, including, for example, by increasing the number of clinical trial sites or changing the geographic mix of sites at which patients are enrolled. Our costs may also exceed our expectations for other reasons, for example, if we experience issues with manufacturing validation, process development, the scale-up of manufacturing activities or activities to enable and qualify second source suppliers or if we decide to increase licensing or preclinical research and development activities.

Our current Phase 3 clinical program for Fovista is expected to continue into 2018, and we expect to incur substantial expenditures to complete the Phase 3 clinical program after the receipt of initial, top-line data, which we expect to be available during the fourth quarter of 2016 for the two Fovista Phase 3 Lucentis Trials and during 2017 for the Fovista Phase 3 Eylea/Avastin Trial based on current enrollment estimates. Furthermore, 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 manufacturing validation activities associated with Fovista, process development and manufacturing scale-up and validation activities associated with 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.

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

- the scope, rate of progress and expense of our research and development activities;
- the potential benefits of our product candidates over other therapies;
- clinical trial results;
- the terms and timing of regulatory approvals;
- our ability, together with any commercialization partner’s ability, to market, commercialize and achieve market acceptance for any of our product candidates; 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 that we currently anticipate

Table of Contents

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 clinical development.

See the "Liquidity and Capital Resources" section on page 32 of this Quarterly Report on Form 10-Q for more information regarding our current and future financial resources and our expectations regarding our research and development expenses and funding requirements.

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, commercial and business development functions. Other general and administrative expenses include facility costs and professional fees for legal, patent, pre-launch commercialization activities, travel expenses, consulting and accounting services.

We anticipate that our general and administrative expenses will increase in future periods to support increases in our research and development, manufacturing, and commercialization activities and as a result of increased personnel, including management personnel to support our research and development, 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 money market funds, U.S. Treasury securities and investment-grade corporate debt 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.

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, CMOs and other vendors in connection with research and development and manufacturing activities.

We base our expenses related to CROs and CMOs on our estimates of the services received and efforts expended pursuant to quotations and contracts with such vendors that conduct research and development and manufacturing 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 applicable research and development or manufacturing 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

Table of Contents

services or the level of effort varies from our estimate, we adjust the accrual or prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in us reporting amounts that are too high or too low in any particular period. There have been no material changes in estimates for the periods presented.

Revenue Recognition—Collaboration Revenue

Prior to 2014, we had not generated any revenue. In May 2014, we received an upfront payment of $200.0 million in connection with our entry into the Novartis Agreement, which has not been recorded as revenue due to certain contingencies associated with the payment. In each of September 2014 and March 2015, we achieved a $50.0 million enrollment-based milestone under the Novartis Agreement, or $100.0 million in the aggregate. We use the relative selling price method to allocate arrangement consideration to our performance obligations under the Novartis Agreement. Below is a summary of the components of our collaboration revenue for the three months ended March 31, 2016 and 2015:

	Three months ended March 31,	
	2016	2015
	(in thousands)	
License revenue	$ —	$ 38,083
Research and development activity revenue	1,275	3,585
API transfer revenue	14,443	—
Joint operating committee revenue	3	10
Total collaboration revenue	$ 15,721	$ 41,678

In the future, we may generate additional revenues from a combination of product sales and license fees, milestone payments, research and development activity-related payments, payments for manufactured material 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 preclinical, clinical or commercial material. Payments to us under these arrangements typically include one or more of the following: non-refundable, upfront license fees; option exercise fees; funding of research and/or development efforts; milestone payments; payments for manufactured material; and royalties on future product sales.

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

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

When management believes the license to our 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

Table of Contents

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 of an application for marketing approval of a product candidate or upon approval to market the product candidate by the FDA or other regulatory authorities. 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, we have concluded that the clinical and development milestones and certain reimbursement approval milestones are not substantive and that the marketing approval milestones are substantive. Milestone payments received that are not considered substantive are included in the allocable arrangement consideration and are recognized as revenue in proportion to the relative-selling price allocation established at the inception of the arrangement. Revenues from commercial milestone payments are accounted for as royalties and are recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met.

Royalty Purchase Liability

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

as a liability on our balance sheet. As we make royalty payments to Novo A/S in accordance with the Novo Agreement, we will reduce the liability balance. At the time that such royalty payments become probable and estimable, and if such amounts exceed the liability balance, we will impute interest accordingly on a prospective basis based on such estimates, which would result in a corresponding increase in the liability balance.

Share-Based Compensation

We account for all share-based compensation payments issued to employees, non-employee directors, and consultants by estimating the fair value of each equity award. 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 option method. In accordance with authoritative guidance, we re-measure the fair value of consultant 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 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. For grants containing performance-based vesting provisions, expense is recognized over the estimated achievement period. Calculating the fair value of share-based awards requires that we make highly subjective assumptions.

We use the Black-Scholes option pricing model to value our stock option awards. Use of this valuation methodology requires that we make assumptions as to the volatility of our common stock, the expected term of our stock options, and the risk-free interest rate for a period that approximates the expected term of our stock options and the expected dividend yield of our common stock. As a recent 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

Table of Contents

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 months ended March 31, 2016 and 2015:

	Three months ended March 31,	
	2016	2015
Expected common stock price volatility	71%	73%
Risk-free interest rate	1.47%-1.90%	1.35%-1.92%
Expected term of options (years)	6.2	6.2
Expected dividend yield	0%	0%

We estimate the fair value of restricted stock units, or RSUs, granted to employees using the closing market price of our common stock on the date of grant.

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 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.

Share-based compensation expense for equity grants to employees, non-employee directors and consultants was $8.3 million and $5.1 million for the three months ended March 31, 2016 and 2015, respectively. As of March 31, 2016, we had $68.6 million of total unrecognized share-based compensation expense, which we expect to recognize over a weighted-average remaining vesting period of approximately 3.0 years. We expect our share-based compensation for our equity awards to employees, non-employee directors and consultants to increase as a result of recognizing our existing unrecognized share-based compensation for awards that will vest and as we issue additional equity awards to attract and retain our employees.

For the three months ended March 31, 2016 and 2015, we allocated share-based compensation as follows:

	Three months ended March 31,	
	2016	2015
	(in thousands)	
Research and development	$ 4,685	$ 3,115
General and administrative	3,647	1,939
Total	$ 8,332	$ 5,054

Income Taxes

In 2014, we received $83.3 million from Novo A/S under the Novo Agreement, which was reported as revenue for income tax purposes. Also in 2014, we received $200.0 million from Novartis upon execution of the Novartis Agreement, a portion of which was reported as revenue for income tax purposes. In addition, we received a milestone payment of $50.0 million in 2014 from Novartis which was reported as revenue for income tax purposes. As a result of these payments, and after taking into account the utilization of our federal and state net operating loss carry-forwards and utilization of our research

and development tax credits, we reported taxable income for tax purposes in 2014. We made income tax payments of $40.2 million during the year ended December 31, 2014.

In 2015, we incurred losses for tax purposes. As of December 31, 2015, we had recorded net deferred tax assets of $23.1 million. We expect to realize these net deferred tax assets in 2016 due to our ability to carryback our 2015 federal tax loss to 2014. As

Table of Contents

such, we reclassified these amounts to income tax receivable on our Balance Sheet as of March 31, 2016. We expect to carry forward our 2015 state tax losses due to various state restrictions on the use of carryback claims. We are projecting tax losses for 2016. The deferred tax assets associated with these losses incurred to date in 2016 have a full valuation allowance recorded against them, however, due to our history of losses and the lack of other positive evidence to support future taxable income against which these losses could be applied. See Note 7 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.

Results of Operations

Comparison of Three Month Periods Ended March 31, 2016 and 2015

	Three months ended March 31,		
	2016	2015	Increase (Decrease)
	(in thousands)		
Statements of Operations Data:			
Collaboration revenue	$ 15,721	$ 41,678	$ (25,957)
Operating expenses:			
Research and development	37,770	24,557	13,213
General and administrative	14,696	9,584	5,112
Total operating expenses	52,466	34,141	18,325
Income (loss) from operations	(36,745)	7,537	(44,282)
Interest income	446	125	321
Other income (loss)	30	(52)	82
Income (loss) before income tax provision	(36,269)	7,610	(43,879)
Income tax provision	32	974	(942)
Net income (loss)	$ (36,301)	$ 6,636	$ (42,937)

Collaboration Revenue

Collaboration revenue for the three months ended March 31, 2016 was $15.7 million. Using the relative selling price method, we recognized $1.3 million to research and development activities performed under the Novartis Agreement, $14.4 million related to Fovista API we transferred to Novartis and a de minimis amount of revenue associated with our joint operating committee participation obligation.

Collaboration revenue for the three months ended March 31, 2015 was $41.7 million, of which $38.1 million was allocated to the license delivered to Novartis under the Novartis Agreement, $3.6 million was allocated to research and development activities performed under the Novartis Agreement and a de minimis amount of revenue associated with our joint operating committee participation obligation during the same period.

Research and Development Expenses

Our research and development expenses were $37.8 million for the three months ended March 31, 2016, an increase of $13.2 million compared to $24.6 million for the three months ended March 31, 2015. The increase in research and development expenses for the three months ended March 31, 2016 was primarily due to an $8.7 million increase in costs associated with our Fovista program, including our Fovista Phase 3 clinical program and our Fovista Expansion Studies. The increased costs for our Fovista program included higher clinical trial costs relating to increased patient enrollment in the Fovista Phase 3 clinical trials and the Fovista Expansion Studies, the initiation of additional Fovista Expansion Studies, as well as higher manufacturing costs to support our clinical trials and for API validation activities. Also contributing to the overall increase was a $1.6 million increase to share-based compensation costs, a $1.7 million increase to personnel expenses associated with additional research and development staffing and a $1.2 million increase in professional services and consulting fees.

General and Administrative Expenses

Our general and administrative expenses were $14.7 million for the three months ended March 31, 2016, an increase of $5.1 million compared to $9.6 million for the three months ended March 31, 2015. The increase was primarily due to an increase in personnel costs of $1.7 million, share-based compensation costs of $1.7 million, an increase of $1.4 million in facility costs, as well as other costs to support the expansion of our operations, including our public company infrastructure, and the early stages of a commercial organization. Also contributing to the increase were increased costs for pre-launch commercialization activities, professional services and consulting fees of $0.3 million.

Table of Contents

Interest Income

Interest income for the three months ended March 31, 2016 was $0.4 million compared to interest income of $0.1 million for the three months ended March 31, 2015. The increase in interest income earned during the three months ended March 31, 2016 was the result of a change in the mix of our

investment portfolio, which previously included only investments in U.S. Treasury securities and now includes investments in certain investment-grade corporate debt securities.

Provision for Income Taxes

The provision for income taxes recorded for the three months ended March 31, 2016 was $32 thousand and primarily related to state income taxes. For the three months ended March 31, 2015, we recorded a provision for income taxes of $974 thousand which primarily related to the $50.0 million enrollment-based milestone we achieved in March 2015.

