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 September 30, 2015

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 October 30, 2015 there were 34,959,573 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 therapy 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 planned trials for Zimura® for the treatment of patients with geographic atrophy, a form of dry AMD and, in combination with anti-VEGF therapy, for the treatment of certain forms 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 planned 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)

	September 30, 2015	December 31, 2014
Assets		
Current assets		
Cash and cash equivalents	$ 254,895	$ 39,814
Available for sale securities	26,147	423,746
Due from Novartis Pharma AG	—	960
Prepaid expenses and other current assets	5,541	8,812
Deferred tax assets	1,500	50
Total current assets	288,083	473,382
Available for sale securities	144,944	—
Property and equipment, net	2,590	1,555
Deferred tax assets, non-current	14,632	4,467
Security deposits	467	282
Other assets	35	100
Total assets	$ 450,751	$ 479,786
Liabilities and Stockholders' Equity		
Current liabilities		
Accrued research and development expenses	$ 20,080	$ 7,918
Accounts payable and accrued expenses	7,850	8,707
Deferred revenue	6,714	3,206
Total current liabilities	34,644	19,831
Deferred revenue, long-term	207,996	206,418
Royalty purchase liability	125,000	125,000
Total liabilities	367,640	351,249
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, 34,903,881 and 33,994,520 shares issued and outstanding at September 30, 2015 and December 31, 2014, respectively	35	34
Additional paid-in capital	452,887	428,390
Accumulated deficit	(369,890)	(299,822)
Accumulated other comprehensive income (loss)	79	(65)
Total stockholders' equity	83,111	128,537
Total liabilities and stockholders' equity	$ 450,751	$ 479,786

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 September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Collaboration revenue	$ 3,448	$ 39,575	$ 46,723	$ 39,575
Operating expenses:				
Research and development	40,479	17,105	97,095	66,189
General and administrative	10,412	8,812	31,955	22,731
Total operating expenses	50,891	25,917	129,050	88,920
Income (loss) from operations	(47,443)	13,658	(82,327)	(49,345)
Interest income and other	339	73	630	189
Income (loss) before income tax provision	(47,104)	13,731	(81,697)	(49,156)

Income tax (benefit) provision	(7,531)	5,179	(11,629)	35,964
Net income (loss)	$ (39,573)	$ 8,552	$ (70,068)	$ (85,120)
Net income (loss) per common share:				
Basic	$ (1.14)	$ 0.26	$ (2.03)	$ (2.57)
Diluted	$ (1.14)	$ 0.25	$ (2.03)	$ (2.57)
Weighted average common shares outstanding:				
Basic	34,782	33,531	34,432	33,074
Diluted	34,782	34,859	34,432	33,074

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 September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Net income (loss)	$ (39,573)	$ 8,552	$ (70,068)	$ (85,120)
Other comprehensive income:				
Unrealized gain on available for sale securities, net of taxes	55	10	144	35
Other comprehensive income	55	10	144	35
Comprehensive income (loss)	$ (39,518)	$ 8,562	$ (69,924)	$ (85,085)

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

Table of Contents

OPHTHOTECH CORPORATION
Unaudited Statements of Cash Flows
(in thousands)

	Nine months ended September 30,	
	2015	2014
Operating Activities		
Net loss	$ (70,068)	$ (85,120)
Adjustments to reconcile net loss to net cash (used in) provided by operating activities		
Depreciation	535	80
Amortization of premiums and discounts on investment securities	2,517	1,338
Gain on sale of marketable securities	(53)	—
Deferred income taxes	(11,722)	(2,144)
Share-based compensation	18,474	9,492
Excess tax benefits from share-based compensation	—	(2,335)
Changes in operating assets and liabilities:		
Due from Novartis Pharma AG	960	(50,000)
Prepaid expenses and other current assets	3,271	4,011
Accrued interest receivable	320	148
Security deposits	(185)	(28)
Other assets	65	(84)
Accrued research and development expenses	12,162	3,857
Accounts payable and accrued expenses	(857)	2,896
Income tax payable	—	7,785
Deferred revenue	5,086	210,425
Net cash (used in) provided by operating activities	(39,495)	100,321
Investing Activities		
Purchase of marketable securities	(382,921)	(385,403)
Sale of marketable securities	367,043	—
Maturities of marketable securities	266,000	70,000
Purchase of property and equipment	(1,576)	(1,342)
Proceeds from sale of assets	6	—
Net cash provided by (used in) investing activities	248,552	(316,745)
Financing Activities		
Proceeds from stock option/warrant exercises	6,024	1,221
Proceeds from follow-on public offering, net	—	55,409
Excess tax benefits from share-based compensation	—	2,335
Proceeds from royalty purchase agreement	—	41,667
Net cash provided by financing activities	6,024	100,632
Net change in cash and cash equivalents	215,081	(115,792)
Cash and cash equivalents		

Beginning of period	39,814	210,596
End of period	$ 254,895	$ 94,804
Supplemental disclosure of non-cash information related to investing activities		
Change in unrealized gains in available for sale securities, net of tax	$ 144	$ 35

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

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 in Phase 3 clinical development for use in combination with anti-VEGF therapy 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) and has completed patient recruitment for two Phase 3 clinical trials of Fovista administered in combination with Lucentis. The Company is also developing its product candidate Zimura® for the treatment of patients with geographic atrophy ("GA"), a form of dry AMD, and, in combination with anti-VEGF therapy for the treatment of wet AMD in patients with suboptimal response to anti-VEGF monotherapy, as well as 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 forumulation 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 September 30, 2015 and for the three and nine months ended September 30, 2015 and 2014 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, 2014 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 2014, as filed with the SEC on March 2, 2015, and as amended on Form 10-K/A and filed with the SEC on July 28, 2015.

In the opinion of management, the unaudited financial information as of September 30, 2015 and for the three and nine months ended September 30, 2015 and 2014, 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 and nine months ended September 30, 2015 and 2014 are not necessarily indicative of the operating results for the full fiscal year or any future period.

Use of Estimates

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

Cash and Cash Equivalents

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

Available for Sale Securities

The Company considers securities with original maturities of greater than 90 days to be available for sale securities. 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 recognized revenue of approximately $46.7 million during the nine months ended September 30, 2015, which primarily related to the $50.0 million milestone it achieved in March 2015. The balance of the milestone payment was recorded as deferred revenue. During the three months ended September 30, 2015, the Company recognized revenue of approximately $3.4 million. 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 and nine months ended September 30, 2015 and 2014:

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
License revenue	$ —	$ 38,373	$ 38,083	$ 38,373
Research and development activity revenue	1,594	1,200	6,768	1,200
API transfer revenue	1,851	—	1,851	—
Joint operating committee revenue	3	2	21	2
Total collaboration revenue	$ 3,448	$ 39,575	$ 46,723	$ 39,575

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 or 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. For example, a milestone payment may be due to the Company upon the FDA's acceptance of a New Drug Application ("NDA"). Commercial milestones are typically achieved when an approved pharmaceutical product reaches certain defined levels of net sales by the licensee, such as when a product first achieves global sales or annual sales of a specified amount.

Revenues from clinical and development and regulatory milestone payments, if the milestones are deemed substantive and the milestone payments are nonrefundable, are recognized upon successful accomplishment of the milestones. With regard to the Novartis Agreement, the Company has concluded that the clinical and development milestones and certain regulatory milestones are not substantive and that the regulatory approval milestones are substantive. 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 U.S. Treasury securities with maturities of 90 days or less and investments in money market funds.

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

Foreign Currency Translation

The Company considers the U.S. dollar to be its functional currency. Expenses 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 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, an anti-platelet derived growth factor ("PDGF") aptamer that the Company is developing for use in combination with anti-VEGF therapy for treatment of wet AMD, and Zimura, an inhibitor of complement factor C5 that the Company is developing for the treatment of patients with GA, and, in combination with anti-VEGF therapy, for the treatment of PCV, in patients who do not respond adequately to treatment with anti-VEGF monotherapy or for whom anti-VEGF monotherapy fails, 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.

The Company anticipates that it will continue to incur significant research and development expenses in connection with conducting its pivotal Phase 3 clinical program for Fovista and, if such trials are successful, seeking marketing approval for Fovista. The Company also expects that its research and development expenses will increase as it further evaluates the potential benefit of Fovista in wet AMD, when administered in combination with anti-VEGF therapy, and in other ophthalmic diseases and conditions with unmet medical need, and as a result of its plan to initiate, in the fourth quarter of 2015, a Phase 2/3 clinical trial to evaluate the safety and efficacy of Zimura in patients with GA. The Company expects these research and development expenses to increase as additional trials are initiated and as patient enrollment increases in trials that have already commenced. In addition, the Company expects that it will incur significant expenses related to manufacturing validation activities associated with Fovista and process development and manufacturing scale-up activities associated with Zimura.

Income Taxes

The Company utilizes the liability method of accounting for deferred income taxes, as set forth in ASC 740-10, *Income Taxes—Overall*. Under this method, deferred tax liabilities and assets are recognized for the expected future tax consequences of temporary differences between the carrying amounts and the tax basis of assets and liabilities. The Company incurred U.S. federal net operating losses ("NOLs") in each year from its inception in 2007 through 2013 and utilized these NOLs in 2014. 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.

Table of Contents

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 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 option grants that do not vest immediately upon grant are recorded as an expense over the vesting period of the underlying stock options. At the end of each financial reporting period prior to vesting, the value of these options, as calculated using the Black-Scholes option-pricing model, will be re-measured using the fair value of the Company's common stock and the non-cash expense recognized during the period will be adjusted accordingly. Since the fair value of options granted to 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 September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
Research and development	$ 5,116	$ 1,928	$ 12,535	$ 5,535
General and administrative	1,925	1,652	5,939	3,957
Total	$ 7,041	$ 3,580	$ 18,474	$ 9,492

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. ASU 2014-09 is effective for public entities for annual reporting periods beginning after December 15, 2016 and interim periods within those annual periods. In August 2015, the FASB issued ASU 2015-14, *Revenue from Contracts with Customers (Topic 606): Deferral of the Effective Date*, which provides 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 consolidated financial statements.

3. Capitalization

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

Table of Contents

convertible preferred stock outstanding at the time of the offering were automatically converted into 21,038,477 shares of common stock.

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

4. Net Income (Loss) Per Common Share

Basic and diluted net income (loss) per common share is determined by dividing net income (loss) 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 loss per common share because their effect would be anti-dilutive. Therefore, the weighted average shares used to calculate both basic and diluted loss per share would be the same. The following table sets forth the computation of basic and diluted net income (loss) per common share for the periods indicated:

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
Basic and diluted net income (loss) per common share calculation:				
Net income (loss)	$ (39,573)	$ 8,552	$ (70,068)	$ (85,120)
Weighted average common shares outstanding - basic	34,782	33,531	34,432	33,074
Plus: effect of dilutive stock options, warrants and unvested restricted stock units	—	1,328	—	—
Weighted average common shares outstanding- dilutive	34,782	34,859	34,432	33,074
Net income (loss) per share of common stock - basic	$ (1.14)	$ 0.26	$ (2.03)	$ (2.57)
Net income (loss) per share of common stock - diluted	$ (1.14)	$ 0.25	$ (2.03)	$ (2.57)

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:

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
Options outstanding	3,224	1,503	3,224	2,244
Warrants	—	—	—	14
Restricted stock units	285	4	285	17
Total	3,509	1,507	3,509	2,275

5. 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 $1.8 million and $4.7 million as of September 30, 2015 and December 31, 2014, respectively. Cash and cash equivalents as of September 30, 2015 and December 31, 2014 also included investments of $253.1 million and $35.1 million, respectively, in U.S. Treasury securities with original maturities of 90 days or less and investments in money market funds.

The Company considers securities with original maturities of greater than 90 days to be available for sale securities. The Company held available for sale securities with a fair value totaling $171.1 million and $423.7 million as of September 30, 2015 and December 31, 2014, respectively. These available for sale securities consisted of U.S. Treasury securities and investment-grade corporate debt securities. At September 30, 2015, the Company held available for sale securities of $26.1 million with maturities less than one year, and $144.9 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 September 30, 2015. 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:

Table of Contents

	As of September 30, 2015			
	Cost	Fair Value	Carrying Value	Unrealized Gain
U.S. Treasury securities	$ 106,340	$ 106,464	$ 106,464	$ 124
Corporate debt securities	64,612	64,626	64,626	14

Total	$ 170,952	$ 171,090	$ 171,090	$ 138

	As of December 31, 2014			
	Cost	Fair Value	Carrying Value	Unrealized Loss
U.S. Treasury securities	$ 423,859	$ 423,746	$ 423,746	$ (113)
Corporate debt securities	—	—	—	—
Total	$ 423,859	$ 423,746	$ 423,746	$ (113)