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 of common stock, which we closed in September 2013, our follow-on public offering of common stock, which we closed in February 2014, and funds we received under the Novartis Agreement. In September 2013, we issued and sold an aggregate of 8,740,000 shares of common stock in our initial public offering at a public offering price of $22.00 per share. We received net proceeds from the initial public offering of $175.6 million. In February 2014, we issued and sold 1,900,000 shares of common stock and selling shareholders sold 728,571 shares of common stock in a follow-on public offering at a public offering price of $31.50 per share. We received net proceeds of $55.4 million from the follow-on offering. The Novo Agreement, which is described in more detail below, provided for financing of up to $125.0 million in the aggregate in return for the sale to Novo A/S of royalty interests in worldwide sales of Fovista. We received an aggregate of $125.0 million from this financing in separate tranches in May 2013, January 2014 and November 2014, which constitutes the full amount of funding under the Novo Agreement. In May 2013, we issued and sold an aggregate of 6,666,667 shares of our series C preferred stock at a price per share of $2.50, for an aggregate purchase price of $16.7 million. In August 2013, we issued and sold an aggregate of 13,333,333 additional shares of our series C preferred stock to the same purchasers at a price per share of $2.50, for an aggregate purchase price of $33.3 million.

In May 2014, we received an upfront payment of $200.0 million upon execution of the Novartis Agreement in connection with the grant of a license for the rights to commercialize Fovista 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-based milestones for our ongoing pivotal Phase 3 clinical program for Fovista, of which $50.0 million was received in October 2014 and $50.0 million was received in April 2015. In connection with the receipt of the upfront payment from Novartis, we made a milestone payment in June 2014 of approximately $19.8 million to Nektar Therapeutics. We are entitled to certain additional future payments from Novartis based on the continued clinical development, regulatory approval and commercial success of Fovista. See “Licensing and Commercialization Agreement with Novartis Pharma AG” below for further information.

Cash Flows

As of March 31, 2016, we had cash, cash equivalents and marketable securities totaling $356.1 million and no debt. We primarily invest our cash, cash equivalents and marketable securities in U.S. Treasury securities, money market funds and certain investment-grade corporate debt securities.

The following table shows a summary of our cash flows for the three months ended March 31, 2016 and 2015:

Table of Contents

	Three months ended March 31,	
	2016	2015
	(in thousands)	
Net cash (used in) provided by:		
Operating activities	$ (36,740)	$ (31,509)
Investing activities	(2,067)	29,503
Financing activities	768	1,760
Net change in cash and cash equivalents	$ (38,039)	$ (246)

Cash Flows from Operating Activities

Net cash used in operating activities for the three months ended March 31, 2016 was $36.7 million and relates primarily to net cash used to fund our Fovista Phase 3 program, our Fovista expansion studies, Fovista manufacturing activities, as well as manufacturing and clinical trial costs for our Zimura program and expenditures related to general and administrative expenses.

Net cash used in operating activities for the three months ended March 31, 2015 was $31.5 million and related primarily to our net income adjusted for non-cash charges and changes in the components of working capital, as well as our increased efforts to advance our Fovista Phase 3 program, including increased spending on clinical trial costs and manufacturing activity for Fovista.

We expect cash used in operating activities to continue to increase substantially compared to prior periods and for the foreseeable future for the reasons described below under “—Funding Requirements”.

Cash Flows from Investing Activities

Net cash used in investing activities for the three months ended March 31, 2016 was $2.1 million and relates primarily to proceeds from the maturities of marketable securities totaling $10.0 million offset by purchases of marketable securities totaling $12.0 million. Net cash provided by investing activities for the three months ended March 31, 2015 was $29.5 million, which related primarily to the purchase of marketable securities totaling $144.4 million, offset by maturities of marketable securities of $174.0 million.

Cash Flows from Financing Activities

Net cash provided by financing activities was $0.8 million for the three months ended March 31, 2016 and $1.8 million for the three months ended March 31, 2015 and related to proceeds from stock option exercises in each respective periods.

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, as we further evaluate the potential benefit of Fovista when administered in combination with anti-VEGF drugs in wet AMD patients through the Fovista Expansion Studies, and potentially in other ophthalmic diseases and conditions with unmet medical need, and as we pursue the development of Zimura through our Zimura development programs. We expect our expenses to increase as we initiate additional trials and as patient enrollment increases in trials that have already commenced. We also expect our expenses to increase as we manufacture validation batches of API and drug product for Fovista. In addition, our expenses will increase prior to obtaining marketing approval for Fovista as we expand our commercial infrastructure and build-up our Fovista API supply to support the anticipated launch of Fovista. Furthermore, 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. For Fovista, our ex-U.S. commercialization partner, Novartis, is responsible for these commercialization expenses outside the United States. We are party to agreements, specifically a divestiture agreement with OSI (Eyetech), Inc., which 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 and Zimura. For example, in connection with our entry into the Novartis Agreement, we made a milestone payment of $19.8 million to Nektar Therapeutics in June 2014. We are also exploring the potential of an ophthalmic formulation for tivozanib, a small molecule VEGF tyrosine kinase inhibitor for which we have an option to

Table of Contents

obtain a license, and expect our expenses to increase as we continue the preclinical development of this compound, including in the event we elect to exercise our option or in the event we trigger certain milestone payment obligations.

We expect that our expenses will further increase if and as we:

- undertake additional clinical development of Fovista, if it is approved, in support of our efforts to broaden the label for Fovista;
- conduct additional clinical trials of Zimura that may be required by regulatory authorities, including a second Phase 3 clinical trial for GA, to seek marketing approval for Zimura in any indication;
- continue to develop tivozanib for the treatment of ophthalmic diseases;
- in-license or acquire the rights to, and pursue research and development of, other complementary products, product candidates or technologies, including drug delivery technology, for the treatment of ophthalmic diseases;
- seek marketing approval for any product candidates that successfully complete clinical trials;
- hire additional clinical, manufacturing, quality control, quality assurance and scientific personnel;
- expand our outsourced manufacturing activities and establish sales, marketing and distribution capabilities, if we receive, or expect to receive, marketing approval for any product candidates;
- maintain, expand and protect our intellectual property portfolio; and
- add operational, financial and management information systems and personnel and expand our facilities.

As of March 31, 2016, we had cash, cash equivalents, and marketable securities of $356.1 million. We also had $365.6 million in total liabilities, $336.6 million of which related to the Novo Agreement and deferred revenue associated with the Novartis Agreement.

We believe that our cash, cash equivalents and marketable securities, together with the potential remaining $30.0 million enrollment-based milestone payment under the Novartis Agreement, will be sufficient to fund our operations and capital expenditure requirements as currently planned through the end of 2017.

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 capital requirements will depend on several 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 or technologies. For example, our expenses may exceed our expectations if we experience delays in enrollment or with the availability of drug supply for our clinical trials. Our expenses may also exceed our expectations if we increase our investigator fees for our clinical trials, if we further expand the scope of our clinical trials and programs, including, for example, by increasing the number of clinical trial sites or changing the geographic mix of sites at which patients are enrolled. Our costs may also exceed our expectations for other reasons, for example, if we experience issues with manufacturing validation, process development, the scale-up of manufacturing activities or activities to enable and qualify second source suppliers or if we decide to increase licensing or preclinical research and development activities or corporate staffing. 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. As a result, we may need or may seek to obtain additional funding in connection with our continuing operations prior than expected.

Moreover, our current Phase 3 clinical program for Fovista is expected to continue into 2018, and we expect to incur substantial expenditures to complete the Phase 3 clinical program after the receipt of initial, top-line data, which we expect to be available during the fourth quarter of 2016 for the two

Fovista Phase 3 Lucentis Trials and during 2017 for the Fovista Phase 3 Eylea/Avastin Trial, based on current enrollment estimates. Furthermore, we expect the clinical development for 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 manufacturing validation activities associated with Fovista, process development and manufacturing scale-up and validation activities associated with 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 any other product candidate we may develop.

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

Table of Contents

- the scope, progress, costs and results of our Phase 3 clinical program for Fovista;
- the scope, progress, costs and results of the Fovista Expansion Studies to further evaluate the potential benefit of Fovista in wet AMD when administered in combination with anti-VEGF drugs, and potentially in other ophthalmic diseases and conditions with unmet need;
- the scope, progress, costs and results of our Zimura clinical programs, including our Zimura Phase 2/3 GA Study and our Zimura Phase 2a Wet AMD Study, as well as any additional clinical trials (including a potential second Phase 3 trial for GA) required by regulatory authorities for us to seek marketing approval for Zimura in any indication;
- the costs and timing of manufacturing validation activities associated with Fovista;
- the costs and timing of process development and manufacturing scale-up and validation activities associated with Zimura;
- the costs, timing and outcome of regulatory reviews 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 outsourced manufacturing activities, including activities to build up our commercial drug supply and to enable and qualify second source suppliers, expanding our commercial operations 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 royalty payments that we will be obligated to make;
- the scope, progress and results of our preclinical studies, formulation development and clinical development plans for tivozanib;
- the scope, progress, results and costs of preclinical development and/or clinical trials for any other product candidates that we may develop;
- our ability to establish collaborations on favorable terms, if at all;
- the extent to which we in-license or acquire rights to, and develop, complimentary products, product candidates or technologies, including drug delivery technologies; and
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending intellectual property-related claims.

We do not have any committed external source of funds other than the Novartis Agreement. The remaining potential milestone payments under the Novartis Agreement are subject to our achievement of specified clinical, regulatory and commercial events related to Fovista, none of which can be assured. Our future commercial revenues, if any, will be derived from sales of Fovista, Zimura or any other products that we are able to successfully develop, which, depending on the product, may not be available for several years, if at all. In addition, if approved, Fovista or Zimura or any product that we acquire or in-license may not achieve commercial success. If that is the case, we may need to obtain substantial additional financing to achieve our business objectives. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

Adequate additional financing may not be available to us on acceptable terms, or at all. If we are unable to raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts.

Until such time, if ever, as we can generate substantial product revenues, we may need or may seek to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. 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. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our existing 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

Table of Contents

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 grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves.

Licensing and Commercialization Agreement with Novartis Pharma AG

In May 2014, we entered into a licensing and commercialization agreement with Novartis Pharma AG, which we refer to as the Novartis Agreement. Under the Novartis Agreement, we granted Novartis exclusive rights under specified patent rights, know-how and trademarks controlled by us to manufacture, from bulk API supplied by us, standalone Fovista products and products combining Fovista with an anti-VEGF drug 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 drug to which Novartis has rights, as well as a pre-filled syringe presentation of such products and to use commercially reasonable efforts, subject to obtaining marketing approval, to commercialize licensed products in the Novartis Territory in accordance with agreed development and marketing plans. Novartis has also granted us options, subject to specified limitations, and to the extent such rights are controlled by Novartis, to obtain exclusive rights from Novartis to develop and commercialize in the United States the co-formulated and pre-filled syringe products developed by Novartis. We and Novartis have each granted the other options, subject to specified limitations, to obtain access to study data from certain clinical trials of licensed products that we or Novartis may conduct, including for use by the other in regulatory filings in its territory. We have agreed to exclusively supply Novartis, and Novartis has agreed to exclusively purchase from us, its clinical and commercial requirements for the bulk API for 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 a $200.0 million upfront fee upon execution of the Novartis Agreement. Under the terms 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-based milestones for our Phase 3 clinical program for Fovista, $50.0 million of which we received in October 2014 and $50.0 million of which we received in April 2015, and up to an aggregate of an additional $300.0 million upon achievement of specified regulatory milestones, including marketing approval and 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 is also 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 for clinical supplies and our manufacturing costs plus a specified percentage margin for commercial supplies of the bulk API for Fovista that we supply to Novartis. If we or Novartis exercise our or its respective rights to obtain access to study data from clinical trials conducted by the other party, the party exercising the option will be obligated to pay the other party's associated past development costs and share with such other party any future associated development costs. If we exercise our option to obtain Novartis-controlled rights to develop, manufacture and commercialize any co-formulated Fovista product in the United States, we will be obligated to pay a specified percentage of Novartis's associated past development costs and share with Novartis any future associated development costs. Novartis and we 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 drug to which Novartis has rights in the Novartis Territory, for use in the Phase 3 clinical trials already initiated and in other Phase 2 and Phase 3 clinical trials in the Novartis Territory initiated 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

Table of Contents

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 Novartis or us, will expire upon the expiration of Novartis's obligation to pay us royalties on net sales of licensed products. Novartis and we 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 us, 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 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 Novartis 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. 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 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 we elect to terminate the Novartis 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 Novartis 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.