The Company's available for sale securities are reported at fair value on the Company's balance sheet. Unrealized gains and losses are reported within accumulated other comprehensive income (loss) in the statements of comprehensive income (loss). The changes in accumulated other comprehensive income (loss) associated with the unrealized gain on available for sale securities during the three and nine months ended September 30, 2015 and September 30, 2014 were as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
Beginning balance	$ 24	$ 25	$ (65)	$ —
Current period changes in fair value before reclassifications, net of tax	85	10	174	35
Amounts reclassified from accumulated other comprehensive income, net of tax	(30)	—	(30)	—
Total other comprehensive income, net of tax	55	10	144	35
Ending balance	$ 79	$ 35	$ 79	$ 35

6. Licensing and Commercialization Agreement with Novartis Pharma AG

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

Table of Contents

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 approval milestones, including reimbursement approval, in certain countries in the Novartis Territory. In addition, Novartis has agreed to pay the Company up to an aggregate of an additional $400.0 million if Novartis achieves specified sales milestones in the Novartis Territory. Novartis also is obligated to pay the Company royalties with respect to standalone Fovista products and combination Fovista products that Novartis successfully commercializes. The Company will receive royalties at a mid-thirties percentage of net sales of standalone Fovista products and a royalty of approximately equal value for sales of combination Fovista products. Such royalties are subject to customary deductions, credits, and reductions for lack of patent coverage or market exclusivity. Novartis's obligation to pay such royalties will continue on a licensed product-by-licensed product and country-by-country basis until Novartis's last actual commercial sale of such licensed product in such country. The Company will continue to be responsible for royalties it owes to third parties on sales of Fovista products.

Novartis has agreed to pay the Company's manufacturing costs plus a specified percentage margin for 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 obtain supplies of products in pre-filled syringes from a third party manufacturer and pay Novartis a low single-digit percentage of the Company's net sales of such products.

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

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

Novartis has agreed to specified limitations on its ability to in-license, acquire or commercialize any anti-PDGF product that does not contain Fovista (an "Alternative Anti-PDGF Product") in the Novartis Territory and, to the extent Novartis develops, in-licenses or acquires such a product, to make such product available to the Company in the United States under specified option

conditions. If the Company exercises its option, the Company will be obligated to make certain payments to Novartis, including specified milestone and royalty payments. The amounts of such payments will vary based on the product's stage of clinical development at the time the Company 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 the certain approval rights of the Company, to sublicense the exclusive royalty-bearing license to commercialize Fovista in the Novartis Territory. The Company's obligation to provide access to clinical and regulatory information as part of the License Deliverable includes the obligation to provide access to all clinical data, regulatory filings, safety data and manufacturing data to Novartis which is necessary for commercialization of Fovista in the Novartis Territory. The R&D Activity Deliverable includes the right and responsibility for the Company to conduct the Phase 3 Fovista clinical program and other studies of Fovista in the Novartis Territory which are necessary or desirable for regulatory approval or commercialization of Fovista. The Manufacturing Deliverable includes the obligation for the Company to supply API to Novartis for development and 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 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, with the specific amount of such fee determined based on the effective date of the termination. The Company has concluded that this termination provision constitutes a contingent event that 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 of the regulatory milestones that may be achieved under the Novartis Agreement do not meet the recognition criteria within the definition of a milestone included in ASU 2010-17, *Revenue Recognition—Milestone Method*, and therefore, payments received for the achievement of the enrollment milestones in excess of the termination fee will be included in the allocable arrangement consideration and allocated to the deliverables based upon BESP using the relative selling price method.

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

Table of Contents

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 recognized revenue of approximately $46.7 million during the nine months ended September 30, 2015, which primarily related to the $50.0 million milestone it achieved in March 2015. The balance of the milestone payment was recorded as deferred revenue. Below is a summary of the components of the Company's collaboration revenue for the three and nine months ended September 30, 2015 and 2014:

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
License revenue	$ —	$ 38,373	$ 38,083	$ 38,373
Research and development activity revenue	1,594	1,200	6,768	1,200
API transfer revenue	1,851	—	1,851	—
Joint operating committee revenue	3	2	21	2
Total collaboration revenue	$ 3,448	$ 39,575	$ 46,723	$ 39,575

7. Financing Agreement with Novo A/S

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

The Novo Agreement provided for up to three separate purchases for a purchase price of $41.7 million each, at a first, second and third closing, for an aggregate purchase price of $125.0 million. In each purchase, Novo A/S 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 September 30, 2015, 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.

8. Income Taxes

The Company utilizes the liability method of accounting for deferred income taxes. Under this method, deferred tax liabilities and assets are recognized for the expected future tax consequences of temporary differences between the carrying amounts and the tax basis of assets and liabilities. A valuation allowance is established against deferred tax assets when, based on the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. The Company's policy is to record interest and penalties on uncertain tax positions as income tax expense. As of September 30, 2015 and December 31, 2014, the

Table of Contents

Company does not believe any material uncertain tax positions were present. Accordingly, the Company has not accrued any interest or penalties due to uncertain tax positions.

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 2015. As such, the Company has recorded a benefit from income taxes during the three and nine months ended September 30, 2015. The Company currently expects to realize its net deferred tax assets recorded as of September 30, 2015 due to the Company's ability to carryback its projected federal tax losses to 2014. 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.

9. Fair Value Measurements

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

The 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 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 Company did not make any reclassifications of Level 1 and Level 2 assets during the three months ended September 30, 2015. The following table presents, for each of the fair value hierarchy levels required under ASC 820, the Company's assets and liabilities that are measured at fair value on a recurring basis as of September 30, 2015:

Table of Contents

	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*	$ 253,068	$ —	$ —
Investments in U.S. Treasury securities	$ 106,464	$ —	$ —
Investments in Corporate debt securities	$ —	$ 64,626	$ —

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

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

* 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.

10. Stock Option and Compensation Plans

The Company adopted its 2007 Stock Incentive Plan (the "2007 Plan") for employees, directors and consultants for the purpose of advancing the interests of the Company'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 option awards, restricted stock awards, and other stock-based and cash-based awards. Following the effectiveness of the 2013 Stock Incentive Plan described below in connection with the closing of the Company's initial public offering, the Company is no longer granting additional awards under the 2007 Plan.

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

In connection with the evergreen provisions of the 2013 Plan, the number of shares available for issuance under the 2013 Plan was increased by approximately 1,257,000 shares, effective as of January 1, 2014 and an additional approximately 1,360,000 shares effective as of January 1, 2015. As of September 30, 2015, the Company had approximately 1,152,000 shares available for grant under the 2013 Plan.

Table of Contents

On September 30, 2014, as an inducement grant issued outside the Company's existing equity compensation plan in accordance with NASDAQ listing rule 5635(c)(4) (an "inducement grant"), the Company issued to an employee an option to purchase 200,000 shares of its common stock at an exercise price of $38.93 per share. On August 31, 2015, outside of its existing equity compensation plan and as an inducement grant, the Company issued to a different employee an option to purchase 120,000 shares of its common stock at an exercise price of $44.03 per share.

Cash proceeds from, and the aggregate intrinsic value of, stock options exercised during the three and nine months ended September 30, 2015 and 2014, respectively, were as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
Cash Proceeds from options exercised	$ 1,813	$ 921	$ 6,024	$ 1,221
Aggregate intrinsic value of options exercised	$ 10,092	$ 3,785	$ 39,033	$ 6,884

A summary of the stock options outstanding and exercisable as of September 30, 2015 is as follows:

	As of September 30, 2015				
	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	527	6.9	$ 7.74	225	$ 5.53
$10.04-$20.00	366	6.8	$ 13.43	123	$ 13.49
$20.01-$30.00	169	8.1	$ 25.63	71	$ 25.54
$30.01-$40.00	1,405	8.4	$ 33.13	551	$ 32.94
$40.01-$55.00	709	9.4	$ 45.53	25	$ 42.77
$55.01-$70.34	48	9.8	$ 70.34	—	$ —
	3,224			995	
Aggregate Intrinsic Value	$ 40,098			$ 16,431	

In connection with stock option awards granted to employees, the Company recognized share-based compensation expense of approximately $3.4 million and $3.1 million for the three months ended September 30, 2015 and 2014, respectively, net of expected forfeitures. In connection with stock option awards granted to employees, the Company recognized share-based compensation expense of approximately $11.4 million and $8.3 million for the nine months ended September 30, 2015 and 2014, respectively, net of expected forfeitures. As of September 30, 2015, there was approximately $41.2 million of unrecognized compensation costs, net of estimated forfeitures, related to stock option awards to employees, which are expected to be recognized over a remaining weighted average period of 2.8 years.

In connection with stock options awards granted to consultants, the Company recognized approximately $1.8 million and $0.4 million in share-based compensation expense during the three months ended September 30, 2015 and 2014, respectively, net of expected forfeitures. In connection with stock options awards granted to consultants, the Company recognized approximately $3.4 million and $1.0 million in share-based compensation expense during the nine months ended September 30, 2015 and 2014, respectively, net of expected forfeitures. As of September 30, 2015, there was approximately $2.4 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 2.3 years.

As of September 30, 2015, the Company had approximately 285,000 RSUs outstanding. In connection with RSUs granted to employees, the Company recognized share-based compensation expense of approximately $1.8 million and $0.1 million during the three months ended September 30, 2015 and 2014, net of expected forfeitures. In connection with RSUs granted to employees, the Company recognized share-based compensation expense of approximately $3.7 million and $0.2 million during the nine months ended September 30, 2015 and 2014, net of expected forfeitures. As of September 30, 2015, there was approximately $8.8 million of unrecognized compensation costs, net of estimated forfeitures, related to RSUs granted to employees, which are expected to be recognized over a remaining weighted average period of 2.8 years.

Table of Contents

11. Property and Equipment

Property and equipment as of September 30, 2015 and December 31, 2014 were as follows:

	Useful Life (Years)	September 30, 2015	December 31, 2014
Manufacturing and clinical equipment	7-10	$ 617	$ 617
Computer and other office equipment	5	916	292
Furniture and fixtures	7	630	591
Leasehold improvements	3-5	463	357
Construction in progress		701	—
		3,327	1,857
Accumulated depreciation		(737)	(302)
Property and equipment, net		$ 2,590	$ 1,555

For the three and nine months ended September 30, 2015, depreciation expense was $77 thousand and $535 thousand, respectively, and includes amounts incurred related to the relocation of the Company's office facilities in Princeton, New Jersey. For the three and nine months ended September 30, 2014, depreciation expense was $36 thousand and $80 thousand, respectively.

12. Restatement of Previously Issued Financial Statements

On July 28, 2015, the Company restated its unaudited financial statements for the quarters ended June 30, 2014, September 30, 2014 and December 31, 2014, its audited financial statements for the year ended December 31, 2014 and its unaudited financial statements for the quarter ended March 31, 2015, to correct the Company's accounting for certain valuation allowances related to deferred tax assets.

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

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

The following discussion of our financial condition and results of operations should be read in conjunction with our financial statements and the notes to those financial statements appearing elsewhere in this Quarterly Report on Form 10-Q and the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the year ended December 31, 2014 included in our Annual Report on Form 10-K/A filed with the Securities and Exchange Commission on July 28, 2015. 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 therapy that represents 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) and have completed patient recruitment for two Phase 3 clinical trials of Fovista administered in combination with Lucentis. We are also developing our product candidate Zimura® as a monotherapy for the treatment of patients with geographic atrophy, or GA, a form of dry AMD, and in combination with anti-VEGF therapy for the treatment of wet AMD in patients with suboptimal response to anti-VEGF monotherapy, as well as 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.

Table of Contents

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 Fovista administered in combination with anti-VEGF therapy 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® (aflibercept) or Avastin® (bevacizumab) compared to Eylea or Avastin monotherapy. Our development strategy for Fovista is to be agnostic with respect to the choice of the anti-VEGF therapy administered in combination with Fovista.

We completed patient recruitment in one of the Fovista Phase 3 Lucentis Trials in May 2015 and in the other Fovista Phase 3 Lucentis Trial in October 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 database from both of the Fovista Phase 3 Lucentis Trials will be locked and analyzed together, which will allow for the pooled analysis for 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. This trial is investigating Fovista in combination with either Eylea or Avastin compared to either Eylea or Avastin monotherapy. Our Phase 2b trial utilized Lucentis as the only anti-VEGF therapy 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 complete the assessment of initial preclinical and clinical studies 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 obtaining favorable data from the Phase 3 clinical program. We are continuing to explore various regulatory filing options. We anticipate that we will 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 obtaining favorable data 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 either initiated, or have plans to initiate in the near future, additional clinical trials to evaluate the potential additional benefits of Fovista administered in combination with anti-VEGF therapy in wet AMD patients. We refer to these trials as the Fovista Expansion Studies.