Manufacturing and Supply Agreements with Agilent Technologies, Inc.

Clinical API Supply Agreement

In May 2014, we entered into a Clinical Manufacturing and Supply Agreement with Agilent Technologies, Inc., or Agilent, 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 Fovista API. The clinical supply agreement has an initial five-year term, which is subject to automatic renewal absent termination by either party in accordance with the terms of the clinical supply agreement. The clinical supply agreement provides for pricing for Fovista API structured on a tiered basis with the price reduced as the volume ordered increases. We may terminate the clinical supply agreement or any statement of work thereunder upon 12 months prior written notice to Agilent and Agilent may terminate the clinical supply agreement if we do not, over a specified period, purchase and take delivery from Agilent of a specified minimum quantity of API for Fovista. Each party also has the right to terminate the clinical supply agreement for other customary reasons such as material breach and bankruptcy. The clinical supply 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.

Commercial API Supply Agreement

In September 2015, we entered into a Commercial Manufacturing and Supply Agreement with Agilent, 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

commercial requirements in all jurisdictions of Fovista API. The commercial supply agreement has an initial term that runs for seven years from the date of our first commercial sale of Fovista, and which is subject to one two-year automatic renewal period, absent termination by either party in accordance with the terms of the commercial supply agreement. The commercial supply agreement provides for pricing for Fovista API structured on a tiered basis, with the price reduced as the volume of Fovista API ordered increases. We may cancel any purchase order under the commercial supply agreement at any time, subject to the payment of specified cancellation fees. We may terminate the commercial supply agreement with no financial penalty in the event that we cannot commercialize Fovista due to regulatory or other medical, scientific or legal reasons. Agilent may terminate the commercial supply agreement in the event that we do not, over a specified period, purchase and take delivery from Agilent of a specified minimum quantity of Fovista API. Each party also has the right to terminate the commercial supply agreement for other customary reasons such as material breach and bankruptcy. The commercial supply agreement contains provisions relating to compliance by Agilent with current Good Manufacturing Practices, cooperation by Agilent in connection with marketing applications for Fovista, indemnification, confidentiality, dispute resolution and other customary matters for an agreement of this kind.

Financing Agreement with Novo A/S

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

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

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

The Novo Agreement requires the establishment by Novo A/S and us 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 March 31, 2016:

	Payments Due by Period				
	Total	Less than 1 year	1-3 years	3-5 years	More than 5 years
			(in thousands)		
Operating Leases (1)	$ 10,646	$ 2,200	$ 7,300	$ 1,146	$ —
Purchase Obligations (2)	20,813	20,813	—	—	—
Total (3)	$ 31,459	$ 23,013	$ 7,300	$ 1,146	$ —

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

Table of Contents

(2) Purchase obligations represent our commitments under purchase orders, including those made under our clinical and commercial supply agreements with Agilent.

(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) anticipated expenditures under supply agreements for periods for which we are not yet bound under binding purchase orders, (d) contracts that are entered into in the ordinary course of business that are not material in the aggregate in any period presented above and (e) our royalty purchase liability of $125.0 million as of March 31, 2016, due to the fact that the royalty payment period, if any, is not known.

In addition to the amounts set forth in the table above, we may be required, under various agreements, to pay royalties and make milestone payments. In addition to the Novo Agreement, these agreements include the following:

- Under our divestiture 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 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, the extent of patent coverage for the licensed product and whether we have granted a third-party commercialization rights to the licensed product. In June 2014, we paid Nektar $19.8 million in connection with our entry into the Novartis Agreement.

- Under an option agreement with AVEO Pharmaceuticals relating to tivozanib, we will be obligated to make milestone payments of $2.0 million upon the submission of an Investigational New Drug Application to the FDA and $6.0 million upon the earlier of demonstration of proof of concept in humans and a specified date in January 2017, subject to any exercise by us of our right to terminate the option agreement.

We also have letter agreements with certain employees that require the funding of a specific level of payments, if certain events, such as a termination of employment in connection with a change in control or termination of employment by the employee for good reason or by us without cause, occur. For a description of these obligations, see our definitive proxy statement on Schedule 14A for our 2016 annual meeting of stockholders, as filed with the SEC on April 29, 2016.

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 and CMOs represent significant costs in clinical development. Subject to required notice periods and our obligations under binding purchase orders, we can elect to

Table of Contents

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 $356.1 million as of March 31, 2016, consisting of cash, investments in money market funds and certain investment-grade corporate debt 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 CMOs 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 March 31, 2016, 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 March 31, 2016. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Based on the evaluation of our disclosure controls and procedures as of March 31, 2016, 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

Effective January 1, 2016, we implemented a new enterprise resource planning system (“ERP”) that materially affected our internal control over financial reporting. In connection with implementation of the ERP system, we are updating our internal control over financial reporting, as necessary, to accommodate modifications to our business processes and accounting procedures. Although our internal control over financial reporting has been materially affected by the implementation of the ERP system, we do not believe that the implementation of the ERP system will have an adverse effect on our internal control over financial reporting.

Except as described in the preceding paragraph, we determined that there were no other changes in our internal control over financial reporting during the quarter ended March 31, 2016 that have materially affected, or are 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.

Table of Contents

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 risks and uncertainties we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. Please see page 1 of this 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

Our limited 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 focused on organizing and staffing our company, acquiring rights to product candidates, business planning, raising capital and developing Fovista, Zimura and other product candidates. We have not yet demonstrated our ability to successfully complete a large-scale, pivotal clinical trial, obtain marketing approval, manufacture at commercial scale, or arrange

for a third party to do so on our behalf, or conduct sales, marketing and distribution activities necessary for successful product commercialization. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history.

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

We have incurred significant operating losses since our inception. We expect to continue to incur losses until such time we successfully commercialize our product candidates and may never achieve or maintain profitability.

Since inception, we have experienced significant cash outflows in funding our operations. As of March 31, 2016, we had an accumulated deficit of $441.8 million. Our net loss was $36.3 million for the three months ended March 31, 2016, and $105.7 million for the year ended December 31, 2015 and we expect to continue to incur significant operating losses in 2016 and potentially 2017. To date, we have not generated any revenues from product sales and have financed our operations primarily through private placements of our preferred stock, venture debt borrowings, funds received under our royalty purchase and sale agreement with Novo A/S, which we refer to as the Novo Agreement, which we entered into in May 2013, 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 have devoted substantially all of our financial resources and efforts to the research and development of Fovista and Zimura and preparations for the potential commercial launch of Fovista, including manufacturing scale-up activities. We expect to continue to incur significant expenses and increasing operating losses over the next few 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, as we further evaluate the potential benefit of Fovista when administered in combination with anti-VEGF drugs in wet AMD patients through the Fovista Expansion Studies, and potentially in other ophthalmic diseases and conditions with unmet medical need, and as we pursue the development of Zimura through our Zimura development programs. We expect our expenses to increase as we initiate additional trials and as patient enrollment increases in trials that have already commenced. We also expect our expenses to increase as we manufacture validation batches of API and drug product for Fovista. In addition, our expenses will increase prior to obtaining marketing approval for Fovista as we expand our commercial infrastructure and build-up our Fovista API supply to support the anticipated launch of Fovista. Furthermore, 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. For Fovista, our ex-U.S. commercialization partner Novartis is responsible for these commercialization expenses outside the United States. We are party to agreements, specifically a divestiture agreement with OSI (Eyetech), Inc., which agreement is now held by OSI Pharmaceuticals, LLC, a subsidiary of Astellas US, LLC, and license agreements with Archemix Corp., or Archemix, and Nektar Therapeutics, or Nektar, that impose significant milestone payment obligations on us in connection with our achievement of specific clinical, regulatory and commercial milestones with respect to Fovista and Zimura. For example, in connection with our entry into the Novartis Agreement, we made a milestone payment of $19.8 million to Nektar in June 2014. We are also exploring the potential of an ophthalmic formulation for tivozanib, a small molecule VEGF tyrosine kinase inhibitor for which we have an option to obtain a

Table of Contents

license, and expect our expenses to increase as we continue the preclinical development of this compound, including in the event we elect to exercise our option or in the event we trigger certain milestone payment obligations.

We expect that our expenses will further increase if and as we:

- undertake additional clinical development of Fovista, if it is approved, in support of our efforts to broaden the label for Fovista;
- conduct additional clinical trials of Zimura that may be required by regulatory authorities, including a second Phase 3 clinical trial for GA, to seek marketing approval for Zimura in any indication;
- continue to develop tivozanib for the treatment of ophthalmic diseases;
- in-license or acquire the rights to, and pursue research and development of, other complementary products, product candidates or technologies, including drug delivery technology, for the treatment of ophthalmic diseases;
- seek marketing approval for any product candidates that successfully complete clinical trials;
- hire additional clinical, manufacturing, quality control, quality assurance and scientific personnel;
- expand our outsourced manufacturing activities, expand our commercial operations and establish sales, marketing and distribution capabilities, if we receive, or expect to receive, marketing approval for any product candidates;
- maintain, expand and protect our intellectual property portfolio; and
- add operational, financial and management information systems and personnel and expand our facilities.

Our ability to become and remain profitable depends on our ability to generate revenue in excess of our expenses. Our ability to generate revenues from product sales, which we do not expect will occur until the end of 2017 at the earliest, if ever, is dependent on our obtaining marketing approval for and commercializing our product candidates, in particular, Fovista and Zimura. 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. See “Risks Related to Product Development and Commercialization” for a further discussion of the risks we face in successfully commercializing our product candidates and achieving profitability.

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

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

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

As of March 31, 2016, we had cash, cash equivalents, and marketable securities of $356.1 million. We also had $365.6 million in total liabilities, $336.6 million of which related to the Novo Agreement and deferred revenue associated with the Novartis Agreement.

We believe that our cash, cash equivalents and marketable securities, together with the potential remaining $30.0 million enrollment-based milestone payment under the Novartis Agreement, will be sufficient to fund our operations and capital expenditure requirements as currently planned through the end of 2017. 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 capital requirements will depend on several 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 or technologies. For example, our expenses may exceed our expectations if we experience delays in enrollment or with the availability of drug supply for our clinical trials. Our expenses may also exceed our expectations if we increase our investigator fees for our clinical trials or if we further expand the scope of our clinical trials and programs, including, for example, by increasing the number of clinical trial sites or changing the geographic

Table of Contents

mix of sites at which patients are enrolled. Our costs may also exceed our expectations for other reasons, for example, if we experience issues with manufacturing validation, process development, the scale-up of manufacturing activities or activities to enable and qualify second source suppliers or if we decide to increase licensing or preclinical research and development activities or corporate staffing. 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. As a result, we may need or may seek to obtain additional funding in connection with our continuing operations prior than expected.