During the third quarter of 2014, we initiated an open-label Phase 2a clinical trial to investigate the optimal regimen of Fovista, when administered in combination with anti-VEGF therapy (Lucentis, Eylea or Avastin), in reducing the formation of subretinal fibrosis in wet AMD patients. As part of this trial, we are investigating in a subset of patients the effect of receiving pre-treatment with Fovista prior to receiving combination therapy of Fovista with an anti-VEGF agent. We completed enrollment in May 2015 with a total of 101 patients enrolled in this 24-month trial.

During the fourth quarter of 2014, we initiated an open-label Phase 2a clinical trial to evaluate the optimal regimen of Fovista, when administered in combination with anti-VEGF therapy (Lucentis, Eylea or Avastin), in reducing the treatment burden for wet AMD patients. We completed enrollment in October 2015 with a total of 64 patients enrolled in this 18-month trial.

We plan to initiate, in late 2015 or early 2016, a randomized, double-masked, controlled Phase 2b clinical trial to evaluate the safety and efficacy of Fovista administered in combination with Avastin for the treatment of wet AMD compared to Avastin monotherapy. Following an induction period, patients will be treated bimonthly with either combination therapy of Fovista with Avastin or Avastin monotherapy. We plan to begin by enrolling patients in the Netherlands, where we believe Avastin is the current standard of care anti-VEGF agent for the treatment of wet AMD. As the trial progresses, we may expand the trial to include sites in additional countries.

Table of Contents

We also plan to initiate, in late 2015 or early 2016, 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 therapy. This trial will include both wet AMD patients who have previously received anti-VEGF treatment, whom we refer to as treatment experienced, as well as wet AMD patients who have not previously received anti-VEGF treatment, whom we refer to as treatment naïve.

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 product to which Novartis has rights in a co-formulated product, for the treatment, prevention, cure or control of any human disease, disorder or condition of the eye, and to develop and commercialize those licensed products in all countries outside of the United States, which we refer to as the Novartis Territory. We have agreed to use commercially reasonable efforts to complete our ongoing pivotal Phase 3 clinical program for Fovista and Novartis has agreed to use commercially reasonable efforts to develop a standalone Fovista product and a co-formulated product containing Fovista and an anti-VEGF to which Novartis has rights, as well as a pre-filled syringe presentation of such products and to use commercially reasonable efforts, subject to obtaining marketing approval, to commercialize licensed products in the Novartis Territory in accordance with agreed development and marketing plans.

Novartis paid us a $200.0 million upfront fee upon execution of the Novartis Agreement. 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 marketing approval milestones in certain countries in the Novartis Territory. In addition, Novartis has agreed to pay us up to an aggregate of an additional $400.0 million if Novartis achieves specified

sales milestones in the Novartis Territory. Novartis also is obligated to pay us royalties with respect to standalone Fovista products and combination Fovista products that Novartis successfully commercializes. We will receive royalties at a mid-thirties percentage of net sales of standalone Fovista products and a royalty of approximately equal value for sales of combination Fovista products. Such royalties are subject to customary deductions, credits, and reductions for lack of patent coverage or market exclusivity. Novartis's obligation to pay such royalties will continue on a licensed product-by-licensed product and country-by-country basis until Novartis's last actual sale of such licensed product in such country. We will continue to be responsible for royalties we owe to third parties on sales of Fovista products.

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

Zimura Clinical Development

We plan to initiate, by the end of 2015, 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 either: 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 the interim analysis, a determination will be made based on safety and efficacy parameters whether to expand the trial and enroll additional patients. Patients in the trial will receive monthly injections over the 24-month study period.

We plan to initiate, in late 2015 or early 2016, an open-label Phase 2a clinical trial to evaluate the safety and efficacy of Zimura administered in combination with anti-VEGF therapy for the treatment of wet AMD in treatment experienced patients with a suboptimal response to anti-VEGF monotherapy.

Table of Contents

We also initiated in late 2014 a very small, open-label Phase 2 clinical trial investigating Zimura administered in combination with anti-VEGF therapy for the treatment of polypoidal choroidal vasculopathy, or PCV, a specific type of wet AMD, in patients who do not respond adequately to treatment with anti-VEGF monotherapy or for whom anti-VEGF monotherapy fails.

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 September 30, 2015, we had an accumulated deficit of $369.9 million. Our net loss was $70.1 million for the nine months ended September 30, 2015, and $116.8 million for the year ended December 31, 2014, and we expect to continue to incur significant operating losses in 2015 and in the future. 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 substantially, particularly as we continue the development of Fovista in our Phase 3 clinical program. We initiated our pivotal Phase 3 clinical program for Fovista in August 2013. We plan to enroll a total of approximately 1,866 patients for this program. In addition, we also expect our expenses to increase as we further evaluate the potential benefit of Fovista when administered in combination with anti-VEGF therapy in wet AMD patients through the Fovista Expansion Studies, and potentially in other ophthalmic diseases and conditions with unmet 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 also exploring the potential of an ophthalmic formulation for tivozanib, an anti-VEGF compound 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. We are party to agreements, specifically an asset acquisition agreement with OSI (Eyetech), Inc., which agreement is now held by OSI Pharmaceuticals, LLC, a subsidiary of Astellas US, LLC, and license agreements with Archemix Corp. and Nektar Therapeutics, that impose significant milestone payment obligations on us in connection with our achievement of specific clinical, regulatory and commercial milestones with respect to Fovista and Zimura. Furthermore, we are incurring and expect to continue to incur additional costs associated with operating as a public company, hiring additional personnel and expanding our facilities. These costs include significant legal, compliance, accounting, and investor and public relations expenses, as well as increased insurance premiums. 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.

Table of Contents

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 earned a $50.0 million enrollment-based milestone, or $100.0 million in aggregate, under the Novartis Agreement. We recognized revenue of approximately $46.7 million during the nine months ended September 30, 2015, which primarily related to the $50.0 million milestone we achieved in March 2015. The balance of the milestone payment was recorded as deferred revenue. 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 and nine months ended September 30, 2015 and 2014:

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
	(in thousands)			
License revenue	$ —	$ 38,373	$ 38,083	$ 38,373
Research and development activity revenue	1,594	1,200	6,768	1,200
API transfer revenue	1,851	—	1,851	—
Joint operating committee revenue	3	2	21	2
Total collaboration revenue	$ 3,448	$ 39,575	$ 46,723	$ 39,575

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 manufacturing development and clinical testing 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 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*. We account for non-refundable advance payments for goods and services that will be used in future research and development activities as expenses when the service has been performed or when the goods have been received, rather than when the payment is made.

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

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

The following table summarizes our research and development expenses for the three and nine months ended September 30, 2015 and 2014:

Table of Contents

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
	(in thousands)			
Fovista	$ 27,351	$ 11,842	$ 65,098	$ 52,520
Zimura	2,391	800	5,548	1,799

Personnel related	4,083	2,335	10,995	6,093
Share-based compensation	5,116	1,928	12,535	5,535
Other	1,538	200	2,919	242
	$ 40,479	$ 17,105	$ 97,095	$ 66,189

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 therapy 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, including the initiation of our Phase 2/3 Zimura GA trial by the end of 2015. 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 or the scale-up of manufacturing activities 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 through at least 2017, 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. Furthermore, we are at the early stages of clinical development for Zimura, which we expect will continue for at least the next several years. At this time, we cannot reasonably estimate the remaining costs necessary to complete the clinical development of either Fovista or Zimura, complete manufacturing validation activities associated with Fovista and process development and manufacturing scale-up and validation activities associated with Zimura and seek marketing approval for Fovista or Zimura, or the nature, timing or costs of the efforts necessary to complete the development of any other product candidate we may develop.

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

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

Table of Contents

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 and business development functions. Other general and administrative expenses include facility costs and professional fees for legal, patent, consulting and accounting services.

We anticipate that our general and administrative expenses will increase in future periods to support increases in our research and development, manufacturing, and commercialization activities and as a result of increased personnel, including management personnel to support our 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 for which we have not yet been invoiced.

We base our expenses related to CROs and CMOs on our estimates of the services received and efforts expended pursuant to quotes 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 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.

Table of Contents

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 recognized collaboration revenue of approximately $46.7 million during the nine months ended September 30, 2015, which primarily related to the $50.0 million milestone we achieved in March 2015. The balance of the milestone payment was recorded as deferred revenue. During the three months ended September 30, 2015, we recognized collaboration revenue of approximately $3.4 million. 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 and nine months ended September 30 2015 and 2014:

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
	(in thousands)			
License revenue	$ —	$ 38,373	$ 38,083	$ 38,373
Research and development activity revenue	1,594	1,200	6,768	1,200
API transfer revenue	1,851	—	1,851	—
Joint operating committee revenue	3	2	21	2
Total collaboration revenue	$ 3,448	$ 39,575	$ 46,723	$ 39,575

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 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.

Table of Contents

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. For example, a milestone payment may be due to us upon the FDA's acceptance of an NDA. Commercial milestones are typically achieved when an approved pharmaceutical product reaches certain defined levels of net sales by the licensee, such as when a product first achieves global sales or annual sales of a specified amount.

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

Royalty Purchase Liability

The proceeds from the financing we received under the Novo Agreement have been recorded as a liability on our balance sheet in accordance with ASC 730, *Research and Development*. Because there is a significant related party relationship between us and Novo A/S, we are treating our obligation to make royalty payments under the Novo Agreement as an implicit obligation to repay the funds advanced by Novo A/S, and thus have recorded the proceeds as a liability on our balance sheet. As we make royalty payments to Novo A/S in accordance with the Novo Agreement, we will reduce the liability balance. At the time that such royalty payments become probable and estimable, and if such amounts exceed the liability balance, we will impute interest accordingly on a prospective basis based on such estimates, which would result in a corresponding increase in the liability balance.

Share-Based Compensation

We account for all share-based compensation payments issued to employees, directors, and 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. 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 information is available and will continue to do so until the historical volatility of our common stock is sufficient to measure expected volatility for future option grants.

We use the simplified method as prescribed by the Securities and Exchange Commission Staff Accounting Bulletin No. 107, *Share-Based Payment*, to calculate the expected term of stock option grants to employees as we do not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term of stock options granted to employees. The risk-free interest rate used for each grant is based on the U.S. Treasury yield curve in effect at the time of grant for instruments with a similar expected life. We utilize a dividend yield of zero based on the fact that we have never paid cash dividends and have no current intention to pay cash dividends. The weighted-average assumptions used to estimate grant date fair value of stock options using the Black-Scholes option pricing model were as follows for the three and nine months ended September 30, 2015 and 2014:

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
Expected common stock price volatility	71%	77%	72%	83%
Risk-free interest rate	1.70%-2.04%	1.84%-2.48%	1.35%-2.04%	1.61%-2.48%
Expected term of options (years)	6.3	6.3	6.2	6.3
Expected annual dividend per share	$ —	$ —	$ —	$ —

We estimate the fair value of 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 $7.0 million for the three months ended September 30, 2015 and $3.6 million for the three months ended September 30, 2014. Share-based compensation expense for equity grants to employees, non-employee directors and consultants was $18.5 million for the nine months ended September 30, 2015 and $9.5 million for the nine months ended September 30, 2014. As of September 30, 2015, we had $52.4 million of total unrecognized share-based compensation expense, which we expect to recognize over a weighted-average remaining vesting period of approximately 2.7 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 and nine months ended September 30, 2015 and 2014, we allocated share-based compensation as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
	(in thousands)			
Research and development	$ 5,116	$ 1,928	$ 12,535	$ 5,535
General and administrative	1,925	1,652	5,939	3,957
Total	$ 7,041	$ 3,580	$ 18,474	$ 9,492

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 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. The valuation allowance on certain of our deferred tax assets has been released, where appropriate. We are projecting tax losses for 2015 and as such, we recorded a benefit for income taxes of approximately $7.5 million during the three months ended September 30, 2015, and a benefit from income taxes of approximately $11.6 million during the nine months ended September 30, 2015. See Note 8 to our financial statements in Part I-Item 1 of this Quarterly Report on form 10-Q for further information regarding our expectations with respect to our income tax provision.