Moreover, our current Phase 3 clinical program for Fovista is expected to continue into 2018, and we expect to incur substantial expenditures to complete the Phase 3 clinical program after the receipt of initial, top-line data, which we expect to be available during the fourth quarter of 2016 for the two Fovista Phase 3 Lucentis Trials and during 2017 for the Fovista Phase 3 Eylea/Avastin Trial, based on current enrollment estimates. Furthermore, we expect the clinical development for 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 manufacturing validation activities associated with Fovista, process development and manufacturing scale-up and validation activities associated with 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 any other product candidate we may develop.

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

- the scope, progress, costs and results of our Phase 3 clinical program for Fovista;
- the scope, progress, costs and results of the Fovista Expansion Studies to further evaluate the potential benefit of Fovista in wet AMD when administered in combination with anti-VEGF drugs, and potentially in other ophthalmic diseases and conditions with unmet need;
- the scope, progress, costs and results of our Zimura clinical programs, including our Zimura Phase 2/3 GA Study and our Zimura Phase 2a Wet AMD Study, as well as any additional clinical trials (including a second Phase 3 trial for GA) required by regulatory authorities for us to seek marketing approval for Zimura in any indication;
- the costs and timing of manufacturing validation activities associated with Fovista;
- the costs and timing of process development and manufacturing scale-up and validation activities associated with Zimura;
- the costs, timing and outcome of regulatory reviews 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 outsourced manufacturing activities, including activities to build up our commercial drug supply and to enable and qualify second source suppliers, expanding our commercial operations 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 royalty payments that we will be obligated to make;
- the scope, progress and results of our preclinical studies, formulation development and clinical development plans for tivozanib;
- the scope, progress, results and costs of preclinical development and/or clinical trials for any other product candidates that we may develop;
- our ability to establish collaborations on favorable terms, if at all;
- the extent to which we in-license or acquire rights to, and develop, complimentary products, product candidates or technologies, including drug delivery technologies; and

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

Table of Contents

We do not have any committed external source of funds other than the Novartis Agreement. The remaining potential milestone payments under the Novartis Agreement are subject to our achievement of specified clinical, regulatory and commercial events related to Fovista, none of which can be assured. Our future commercial revenues, if any, will be derived from sales of Fovista, Zimura or any other products that we are able to successfully develop, which, depending on the product, may not be available for several years, if at all. In addition, if approved, Fovista or Zimura or any product that we acquire or in-license may not achieve commercial success. If that is the case, we may need to obtain substantial additional financing to achieve our business objectives. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

Adequate additional financing may not be available to us on acceptable terms, or at all. If we are unable to raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates. In addition, a default under the Novo Agreement would permit Novo A/S to foreclose on the Fovista intellectual property.

Until such time, if ever, as we can generate substantial product revenues, we may need or may seek to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. 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. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our existing 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.

We are 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.

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 grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves.

Risks Related to Product Development and Commercialization

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

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

Table of Contents

We expect to have initial, top-line data from our two Fovista Phase 3 Lucentis Trials in our Phase 3 clinical program for Fovista during the fourth quarter of 2016. Although we expect to have initial top-line data from the Fovista Phase 3 Eylea/Avastin Trial in 2017, the timing of the availability of such data is dependent, in part, on our ability to locate and enroll a sufficient number of eligible patients and may be subject to particular variability. 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.

The Fovista Phase 3 Eylea/Avastin Trial commenced nine months later than the two Fovista Phase 3 Lucentis Trials. We will not have data from our Fovista Phase 3 Eylea/Avastin Trial at the time data from the other two Fovista Phase 3 Lucentis Trials become 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. We plan to initially submit a New Drug Application, or NDA,

to the FDA for Fovista in combination with Lucentis based upon data from the two Fovista Phase 3 Lucentis Trials and subsequently submit an amendment to the NDA with data from the Fovista Phase 3 Eylea/Avastin Trial, subject to a favorable data outcome from these trials. Alternatively, we may choose to file a supplemental NDA for Fovista in combination with Eylea or Avastin following FDA review of the NDA for Fovista in combination with Lucentis. If we determine to delay seeking approval of Fovista in combination with Eylea or Avastin 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 the Fovista Phase 3 Eylea/Avastin Trial. Furthermore, although we may wish to amend our applications for marketing approval once we have data available from the Fovista Phase 3 Eylea/Avastin Trial, the FDA may not accept such an amendment. Moreover, if we subsequently amend our applications for marketing approval when data from the Fovista Phase 3 Eylea/Avastin Trial become available, we may experience further delays in our application process. The manner and timing in which we and our ex-U.S. commercialization partner, Novartis, seek marketing approval for Fovista may differ in the United States and in the European Union.

We expect that our Phase 3 clinical trials and the Fovista Expansion Studies 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. We are also supplying Fovista for third-party sponsored clinical trials. In addition, Novartis may commence additional preclinical and clinical trials for Fovista including those that it deems necessary for regulatory approval and/or pricing reimbursement outside of the United States. Adverse safety events or negative or inconclusive efficacy results in any of these trials may impact the progress of our Phase 3 clinical program, including our ability to receive marketing approval, and, if such data are received following a potential approval, our future sales of Fovista. As a result of these and other factors, we cannot accurately predict when or if Fovista will prove effective or safe in humans or will receive marketing approval.

In addition, we have invested substantial financial resources in the development of Zimura for the treatment of patients with both dry and wet AMD. There is 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.

Despite our current development plans and ongoing efforts, we may not complete any of our ongoing clinical trials or any other clinical trial for Fovista, Zimura or any other product candidates that we may develop in accordance with our plans. Moreover, the timing of the completion of, and the availability of initial results from, clinical trials is difficult to predict and is dependent, in part, on our ability to locate and enroll a sufficient number of eligible patients on a timely basis and, in the case of Zimura, on our ability to complete process development and manufacturing scale-up activities. Furthermore, our development plans may change based on feedback we may receive from regulatory authorities throughout the development process.

Our ability to successfully commercialize our product candidates, in particular Fovista and Zimura, will require us to be successful in a range of challenging activities, including:

- obtaining favorable results from our clinical trials, including, for Fovista, from our Phase 3 clinical program and in our other clinical trials involving Fovista, including the Fovista Expansion Studies, and for Zimura, our ongoing Zimura clinical programs;

- for Fovista, applying for and receiving 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 approved label given that Avastin, one of the current standard of care anti-VEGF drugs, is not approved for intravitreal use;

Table of Contents

- for Zimura, applying for and receiving marketing approvals from applicable regulatory authorities for the use of Zimura for the treatment of GA or the use of Zimura for other indications for which we may seek approval;

- making arrangements with third-party manufacturers, receiving regulatory approval of our manufacturing processes and our third-party manufacturers' facilities from applicable regulatory authorities and ensuring adequate supply of drug product;

- 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;

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

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

- if our product candidates are approved, obtaining from governmental and third-party payors adequate coverage and reimbursement for our product candidates and associated injection procedures;

- 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;

- protecting and enforcing our rights in our intellectual property portfolio; and

- complying with all applicable regulatory requirements, including FDA Good Manufacturing Practices, or GMP, standards and rules and regulations governing promotional and other marketing activities.

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 commercial success and profitability. In addition, our profitability and commercial success will depend, in part, on the ability of our commercialization partners, including, with respect to Fovista, the ability of Novartis as our ex-U.S. commercialization partner, to effectively market and sell product candidates that we develop, if approved outside the United States, and to obtain adequate coverage and reimbursement of such product candidates from governmental and third-party payors. Our failure to be commercially successful and 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 decrease in the value of our company would also cause our stockholders to lose all or part of their investment.

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

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

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

Table of Contents

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 two Fovista Phase 3 Lucentis Trials, which are evaluating 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 3 clinical program will be measured 12 months after the first dose of Fovista. This time point is substantially longer than 24 weeks after the first dose of Fovista, which was the time point at which the primary endpoint of mean change in visual acuity in our Phase 2b clinical trial was measured. Additionally, 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 the two Fovista Phase 3 Lucentis Trials testing a combination of 1.5 mg of Fovista and Lucentis for the treatment of wet AMD and the one Fovista Phase 3 Eylea/Avastin Trial testing a combination of 1.5 mg of Fovista with each of Eylea or Avastin for the treatment of wet AMD. We have very limited clinical data on the effects of Fovista when administered in combination with intravitreal injections of either Eylea or Avastin for the treatment of patients with wet AMD. Avastin is not approved for such use.

Fovista administered in combination with Lucentis was generally well tolerated in our Phase 1 and Phase 2b clinical trials. However, the results of these clinical trials may not be predictive of the results of our Phase 3 clinical program for Fovista. We have clinical data for Fovista administered in combination with Lucentis from only these two clinical trials with a limited follow-up of a maximum of 24 weeks. As compared to our Phase 2b clinical trial, our three Phase 3 trials are longer in duration (24 months) with a 12-month time point for the primary efficacy endpoint, have a greater number of patients (approximately 1,866), have a greater number of sites (more than 250), which encompass a much larger geographical recruitment area, and result in chronic exposure to a higher rate of intraocular pressure due to an increased injection volume. Consequently, there is potential for an increase in cumulative side effects resulting from two separate intravitreal injections and increased intraocular pressure in the Fovista combination therapy patients as compared to the patients receiving monotherapy anti-VEGF treatment and there is a much longer duration of therapy and greater geographic diversity of patients in our Phase 3 trials. This increase in the number of intravitreal injections and treatment burden, increased variability of patient care due to the larger number of clinical trial sites and the broader genetic profile of the enrolled patients from a larger geographic region may result in increased susceptibility to side effects of Fovista and/or resulting from the treatment procedure. Therefore, there is the potential for an unfavorable safety and tolerability profile in the Fovista combination therapy arm of the study as compared to our Phase 2b trial and monotherapy anti-VEGF trials which may be reflected in an increase in adverse events and/or serious adverse event rates (either ocular, systemic or both) in patients receiving Fovista combination therapy. For example, there may be, among others, an increase in the rates of intraocular infections, or endophthalmitis, intraocular pressure, glaucoma, retinal tears, cataracts, retinal detachment, intraocular inflammation, retinal and/or choroidal circulation compromise, cardiovascular disease such as myocardial infarctions, stroke, blood clots or emboli, or hospitalizations in the Fovista combination therapy patients.

In general, the FDA and similar regulatory authorities outside the United States require two adequate and well controlled clinical trials demonstrating safety and effectiveness for marketing approval for an ophthalmic pharmaceutical product. 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 the Fovista Phase 3 Lucentis Trials, we likely will not receive marketing approval for Fovista even if the combination of 1.5 mg of Fovista with Eylea or Avastin achieves superiority over Eylea or Avastin monotherapy with statistical significance on the primary endpoint in the Fovista Phase 3

Table of Contents

Eylea/Avastin Trial. 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 the Fovista Phase 3 Lucentis Trials and the combination of 1.5 mg of Fovista with Eylea or Avastin does not achieve superiority over Eylea or Avastin monotherapy with statistical significance on the primary endpoint in the Fovista Phase 3 Eylea/Avastin Trial, 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 the Fovista Phase 3 Lucentis Trials and the combination of 1.5 mg of Fovista with Eylea or Avastin achieves superiority over Eylea or Avastin monotherapy with statistical significance on the primary endpoint in the Fovista Phase 3 Eylea/Avastin Trial, the FDA or similar regulatory authorities outside the United States, nonetheless, may 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, nonetheless, may 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, Eylea and Avastin are widely used for the treatment of wet AMD. If a combination of 1.5 mg of Fovista with Eylea or Avastin does not achieve superiority over Eylea or Avastin monotherapy with statistical significance on the primary endpoint of mean change in visual acuity from baseline at 12 months in the Fovista Phase 3 Eylea/Avastin Trial, 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 Eylea or Avastin to achieve superiority over Eylea or Avastin 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 submitted the protocols to the FDA for the two Fovista Phase 3 Lucentis Trials in August 2013 and for the Fovista Phase 3 Eylea/Avastin Trial in April 2014. The FDA or other regulatory authorities may request additional information, require us to conduct additional nonclinical 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 obtained all of the necessary country approvals to proceed with our Phase 3 trials except for the Brazilian approval required to proceed with our Fovista Phase 3 Eylea/Avastin Trial, which we are pursuing. In the European Union, 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 Committee for Medicinal Products for Human Use, or CHMP, which is the committee responsible for preparing opinions on questions concerning medicines for human use. The CHMP informed us that the final label for Fovista, if it receives marketing approval, may be required to specify the licensed anti-VEGF drugs that were studied in combination with Fovista, given that Avastin is not approved for intravitreal use, rather than a label specifying Fovista for use in combination with any anti-VEGF drug.