Results of Operations

Comparison of Three Month Periods Ended September 30, 2015 and 2014

	Three months ended September 30,		Increase (Decrease)
	2015	2014	
	(in thousands)		
Statement of Operations Data:			
Collaboration revenue	$ 3,448	$ 39,575	$ (36,127)
Operating expenses:			
Research and development	40,479	17,105	23,374
General and administrative	10,412	8,812	1,600
Total operating expenses	50,891	25,917	24,974
Income (loss) from operations	(47,443)	13,658	(61,101)
Interest income and other	339	73	266
Income (loss) before income tax provision	(47,104)	13,731	(60,835)
Income tax (benefit) provision	(7,531)	5,179	(12,710)
Net income (loss)	$ (39,573)	$ 8,552	$ (48,125)

Collaboration Revenue

Collaboration revenue for the three months ended September 30, 2015 was $3.4 million, a decrease of $36.1 million compared to $39.6 million for the three months ended September 30, 2014. Using the relative selling price method, for the three months ended September 30, 2015, we recognized $1.6 million related to the research and development activities we performed under the Novartis Agreement, $1.9 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 September 30, 2014 was $39.6 million, of which $38.4 million was allocated to the license delivered to Novartis under the Novartis Agreement and $1.2 million was allocated to research and development activities we performed under the Novartis Agreement during the same period.

Research and Development Expenses

Our research and development expenses were $40.5 million for the three months ended September 30, 2015, an increase of $23.4 million compared to $17.1 million for the three months ended September 30, 2014. The increase was primarily due to costs associated with our Fovista Phase 3 clinical program, including clinical trial costs and the costs to manufacture Fovista for the clinical trials, as well as increased manufacturing costs related to our Zimura program. Also contributing to the increase were higher compensation expenses, including share-based compensation, associated with additional research and development staffing.

General and Administrative Expenses

Our general and administrative expenses were $10.4 million for the three months ended September 30, 2015, an increase of $1.6 million compared to $8.8 million for the three months ended September 30, 2014. The increase was primarily due to an increase in costs to support the expansion of our operations, including our public company infrastructure, and the hiring of additional management and corporate staffing, including the early stages of a commercial organization. Also contributing to the increase were higher share-based compensation, and professional services and consulting fees.

Interest Income

Interest income for the three months ended September 30, 2015 was $0.3 million compared to interest income of $0.1 million for the three months ended September 30, 2014. The increase in interest income earned during the three months ended September 30, 2015 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.

Income tax (benefit) provision

During the three months ended September 30, 2015, we recorded a benefit from income taxes of approximately $7.5 million, which related to our projected tax losses for tax year 2015 and our ability to carry these losses back to 2014 to recapture a portion of the federal income tax payments we paid in 2014. During the three months ended September 30, 2014, we recorded a provision for income taxes of approximately $5.2 million, which primarily related to taxable income that resulted from payments we received under the Novartis Agreement and the Novo Agreement in 2014.

Table of Contents

Comparison of Nine Month Periods Ended September 30, 2015 and 2014

	Nine months ended September 30, 2015	2014	Increase (Decrease)
	(in thousands)		
Statement of Operations Data:			
Collaboration revenue	$ 46,723	$ 39,575	$ 7,148
Operating expenses:			
Research and development	97,095	66,189	30,906
General and administrative	31,955	22,731	9,224
Total operating expenses	129,050	88,920	40,130
Loss from operations	(82,327)	(49,345)	32,982
Interest income and other	630	189	441
Loss before income tax provision	(81,697)	(49,156)	32,541
Income tax (benefit) provision	(11,629)	35,964	(47,593)
Net loss	$ (70,068)	$ (85,120)	$ (15,052)

Collaboration Revenue

Collaboration revenue for the nine months ended September 30, 2015 was $46.7 million, an increase of $7.1 million compared to $39.6 million for the nine months ended September 30, 2014. Using the relative selling price method, for the nine months ended September 30, 2015, we allocated $38.1 million to the license delivered to Novartis under the Novartis Agreement, $6.8 million to research and development activities performed under the Novartis Agreement, $1.9 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 nine months ended September 30, 2014 was $39.6 million, of which $38.4 million was allocated to the license delivered to Novartis under the Novartis Agreement and $1.2 million was allocated to research and development activities we performed under the Novartis Agreement during the same period.

Research and Development Expenses

Our research and development expenses were $97.1 million for the nine months ended September 30, 2015, an increase of $30.9 million compared to $66.2 million for the nine months ended September 30, 2014. Research and development expenses during the nine months ended September 30, 2014 included a milestone payment of $19.8 million that we paid in June 2014 in connection with our entry into the Novartis Agreement. Excluding this milestone payment, research and development expenses increased by approximately $50.7 million for the nine months ended September 30, 2015 as compared to the nine months ended September 30, 2014. The increase was primarily due to costs associated with our Fovista Phase 3 clinical program, including clinical trial costs and the costs to manufacture Fovista for the clinical trials, as well as increased manufacturing costs related to our Zimura program. Other contributing factors include increased personnel costs associated with additional management and research and development staffing, including share-based compensation expense.

General and Administrative Expenses

Our general and administrative expenses were $32.0 million for the nine months ended September 30, 2015, an increase of $9.3 million compared to $22.7 million for the nine months ended September 30, 2014. The increase was primarily due to an increase in costs to support the expansion of our operations, including our public company infrastructure, and the hiring of additional management and corporate staffing, including the early stages of a commercial organization. Also contributing to the increase were higher share-based compensation, and professional services and consulting fees.

Interest Income

Interest income for the nine months ended September 30, 2015 was $0.6 million compared to interest income of $0.2 million for the nine months ended September 30, 2014. Interest income earned during the nine months ended September 30, 2015 was a result of an increase in our cash, cash equivalents and marketable securities average balances during the nine months ended September 30, 2015 as compared to the nine months ended September 30, 2014. Also contributing to the increase was a change in the mix of our

Table of Contents

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

Income tax (benefit) provision

During the nine months ended September 30, 2015, we recorded a benefit from income taxes of approximately $11.6 million, which related to our projected tax losses for tax year 2015 and our ability to carry these losses back to 2014 to recapture a portion of the federal income tax payments we paid in 2014. During the nine months ended September 30, 2014, we recorded a provision for income taxes of approximately $36.0 million, which primarily related to taxable income that resulted from payments we received under the Novartis Agreement and the Novo Agreement in 2014.

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 under one of our license agreements. 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 September 30, 2015, we had cash, cash equivalents and marketable securities totaling $426.0 million and no debt. We primarily invest our cash, cash equivalents and marketable securities in U.S. Treasury securities, money market funds that invest in U.S. Treasury securities and certain investment-grade corporate debt securities.