The protocol for our Zimura Phase 2/3 GA Study calls for an initial stage to include approximately 300 patients. At month 18 of the trial, we plan to conduct an interim analysis to assess the safety and efficacy of Zimura compared to sham. Upon review of this interim analysis, a determination will be made whether to continue the trial and whether to expand the trial by enrolling additional patients. We may not have access to all of the available data from the trial when performing the interim analysis and in making the determination to continue and/or expand the trial. In addition, even if the trial is expanded following the interim analysis based on 18-month data, the trial may not yield positive data at the 24-month time point or for the additional patients enrolled in the trial. Moreover, assuming the Zimura Phase 2/3 GA Study progresses into the expansion stage, prior to seeking marketing approval for Zimura, we will need to conduct an additional Phase 3 clinical trial for Zimura for GA, which we may decide to initiate before having

Table of Contents

access to all of the data from the Zimura Phase 2/3 GA Study and based solely on the determination to expand the Zimura Phase 2/3 GA Study upon review of the interim analysis. Furthermore, we may be required by regulatory authorities to conduct other, additional clinical trials of Zimura, prior to seeking marketing approval in GA. Our development plans for Zimura, including our plans for our Zimura Phase 2/3 GA Study, may change based on feedback we may receive from regulatory authorities during development, including during the Zimura Phase 2/3 GA Study, or for other reasons.

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

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

Fovista administered in combination with Lucentis was generally well tolerated in our Phase 1 clinical trial and our Phase 2b clinical trials. However, we have complete clinical data for Fovista administered in combination with Lucentis from only these two clinical trials with a limited follow-up of a maximum of 24 weeks. As compared to our Phase 2b clinical trial, our three Phase 3 trials are longer in duration (24 months) with a 12 month time point for the primary efficacy endpoint, have a greater number of patients (approximately 1,866), have a greater number of sites (more than 250), which encompass a much larger geographical recruitment area, and result in chronic exposure to a higher rate of intraocular pressure due to an increased injection volume. Consequently, there is potential for an increase in cumulative side effects resulting from two separate intravitreal injections and increased intraocular pressure in the Fovista combination therapy patients as compared to the patients receiving monotherapy anti-VEGF treatment and there also is a much longer duration of therapy and greater geographic diversity of patients in our Phase 3 trials. This increase in the number of intravitreal injections and treatment burden, increased variability of patient care due to the larger number of clinical trial sites and the broader genetic profile of the enrolled patients from a larger geographic region may result in increased susceptibility to side effects of Fovista and/or resulting from the treatment procedure. Therefore there is the potential for an unfavorable safety and tolerability profile in the Fovista combination therapy arm of the study as compared to our Phase 2b trial and monotherapy anti-VEGF trials which may be reflected in an increase in adverse events and/or serious adverse event rates (either ocular, systemic or both) in patients receiving Fovista combination therapy. For example, there may be, among others, an increase in the rates of intraocular infections, or endophthalmitis, intraocular pressure, glaucoma, retinal tears, cataracts, retinal detachment, intraocular inflammation, retinal and/or choroidal circulation compromise, cardiovascular disease such as myocardial infarctions, stroke, blood clots or emboli, or hospitalizations in patients in the Fovista combination therapy arm of each trial.

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

Table of Contents

We have limited data regarding the safety, tolerability and efficacy of Zimura administered for the treatment of GA or administered in combination with anti-VEGF drugs for the treatment of wet AMD. Our clinical trials for Zimura will involve multiple intravitreal injections over an extended period of time and, as such, may involve risks regarding multiple and chronic intravitreal injections, similar to those described above for Fovista.

If we experience any of a number of possible unforeseen events in connection with our clinical trials, including delays in patient enrollment, potential marketing approval or commercialization of our product candidates 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, 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.

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.

Moreover, we may experience numerous other 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 the following:

- 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 to demonstrate statistically significant results may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;

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

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

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

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

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

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

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

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

Our third-party manufacturer of API for Fovista and Zimura has made only a limited number of batches of Fovista and Zimura to date. Fovista API has been manufactured at commercial scale only on a limited basis, and Zimura API has never been manufactured at commercial scale. Although we have produced commercial scale batches of Fovista API, these batches were not produced using PEG reagent produced at a commercial scale, which we are in the process of validating. The regulatory requirement to complete process validation 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, are subject to inspection and approval by the FDA before we can commence the commercial manufacture and sale of Fovista or Zimura, and thereafter on an ongoing basis. Our third-party API manufacturer has undergone only one Pre-Approval Inspection by the FDA. 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.

In addition, in order to manufacture and supply Fovista or any of our other product candidates on a commercial scale, we will need to bolster our quality control and quality assurance capabilities, including by augmenting our manufacturing processes and adding personnel. As we or any manufacturer we engage scales up manufacturing of any approved product, we may encounter

Table of Contents

unexpected issues relating to the manufacturing processes or the quality, purity or 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 underestimate the demand for an approved product, given the long lead times required to manufacture our products, we could potentially face commercial drug product supply shortages. 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.

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

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

- efficacy and potential advantages compared to alternative treatments, including the existing standard of care;
- any restrictions in the label 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 in the label on the use of our products by a subgroup of patients, such as by excluding from the Fovista label patients who would have been excluded from our clinical trials, for example, based on visual acuity measurements, comorbidities, such as patients with diabetes mellitus, previous treatment status or lesion characteristics, such as 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 ex-U.S. commercialization partner’s ability to offer our products at competitive prices, particularly in light of the additional cost of Fovista together with an anti-VEGF drug;
- availability of third-party coverage and adequate reimbursement, particularly by Medicare given our target market for persons over age 55;
- increasing reimbursement pressures on retinal specialists due to the formation of accountable care organizations and the shift away from traditional fee-for-service reimbursement models to reimbursement based on quality of care and patient outcomes;
- willingness of the target patient population to try new therapies and of physicians to prescribe these therapies, particularly in light of the existing available standard of care;
- prevalence and severity of any side effects;
- whether competing products or other alternatives are more convenient or easier to administer, including whether co-formulated alternatives, alternatives that can be co-administered in a single syringe or alternatives that offer a less invasive method of administration than intravitreal injection come to market; and
- the strength of our marketing and distribution support and that of Novartis, our partner for Fovista commercialization outside of the United States.

Table of Contents

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, including the presence of subretinal hyper-reflective material, or SHRM, using the commonly employed modality of spectral domain optical coherence tomography, or SD-OCT. We are not aware of any third-party clinical trials that have used this criteria to assess patient inclusion and as such do not know the

proportion of total cases of subfoveal neovascularization that are represented using this specific definition of SD-OCT inclusion criteria. However, a recent third-party retrospective analysis based on a treatment-naïve wet AMD population with relatively broad entry criteria in a National Eye Institute sponsored study showed that approximately 77% of patients in that study demonstrated the presence of SHRM. We cannot easily assess the impact on the potential market opportunity for Fovista 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 monotherapy limits further enhancement of visual outcome.

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

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

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

There are a number of products in preclinical research and clinical development by third parties to treat wet AMD. We expect that product candidates currently in clinical or preclinical development that directly or indirectly inhibit PDGF, the molecule that Fovista inhibits, or that inhibit the function of other molecules and which could obviate the use of an anti-PDGF agent such as

Table of Contents

Fovista may represent significant competition if approved. These product candidates may provide better efficacy, a safety, profile and/or convenience and other benefits that are not provided by our product candidates or currently marketed therapies. Based on publicly available information, we have identified, among others, the following ophthalmic product candidates in clinical and preclinical development that, like Fovista, are based on PDGF inhibition:

- Regeneron Pharmaceuticals and Bayer HealthCare have an anti-PDGF product candidate that is being co-formulated with Eylea for administration in a single intravitreal injection that entered clinical development in February 2014 and entered Phase 2 clinical trials in the second quarter of 2015.
- Ohr Pharmaceutical is developing an eye drop formulation of squalamine for wet AMD for which Ohr has completed a Phase 2 clinical study and recently announced the commencement of a Phase 3 clinical study.
- Santen has a dual inhibitor of VEGF and PDGF in Phase 1/2a clinical development.
- Tyrogenex has an orally-administered dual inhibitor of VEGF and PDGF that has completed a Phase 1 trial and commenced a Phase 2 study in the first quarter of 2015.
- Allergan has an anti-PDGF, anti-VEGF DARPin product candidate in preclinical development that is being co-formulated for administration in a single intravitreal injection.
- Neurotech has a PDGF antagonist that is in preclinical development that is designed as an encapsulated cell technology implant, potentially delivered in combination with an anti-VEGF drug.

- GrayBug has a dual VEGF and PDGF inhibitor administered in a sustained delivery injection that it plans to study in a Phase 1/2 clinical program.

- Somalogic has an anti-PDGF product candidate in preclinical development.

Because there are a variety of means to block the activity and signaling of PDGF, our patents and other proprietary protections for Fovista may not prevent development or commercialization of product candidates that are different from Fovista.

Moreover, several companies, including Novartis, Allergan, Neurotech, Genentech, Regeneron, Ophthea, Quark Pharmaceuticals, PanOptica, Inc., and others, have treatments targeting other molecular targets in various phases of clinical development for the treatment of wet AMD. RegenexBio and other companies are investigating potential gene therapy treatments for the treatment of wet AMD. Additionally, the London Project to Cure Blindness, which is a partnership involving the University College London and Pfizer, recently announced a successful pilot procedure for the transplant of retinal pigment epithelium cells derived from stem cells for the treatment of wet AMD and the commencement of a broader clinical trial.

In addition, other companies are undertaking efforts to develop technologies to allow for a less frequent dosing schedule for anti-VEGF drugs 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.

There are a number of products in preclinical research and clinical development by third parties to treat dry AMD. In general, these product candidates can be categorized based on their proposed mechanisms of action. The mechanisms of action for these product candidates include inflammation suppression, such as complement system inhibitors and corticosteroids, visual cycle modulators, antioxidants and neuroprotectants, cell and gene therapies and vascular enhancers. Based upon publicly available information, we have identified, among others, the following ophthalmic product candidates in clinical development that, like Zimura, are based on complement system inhibition:

- Genentech has a humanized Fab fragment administered by intravitreal injection that targets complement factor D, for which it completed a Phase 2 clinical trial and commenced Phase 3 trials.

- Novartis and MorphoSys have a fully human antibody targeting complement factor C5, which is in Phase 2 clinical development and for which data were recently presented.

- Apellis has a product candidate administered by intravitreal injection that inhibits complement factor C3, which is in Phase 2 clinical development.

- Alexion Pharmaceuticals has an intravenously administered product candidate targeting complement factor C5 approved for unrelated conditions, which completed a Phase 2 clinical trial for dry AMD in 2014.

Moreover, we have identified, among others, the following additional ophthalmic product candidates that are in the later stages of clinical development for the treatment of dry AMD:

Table of Contents

- Alimera Sciences has a corticosteroid intravitreal implant, Iluvien, which was recently approved for diabetic macular edema and which is being tested as a possible treatment for dry AMD.

- Acucela has an orally bioavailable selective visual cycle modulator, which is in a Phase 2b/3 clinical trial.

- Colby Pharmaceuticals has an ocular esterase cleavable prodrug of tempol hydroxylamine, which is in a Phase 2 clinical trial.

- Allergan has an α2-adrenergic receptor agonist, which has completed a Phase 2 clinical trial.

- Vision Medicines has a humanized monoclonal antibody that binds amyloid-β (Aβ), which is in a Phase 2 clinical trial.

- GlaxoSmithKline has an anti-amyloid B antibody, which is in a Phase 2 clinical trial.

- MacuClear has a topical systemic antihypertensive agent administered as an eye drop, which is in a Phase 2/3 clinical trial.

Several additional companies, including Ocata Therapeutics, CHA Biotech, Cell Cure Neurosciences and Catalyst Biosciences, have announced dry AMD programs.

Our commercial opportunity could be reduced or eliminated if one or more of our competitors develop and commercialize products that are more effective, safer, have fewer or less severe side effects, are more convenient to use or are less expensive than Fovista, Zimura or other products or product candidates 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 relevant 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 Lucentis, Eylea or particularly Avastin monotherapy. Insurers and other third-party payors may encourage the use of anti-VEGF drugs as monotherapy and discourage the use of Fovista in combination with these drugs. This could limit sales of Fovista.

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

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.

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

There are risks involved with establishing our own sales, marketing and distribution capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing and distribution capabilities is delayed or does not occur for any reason, we would have prematurely or

Table of Contents

unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

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

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to adequate numbers of physicians who may prescribe our products;
- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenues and our profitability, if any, are likely to be lower than if we were to market, sell and distribute ourselves any products that we develop. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute our product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

If we do not maintain a productive collaborative relationship with Novartis, to whom we have granted exclusive commercialization rights for Fovista outside of the United States, or if Novartis is unable to 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 any commercialization partner's 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 any commercialization partner, including Novartis, our ex-U.S. commercialization partner for Fovista, 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 any commercialization partner commercializes 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

Table of Contents

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 any commercialization partner may be required to conduct expensive pharmacoeconomic studies to justify coverage and reimbursement or the level of reimbursement relative to other therapies, including in the case of Fovista, relative to monotherapy with anti-VEGF drugs. If coverage and adequate reimbursement are not available or reimbursement is available only to limited levels, we may not be able to successfully commercialize Fovista, Zimura or any other product candidate for which we obtain marketing approval.

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

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 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 a commercialization partner 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.

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, including drug delivery technologies, for the treatment of ophthalmic diseases. Because we expect generally that we will not engage directly in early stage research and drug discovery, the future growth of our business will depend significantly on our ability to in-license or acquire the rights to approved products, additional product candidates or technologies. However, we may be unable to in-license or acquire the rights to any such products, product candidates or technologies from third parties. The in-licensing and acquisition of pharmaceutical products is an area characterized by intense competition, and a number of more established companies are also pursuing strategies to in-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 any commercialization partner could divert 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 any commercialization partner 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

Table of Contents

combination with our product candidates or our products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

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

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

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

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

Table of Contents

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

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

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations and may not perform their obligations as expected;
- collaborators may deemphasize or not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus, changes in product candidate priorities or available funding or changes in priorities as a result of a merger, acquisition or other corporate restructuring or transaction;
- 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

- 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 or other transaction, the foregoing risks would be heightened, and the business combination or transaction 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.

Table of Contents

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

If we are not able to establish additional, future 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 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.

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

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

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

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

If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates

and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors.

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

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

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

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

We currently rely exclusively upon Nektar to supply us with a proprietary polyethylene glycol, or PEG, reagent for Fovista under a manufacturing and supply agreement. PEG reagent is a chemical we use to modify the chemically synthesized aptamer in Fovista. The PEG reagent made by Nektar is proprietary to Nektar.

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 API, Zimura API or the PEG reagent we use for Fovista or Zimura fail to fulfill our purchase orders, or if any of these manufacturers should become unavailable to us for any reason, including as a result of capacity constraints, financial difficulties or insolvency, 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 agreements with such replacement manufacturers or fill-finish providers or to do so on acceptable terms.

Under the supply agreement with Nektar, we must purchase our entire requirements for PEG reagent for Fovista exclusively from Nektar at agreed prices based on volume. Similarly, under our clinical and commercial supply agreements with Agilent, we must purchase a specific percentage of our requirements for Fovista API from Agilent at agreed prices based on the volume of Fovista API ordered. In the event either of these suppliers breaches its supply obligations as specified in the applicable agreement, such supplier has agreed to enable a third-party manufacturer, if one is available, to supply us with PEG reagent and Fovista API, as applicable. In the case of Nektar, this alternative supply would last only until Nektar demonstrates that Nektar has the ability to supply all of our requirements for PEG reagent. The agreements of Nektar and Agilent to enable a third-party manufacturer may be difficult to enforce in the context of a breach by either of these suppliers of their supply obligations. In particular with respect to the potential replacement of Nektar, 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 API for use in any other FDA approved drug. Furthermore, with respect to the potential replacement of Nektar, the replacement manufacturer'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, in the case of the potential replacement of either Nektar or Agilent, 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 or Fovista API, as applicable, 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, in the case of a replacement of Nektar, equivalence between the Fovista API made using the Nektar PEG reagent and the Fovista API 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, or, in the case of a replacement of Agilent, equivalence between the Fovista API made by Agilent and the Fovista API made by the alternative manufacturer. 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 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 a divestiture 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 divestiture 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 divestiture 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 for Fovista, 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

Table of Contents

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 by June 30, 2018, we and Nektar may agree in good faith to extend such date in specified circumstances. If such date is not extended, Nektar may either terminate our license or convert our license for such country to a non-exclusive license. In addition, if we fail to use commercially reasonable efforts to develop Fovista and file and seek approval of new drug applications on a schedule permitting us to make first commercial sales of Fovista in specified countries by June 30, 2019, do not make such first commercial sales of Fovista by such date, or thereafter fail to use commercially reasonable efforts to continue to commercialize and market Fovista in such countries, we will be in material breach of the agreement and Nektar will have the right to terminate the agreement.

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

Risks Related to Our Intellectual Property

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

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

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. In addition, we may not pursue or obtain patent protection in all major markets. Moreover, in some circumstances, we do not have the right to control the preparation, filing or prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties or covering technology that a collaboration or commercialization partner 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 U.S. Patent and Trademark Office, or USPTO, 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 pending and future patent applications, and any collaboration or commercialization partner's 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 any collaboration or commercialization partner 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 a collaboration or commercialization partner's patents or narrow the scope of our or their patent protection.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art, may affect patent litigation and switch the U.S. patent system from a "first-to-invent" system to a "first-to-file" system. Under a first-to-file system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to the patent on an invention regardless of whether another inventor had made the invention earlier. The USPTO 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 USPTO, 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. Based on available information, we believe that *inter partes* review proceedings, brought by financial investors who may be selling short the stock of the patent holder, are becoming more prevalent. 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 early 2017. Such expiration date is prior to the date by which we expect Fovista to be commercialized in the United States if we obtain marketing approval. 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 this U.S. patent, and we expect such extension to be for approximately three years. 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. Similar to the patent term restoration available in the United States, the regulatory framework in the European Union and certain other foreign jurisdictions provides the opportunity to extend the term of a patent that covers an approved drug in certain circumstances. Notwithstanding the availability of patent term extension or restoration provisions, we may not be granted patent term extensions 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.

In addition to the patents described above, 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 projected patent expiry, absent any patent term adjustment or extension or patent restoration, would be in 2030.

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

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

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

Our issued patents may not be sufficient to provide us with a competitive advantage. For example, competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. Even if our owned or licensed patent applications issue as patents, they may not issue with a scope broad enough to provide us with

any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. We could also fail to take the required actions and pay the necessary governmental fees to maintain our patents.

The issuance of a patent is not conclusive as to its inventorship, ownership, scope, term, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. For example, if we receive marketing approval for our product candidates, other pharmaceutical companies may seek approval of generic versions of our products with the FDA or regulatory authorities in other jurisdictions. We may then be required to initiate proceedings against such companies in order to enforce our intellectual property rights. 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.

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, *inter partes* review, opposition, cancellation or similar proceedings before the USPTO 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. 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

Table of Contents

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.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact conceives or develops intellectual property that we regard as our own. Moreover, because we acquired rights to Fovista from Eyetech, Archemix and Nektar and rights to Zimura from Archemix, we must rely upon these parties' practices, and those of their predecessors, with regard to the assignment of intellectual

property therein. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

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

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

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

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

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

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

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

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

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

Risks Related to Regulatory Approval and Marketing of our Product Candidates and Other Legal Compliance Matters

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of some or all of our product candidates. 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, export and import, are subject to comprehensive regulation by the FDA and by the EMA and comparable regulatory agencies in other countries.

Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market Fovista, Zimura or any other product candidate from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely upon third-party CROs and Novartis to assist us in this process. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant 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.

Table of Contents

Accordingly, if we or our collaborators 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.

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

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, our ex-U.S. commercialization partner for Fovista, must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional preclinical or clinical 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, our ex-U.S. commercialization partner for Fovista, may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We and our third-party commercialization partners may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market.

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

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

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

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

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

Table of Contents

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 and 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 or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the medicine, including the requirement to implement a risk evaluation and mitigation strategy.

The FDA and other agencies, including the Department of Justice, or the DOJ, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA and DOJ impose stringent restrictions on manufacturers' communications regarding off-label use and if we do not market our products for their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug, and Cosmetic Act and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription drugs may lead to investigations and enforcement actions alleging violations of federal and state health care 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 on the labeling or marketing of a product;
- restrictions on distribution or use of a product;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters 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;
- damage to relationships with any potential collaborators;
- unfavorable press coverage and damage to our reputation;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure;
- injunctions or the imposition of civil or criminal penalties; and
- litigation involving patients using our products.

Table of Contents

Non-compliance with European Union requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also 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 healthcare providers, physicians and third-party payors 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 or arranging 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 through civil whistleblower or qui tam actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented, false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties, currently set at $5,500 to $11,000 per false claim;

- 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 Physician Payments Sunshine Act requires applicable manufacturers of covered drugs to report payments and other transfers of value to physicians and teaching hospitals, with data collection beginning in August 2013; and

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

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

If our operations are found to be in violation of any of the laws described above or any governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines and the curtailment or restructuring of our operations. Any penalties, damages, fines, curtailment or restructuring of our operations could adversely affect our financial results. We are developing and implementing a corporate compliance program designed to ensure that we will market and sell any future products that we successfully develop from our product candidates in compliance with all applicable laws and regulations, but we cannot guarantee that this program will protect us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Table of Contents

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 healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

Current and future legislation may increase the difficulty and cost for us and any collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, 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 any commercialization partner's 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 a commercialization partner receives for any approved product.

For example, 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 the ACA. Among the provisions of the ACA of potential importance to our business and our product candidates are the following:

- an annual, non-deductible 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;

- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;

- expansion of healthcare fraud and abuse laws, including the civil False Claims Act and the federal 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 of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to individuals enrolled in Medicaid managed care organizations;
- 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 certain financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research;
- a new Independent Payment Advisory Board, or IPAB, which has authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription drugs; and
- establishment of the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models.

Table of Contents

Other legislative changes have been proposed and adopted since the ACA was enacted. These changes include the Budget Control Act of 2011, which, among other things, led to aggregate reductions to Medicare payments to providers of up to 2% per fiscal year that started in 2013 and, due to subsequent legislation, will continue until 2025. In addition, the American Taxpayer Relief Act of 2012, 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 and otherwise affect the prices we may obtain for any of our product candidates for which regulatory approval is obtained.

We expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our products are prescribed or administered. By way of example, in March 2016, CMS proposed a rule to test, or demonstrate, a new model of reimbursement for Medicare Part B drugs that could potentially negatively impact the reimbursement available to physicians who administer drugs by intravitreal injection in their offices and clinics. Each of Lucentis, Eylea and Avastin are administered by intravitreal injection for the treatment of wet AMD directly by physicians, typically in their offices or clinics. Our product candidates, Fovista and Zimura, are also administered by intravitreal injection directly by physicians, and, if approved, may ultimately be reimbursed under Medicare Part B for patients receiving Medicare benefits. Comments on the proposed rule are due in May 2016. The proposed rule, if adopted in its current form, would implement the reimbursement model demonstration beginning before the end of 2016. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors.

The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products. Moreover, 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, may be. In addition, increased scrutiny by the United States Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us and any collaborators to more stringent product labeling and post-marketing testing and other requirements.

We are subject to U.S. and foreign anti-corruption and anti-money laundering laws with respect to our operations and non-compliance with such laws can subject us to criminal and/or civil liability and harm our business.

We are subject to the U.S. Foreign Corrupt Practices Act of 1977, as amended, or the FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and possibly other state and national anti-bribery and anti-money laundering laws in countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, third-party intermediaries, joint venture partners and collaborators from authorizing, promising, offering, or providing, directly or indirectly, improper payments or benefits to recipients in the public or private sector. We may have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. In addition, we may engage third party intermediaries to promote our clinical research activities abroad and/or to obtain necessary permits, licenses, and other regulatory approvals. We can be held liable for the corrupt or other illegal activities of these third-party intermediaries, our employees, representatives, contractors, partners, and agents, even if we do not explicitly authorize or have actual knowledge of such activities.

Noncompliance with anti-corruption and anti-money laundering laws could subject us to whistleblower complaints, investigations, sanctions, settlements, prosecution, other enforcement actions, disgorgement of profits, significant fines, damages, other civil and criminal penalties or injunctions, suspension and/or debarment from contracting with certain persons, the loss of export privileges, reputational harm, adverse media coverage, and other collateral consequences. If any subpoenas, investigations, or other enforcement actions are launched, or governmental or other sanctions are imposed, or if we do not prevail in any possible civil or criminal litigation, our business, results of operations and financial condition could be materially harmed. In addition, responding to any action will likely result in a materially significant diversion of management's attention and resources and significant defense and compliance costs and other professional fees. In certain cases, enforcement authorities may even cause us to appoint an independent compliance monitor which can result in added costs and administrative burdens.

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

Table of Contents

from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

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

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

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

Risks Related to Employee Matters and Managing Growth and Our Operations

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

We are highly dependent on David R. Guyer, M.D., our Chief Executive Officer, Samir C. Patel, M.D., our President, and Glenn P. Sblendorio, our Executive Vice President, Chief Operating Officer and Chief Financial Officer, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into letter 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, quality assurance, quality control 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.

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

We are currently experiencing significant and rapid growth in the number of our employees and the scope of our operations, particularly in the areas of clinical development, manufacturing development, quality control and quality assurance. During the 12-month period ending March 31, 2016, we hired close to half of our over 130 employees. We also expect to continue to hire additional employees and expand the scope of our operations in the area of clinical development, manufacturing, quality control, quality assurance and, as we approach potential marketing approval for any of our product candidates, in the area of sales, marketing, market access 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.

Table of Contents

If we fail to establish and maintain effective internal control over financial reporting, our ability to accurately report our financial results could be adversely affected.

A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the company's annual or interim financial statements will not be prevented or detected on a timely basis. If any material weakness in our internal control over financial reporting is discovered or occurs in the future, our financial statements may contain material misstatements and we could be required to restate our financial results. As previously disclosed in July 2015, our management concluded that we experienced

a material weakness in internal controls related to technical accounting expertise over the accounting for deferred tax assets and income tax accounting in general during certain prior financial reporting periods. The deficiency in the application of our controls relating to technical accounting expertise over the accounting for deferred tax assets and income tax accounting in general resulted in the audit committee of our board of directors concluding that the relevant financial statements should not be relied upon, and our subsequent restatement of the relevant financial statements.

During the year ended December 31, 2015, we took the following steps to remediate the identified material weakness: we added staffing within our finance department and engaged a nationally recognized accounting firm, in each case, with technical expertise in the area of tax accounting and financial reporting. Our management has concluded that the identified material weakness in internal control over financial reporting was fully remediated as of December 31, 2015. Although we have remediated this deficiency in internal control over financial reporting, we cannot be certain that the remedial measures that we have taken will ensure that we maintain adequate controls over our financial reporting in the future and, accordingly, additional material weaknesses could occur or be identified. Any additional material weaknesses or combination of deficiencies could materially and adversely affect our ability to provide timely and accurate financial information, and the current and future deficiencies may impact investors' confidence in our internal controls and our company, which could cause our stock price to decline.

Risks Related to Information Technology

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

In the ordinary course of our business, we and our third-party contractors maintain sensitive data on our and their respective networks, including our intellectual property and proprietary or confidential business information relating to our business and that of our clinical trial participants and business partners. In particular, we rely on contract research organizations and other third parties to store and manage information from our clinical trials, including our Fovista Phase 3 clinical program. The secure maintenance of this sensitive information is critical to our business and reputation. Despite the implementation of security measures, our internal computer systems and those of our third-party contractors are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. In particular, we believe that companies have been increasingly subject to a wide variety of security incidents, cyber-attacks and other attempts to gain unauthorized access. These threats can come from a variety of sources, ranging in sophistication from an individual hacker to a state-sponsored attack. Cyber threats may be generic, or they may be custom-crafted against our information systems or those of our third-party contractors. For information stored with our third-party contractors, we rely upon, and the integrity and confidentiality of such information is dependent upon, the risk mitigation efforts such third-party contractors have in place. In the recent past, cyber-attacks have become more prevalent and much harder to detect and defend against. Our and our third-party contractors' respective network and storage applications may be subject to unauthorized access by hackers or breached due to operator error, malfeasance or other system disruptions. It is often difficult to anticipate or immediately detect such incidents and the damage caused by such incidents. System failures, data breaches and any unauthorized access or disclosure of our information or intellectual property could compromise our intellectual property and expose sensitive business information. System failures or accidents or security breaches could cause interruptions in our operations, and could result in a material disruption of our clinical and commercialization activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. A data security breach could also lead to public exposure of personal information of our clinical trial patients and others. Cyber-attacks could cause us to incur significant remediation costs, result in product development delays, disrupt key business operations and divert attention of management and key information technology resources. The loss of clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate public disclosure of confidential or proprietary information, we could incur liability and our product research, development and commercialization efforts could be delayed.

Table of Contents

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 March 31, 2016, our executive officers, directors and a small group of stockholders, in the aggregate, beneficially owned shares representing a significant percentage 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.

If a significant portion of our total outstanding shares are sold into the market, the market price of our common stock could 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 March 31, 2016, we had outstanding 35,290,135 shares of common stock. Of these shares, approximately 5,185,000 shares are restricted or control securities under Rule 144 under the Securities Act. Any of our remaining shares that are not restricted or control 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 4,455,000 shares of our common stock have rights, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We have filed registration statements on Form S-8 registering all shares of common stock that we may issue under our equity compensation plans prior to awards becoming exercisable. As of March 31, 2016, we had outstanding stock options to purchase an aggregate of approximately 3,495,000 shares of our common stock, of which options to purchase approximately 1,202,000 shares were vested, as well as approximately 404,000 unvested RSUs. Once registered on Form S-8, shares underlying these equity awards can be freely sold in the public market upon issuance, subject to volume, notice and manner of sale limitations applicable to affiliates.

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

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

- provide for a classified board of directors such that only one of three classes of directors is elected each year;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board of directors;
- provide for advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and

Table of Contents

- 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 and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. 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. In particular, we expect that there may be increased trading volumes and volatility in our stock price as we approach the announcement of the initial, top-line data from the two Fovista Phase 3 Lucentis Trials as part of our Fovista Phase 3 clinical program, which we expect to be available during the fourth quarter of 2016. 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 products or technologies that compete with our product candidates;
- results of clinical trials of Fovista, Zimura and any other product candidate that we may develop and the timing of the receipt of such results;
- results of clinical trials of product candidates of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to in-license or acquire the rights to other products, product candidates and technologies for the treatment of ophthalmic diseases, the costs of commercializing any such products and the costs of development of any such product candidates or technologies;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

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

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

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

Table of Contents

initiatives. Moreover, these rules and regulations have increased our legal and financial compliance costs and will make some activities more time-consuming and costly.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to furnish with our periodic Exchange Act reports a report by our management on our internal control over financial reporting. We are also required to include with our annual report an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404, we must 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 and engage outside consultants 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. There is a risk that our internal control over financial reporting may, in the future, be found to be ineffective under Section 404. Our identification of one or more material weaknesses 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.

Table of Contents

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

Recent Sales of Unregistered Securities

We did not sell any unregistered equity securities during the period covered by this Quarterly Report on Form 10-Q.

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 March 31, 2016, we have used approximately $99.1 million of the net proceeds from our initial public offering as follows:

- approximately $72.5 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 $26.6 million for working capital and other general corporate purposes.

Other than payments related to executive officer and director compensation, all as described in our public filings, 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 our 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.

On May 9, 2016, we and PSN Partners, L.P. entered into an Addendum to Office Lease Agreement to extend our lease with respect to the approximate 1,800 square feet of office space in Princeton, New Jersey three additional years, through August 2019. As part of the extension, the annual fixed rent for this lease was increased based on current market conditions.

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.

Table of Contents

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: May 9, 2016

By: /s/ Glenn P. Sblendorio
Glenn P. Sblendorio
Executive Vice President, Chief Operating Officer and Chief Financial Officer
(Principal Financial and Accounting Officer)

Table of Contents

EXHIBIT INDEX

Exhibit Number	Description of Exhibit
10.1	Offer of Employment between the Registrant and Glenn P. Sblendorio, dated January 4, 2016
10.2	Letter Agreement between the Registrant and Glenn P. Sblendorio, dated January 4, 2016
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.DEF*	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE*	XBRL Taxonomy Extension Presentation Linkbase Document

* Submitted electronically herewith.

Attached as Exhibit 101 to this report are the following formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheets at March 31, 2016 and December 31, 2015 (unaudited), (ii) Statements of Operations (unaudited) for the three month period ended March 31, 2016 and 2015, (iii) Statements

of Cash Flows (unaudited) for the three month period ended March 31, 2016 and 2015 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
19th Floor
New York, NY 10119
(212) 845-8200

January 4, 2016

Mr. Glenn Sblendorio

Dear Glenn:

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 Operating Officer, Chief Financial Officer and Treasurer, effective upon the date on which the Company's current Executive Vice President, Chief Financial and Business Officer, and Treasurer ceases to serve in such position pursuant to the terms of a separation agreement and general release entered into with the Company on or about the date hereof (the "Start Date"). Notwithstanding the foregoing, the Start Date shall not be earlier than March 31, 2016 without your consent. As the Company's Executive Vice President, Chief Operating Officer, Chief Financial Officer and 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 companies for which you currently serve or are engaged with that will be creating a board, may serve on civic, charitable, educational, religious, public interest or public service boards (other than the Company), 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. You will resign as a

---

member of the Board of Directors (the "Board) of the Company prior to commencement of your employment with the Company.

2. **Base Salary.** Your base salary will be at the rate of $19,038 per bi-weekly pay period (which if annualized equals $495,000), 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 adjusted from time to time in accordance with normal business practices and in the sole discretion of the Company.

3. **Discretionary Bonus.** Following the end of each calendar year and subject to the approval of the Company's Board, you will be eligible for a performance bonus of up to 55% 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, 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 pro-rata discretionary bonus for 2016.

4. **Equity.** In connection with the commencement of your employment with the Company, you will be eligible to receive an option to purchase 150,000 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. In addition, you will be awarded a one-time grant of 75,000 Restricted Stock Units that will be granted on the Start Date (the "RSU Grant"). If the Board approves the grant, the Restricted Stock Unit will 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 at 25% on each anniversary date thereafter, pursuant to the terms of the restricted stock agreement. The Company grants additional equity awards annually based on performance. You would be eligible for an annual performance-based option and restricted stock unit grant in January of 2017 on a pro-rata basis.

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. **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.

7. **Sign-On Bonus**. Ophthotech will pay you a sign-on bonus of $100,000, less applicable taxes and withholdings, payable in the first pay period after you join. Ophthotech reserves the right to require repayment of this amount should you voluntarily leave employment during your first 12 months with the Company.

8. **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.

9. **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.

10. **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.

11. **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 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.

12. **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.

13. **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.

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 January 5, 2016 the offer will be deemed withdrawn.

Sincerely,

By: /s/ Amy R. Sheehan
Amy R. Sheehan
Vice President, 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/ Glenn Sblendorio
Glenn Sblendorio

1/4/2016
Date

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

January 4, 2016

Mr. Glenn Sblendorio

Dear Glenn:

The board of directors (the "Board") of Ophthotech Corporation (the "Company") has provided for the following severance benefits to be provided to you in the event of your termination of employment with the Company, on the terms and conditions set forth herein.

1. **Severance.**

(a) If your employment is terminated (1) at any time by the Company without Cause or by you for Good Reason (as such terms are herein defined) or (2) within one year following a Change in Control Event (as defined in the Company's 2013 Stock Incentive Plan), by the Company, or its successor, without Cause or by you for Good Reason, the Company or its successor 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 a pro-rated portion of your Target Bonus (as such term is defined in your offer of employment with us dated January 4, 2016 (the "Offer Letter")) for the year in which your employment terminates, provided, however, that if your employment is terminated under the circumstances described in (2) of this Section 1(a), the Company or its successor will instead pay you an amount equal to your Target Bonus for the year in which your employment terminates, in either case, without regard to whether the performance goals with respect to such Target Bonus have been established or met and 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 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 (the "Code"), 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).

(b) 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.

(c) 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.

(d) 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 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.

2. **Equity Acceleration**. If your employment with the Company, or its successor, is terminated by the Company or such successor without Cause or by you for Good Reason within the one (1) year period following a Change in Control Event, then the then-unvested portion of any equity awards held by you that vest solely based on the passage of time shall immediately vest in full and become exercisable or free from forfeiture or repurchase, as applicable, as of the date of such termination.

3. **Modified Cutback**.

(a) Notwithstanding any other provision of this letter agreement, the Offer Letter, or any other agreements between you and us, except as set forth in Section 3(b) hereof, in the event that the Company undergoes a "Change in Ownership or Control" (as defined below), the Company shall not be obligated to provide you a portion of any "Contingent Compensation Payments" (as defined below) that you would otherwise be entitled to receive to the extent necessary to eliminate any "excess parachute payments" (as defined in Section 280G(b)(1) of the Code) for you. For purposes of this Section 3(a), the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Payments" and the aggregate amount (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-30 or any successor provision) of the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Amount."

(b) Notwithstanding the provisions of Section 3(a), no such reduction in Contingent Compensation Payments shall be made if (1) the Eliminated Amount (computed without regard to this sentence) exceeds (2) 100% of the aggregate present value (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-31 and Q/A-32 or any successor provisions) of the amount of any additional taxes that would be incurred by you if the Eliminated Payments (determined without regard to this sentence) were paid to you (including, state and federal income taxes on the Eliminated Payments, the excise tax imposed by Section 4999 of the Code payable with respect to all of the Contingent Compensation Payments in excess of your "base amount" (as defined in Section 280G(b)(3) of the Code), and any withholding taxes). The override of such reduction in Contingent Compensation Payments pursuant to this Section 3(b) shall be referred to as a "Section 3(b) Override." For purpose of this paragraph, if any federal or state income taxes would be attributable to the receipt of any Eliminated Payment, the amount of such taxes shall be computed by multiplying the amount of the Eliminated Payment by the maximum combined federal and state income tax rate provided by law.

(c) For purposes of this Section 3 the following terms shall have the following respective meanings:

(i) "Change in Ownership or Control" shall mean a change in the ownership or effective control of the Company or in the ownership of a substantial portion of the assets of the Company determined in accordance with Section 280G(b)(2) of the Code.

(ii) "Contingent Compensation Payment" shall mean any payment (or benefit) in the nature of compensation that is made or made available (under this Agreement or otherwise) to a "disqualified individual" (as defined in Section 280G(c) of the Code) and

that is contingent (within the meaning of Section 280G(b)(2)(A)(i) of the Code) on a Change in Ownership or Control of the Company.

(d) Any payments or other benefits otherwise due to you following a Change in Ownership or Control that could reasonably be characterized (as determined by the Company) as Contingent Compensation Payments (the "Potential Payments") shall not be made until the dates provided for in this Section 3(d). Within 30 days after each date on which you first become entitled to receive (whether or not then due) a Contingent Compensation Payment relating to such Change in Ownership or Control, the Company shall determine and notify you (with reasonable detail regarding the basis for its determinations) (1) which Potential Payments constitute Contingent Compensation Payments, (2) the Eliminated Amount and (3) whether the Section 3(b) Override is applicable. Within 30 days after delivery of such notice to you, you shall deliver a response to the Company (the "Executive Response") stating either (A) that you agree with the Company's determination pursuant to the preceding sentence or (B) that you disagree with such determination, in which case you shall set forth (x) which Potential Payments should be characterized as Contingent Compensation Payments, (y) the Eliminated Amount, and (z) whether the Section 3(b) Override is applicable. In the event that you fail to deliver an Executive Response on or before the required date, the Company's initial determination shall be final. If you state in the Executive Response that you agree with the Company's determination, the Company shall make the Potential Payments to you within three business days following delivery to the Company of the Executive Response (except for any Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). If you state in the Executive Response that you disagree with the Company's determination, then, for a period of 60 days following delivery of the Executive Response, you and the Company shall use good faith efforts to resolve such dispute. If such dispute is not resolved within such 60-day period, such dispute shall be settled exclusively by arbitration in New York, New York, in accordance with the rules of the American Arbitration Association then in effect. Judgment may be entered on the arbitrator's award in any court having jurisdiction. The Company shall, within three business days following delivery to the Company of the Executive Response, make to you those Potential Payments as to which there is no dispute between the Company and you regarding whether they should be made (except for any such Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). The balance of the Potential Payments shall be made within three business days following the resolution of such dispute.

(e) The Contingent Compensation Payments to be treated as Eliminated Payments shall be determined by the Company by determining the "Contingent Compensation Payment Ratio" (as defined below) for each Contingent Compensation Payment and then reducing the Contingent Compensation Payments in order beginning with the Contingent Compensation Payment with the highest Contingent Compensation Payment Ratio. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio, such Contingent Compensation Payment shall be reduced based on the time of payment of such Contingent Compensation Payments with amounts having later payment dates being reduced first. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio and the same time of payment, such Contingent Compensation Payments shall be reduced on a pro rata basis (but not below zero) prior to reducing Contingent Compensation Payment

with a lower Contingent Compensation Payment Ratio. The term "Contingent Compensation Payment Ratio" shall mean a fraction the numerator of which is the value of the applicable Contingent Compensation Payment that must be taken into account by you for purposes of Section 4999(a) of the Code, and the denominator of which is the actual amount to be received by you in respect of the applicable Contingent Compensation Payment. For example, in the case of an equity grant that is treated as contingent on the Change in Ownership or Control because the time at which the payment is made or the payment vests is

accelerated, the denominator shall be determined by reference to the fair market value of the equity at the acceleration date, and not in accordance with the methodology for determining the value of accelerated payments set forth in Treasury Regulation Section 1.280G-1Q/A-24(b) or (c)).

(f) The provisions of this Section 3 are intended to apply to any and all payments or benefits available to you under this letter agreement or any other agreement or plan of the Company under which you receive Contingent Compensation Payments.

4. **Miscellaneous**.

(a) 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 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 Section 105(a) of the Code 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 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. Termination of employment as used herein shall mean 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.

(b) 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.

(c) Conflict; Amendment; Counterparts. This letter agreement sets forth the Company's sole obligation, subject to the terms and conditions set forth herein, to provide severance benefits to you. The severance benefits set forth in this letter agreement are therefore in lieu of, and not in addition to, the severance benefits described in Section 6 the Offer Letter or any other provision thereof or any other agreement or arrangement between you and us. Except as modified hereby, the terms of the Offer Letter remain in full force and effect. This agreement may only be modified in a document signed by both the Company and you. This agreement may be executed in counterparts, each of which will be deemed an original, but all of which will be deemed one and the same instrument.

[*Remainder of page intentionally left blank*]

If the provisions of this agreement are acceptable to you, please sign and date this agreement below and return the signed and dated version to me on or before January 5, 2016.

Sincerely,

OPHTHOTECH CORPORATION

By: /s/ Amy R. Sheehan
Amy R. Sheehan
Vice President, Human Resources

ACCEPTED AND AGREED:

/s/ Glenn Sblendorio
Glenn Sblendorio

Date: 1/4/2016

Exhibit 31.1

CERTIFICATIONS

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

1. I have reviewed this Quarterly Report on Form 10-Q for the quarter ended March 31, 2016 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) 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) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) 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

(d) 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: May 9, 2016

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

Exhibit 31.2

CERTIFICATIONS

I, Glenn P. Sblendorio, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the quarter ended March 31, 2016 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) 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) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) 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

(d) 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: May 9, 2016

By: /s/ Glenn P. Sblendorio
Glenn P. Sblendorio
Executive Vice President, Chief Operating Officer and
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

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 March 31, 2016 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: May 9, 2016

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

Exhibit 32.2

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 March 31, 2016 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Glenn P. Sblendorio, 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: May 9, 2016

By: /s/ Glenn P. Sblendorio
Glenn P. Sblendorio
Executive Vice President, Chief Operating Officer and Chief Financial Officer
(Principal Financial Officer)