The following table shows a summary of our cash flows for the nine months ended September 30, 2015 and 2014:

	Nine months ended September 30,	
	2015	2014
	(in thousands)	
Net cash provided by (used in) :		
Operating Activities	$ (39,495)	$ 100,321
Investing Activities	248,552	(316,745)

Financing Activities	6,024	100,632
Net change in cash and cash equivalents	$ 215,081	$ (115,792)

Table of Contents

Cash Flows from Operating Activities

Net cash used in operating activities of $39.5 million for the nine months ended September 30, 2015 relates primarily to our net loss adjusted for non-cash charges and changes in the components of working capital. The increase in net cash used in the nine months ended September 30, 2015 compared to the net cash provided by operating activities during the nine months ended September 30, 2014 of $100.3 million relates primarily to the $200.0 million upfront payment we received in connection with our entry into the Novartis Agreement, offset by a milestone payment of approximately $19.8 million that we paid during the nine months ended September 30, 2014 in connection with our entry into this Agreement. The increase in net cash used also relates to increased expenditures in our efforts to advance Fovista in Phase 3 clinical trials, including increased spending on Phase 3 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 provided by investing activities for the nine months ended September 30, 2015 was $248.6 million and relates primarily to the sale or maturity of marketable securities totaling $633.0 million offset by purchases of marketable securities totaling $383.0 million and capital expenditures associated with the expansion of our office facilities in New York, New York and the relocation to a new office facility in Princeton, New Jersey. Net cash used in investing activities for the nine months ended September 30, 2014 was $316.7 million, which relates primarily to the purchase of marketable securities totaling $385.4 million, offset by maturities of marketable securities of $70.0 million.

Cash Flows from Financing Activities

Net cash provided by financing activities was $6.0 million for the nine months ended September 30, 2015 and $100.6 million for the nine months ended September 30, 2014. Net cash provided by financing activities for the nine months ended September 30, 2015 consisted of proceeds from stock option exercises. Net cash provided by financing activities for the nine months ended September 30, 2014 consisted primarily of proceeds of $55.4 million from our follow-on public offering in February 2014, and proceeds of $41.7 million from our royalty agreement with Novo A/S in January 2014.

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. We initiated our pivotal Phase 3 clinical program for Fovista in August 2013. We plan to enroll a total of approximately 1,866 patients for this program. In addition, we also expect our expenses to increase as we further evaluate the potential benefit of Fovista when administered in combination with anti-VEGF therapy 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 also exploring the potential of an ophthalmic formulation for tivozanib, an anti-VEGF compound 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.

We are party to agreements, specifically an asset acquisition agreement with OSI (Eyetech), Inc., which agreement is now held by OSI Pharmaceuticals, LLC, a subsidiary of Astellas US, LLC, and license agreements with Archemix Corp., or Archemix, and Nektar Therapeutics, or Nektar, that impose significant milestone payment obligations on us in connection with our achievement of specific clinical, regulatory and commercial milestones with respect to Fovista 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.

Furthermore, we are incurring and expect to continue to incur additional costs associated with operating as a public company, hiring additional personnel and expanding our facilities. These costs include significant legal, compliance, accounting and investor and public relations expenses as well as increased insurance premiums. Moreover, additional rules and regulations applicable to public companies have increased our legal and financial compliance costs and have made, and will continue to make, some activities more time-consuming and costly.

Table of Contents

Our expenses also 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, for us 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 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, including personnel to support our product development and planned future commercialization efforts.

As of September 30, 2015, we had cash, cash equivalents, and marketable securities of $426.0 million. We also had $367.6 million in total liabilities, $339.7 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 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 or the scale-up of manufacturing activities 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 through at least 2017, 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. Furthermore, we are at the early stages of clinical development for Zimura, which we expect will continue for at least the next several years. At this time, we cannot reasonably estimate the remaining costs necessary to complete the clinical development of either Fovista or Zimura, complete 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.

Table of Contents

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 therapy, and potentially in other ophthalmic diseases and conditions with unmet need;
- the scope, progress, costs and results of our Zimura clinical programs, including our Phase 2/3 Zimura GA trial, our Phase 2a Zimura wet AMD Trial and our Phase 2a Zimura PCV trial, 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 review of Fovista and Zimura;
- the timing, scope and cost of commercialization activities for Fovista or Zimura if we receive, or expect to receive, marketing approval for either product candidate, including the costs and timing of expanding our outsourced manufacturing activities, including activities 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 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. 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

Table of Contents

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

Licensing and Commercialization Agreement with Novartis Pharma AG

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 agreement with Novartis, we granted Novartis exclusive rights under specified patent rights, know-how and trademarks controlled by us to manufacture, from bulk API supplied by us, standalone Fovista products and products combining Fovista with an anti-VEGF product to which Novartis has rights in a co-formulated product, for the treatment, prevention, cure or control of any human disease, disorder or condition of the eye, and to develop and commercialize those licensed products in all countries outside of the United States, which we refer to as the Novartis Territory. We have agreed to use commercially reasonable efforts to complete our ongoing pivotal Phase 3 clinical program for Fovista and Novartis has agreed to use commercially reasonable efforts to develop a standalone Fovista product and a co-formulated product containing Fovista and an anti-VEGF product to which Novartis has rights, as well as a pre-filled syringe presentation of such products and to use commercially reasonable efforts, subject to obtaining marketing approval, to commercialize licensed products in the Novartis Territory in accordance with agreed development and marketing plans. Novartis has also granted us options, subject to specified limitations, and to the extent such rights are controlled by Novartis, to obtain exclusive rights from Novartis to develop and commercialize in the United States the co-formulated and pre-filled syringe products developed by Novartis. We and Novartis have each granted the other options, subject to specified limitations, to obtain access to study data from certain clinical trials of licensed products that we or Novartis may conduct, including for use by the other in regulatory filings in its territory. We have agreed to exclusively supply Novartis, and Novartis has agreed to exclusively purchase from us, its clinical and commercial requirements for the bulk API 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 $200.0 million 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 ongoing pivotal 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 approval milestones, including reimbursement approval in certain countries in the Novartis Territory. In addition, Novartis has agreed to pay us up to an aggregate of an additional $400.0 million if Novartis achieves specified sales milestones in the Novartis Territory. Novartis also is obligated to pay us royalties with respect to standalone Fovista products and combination Fovista products that Novartis successfully commercializes. We will receive royalties at a mid-thirties percentage of net sales of standalone Fovista products and a royalty of approximately equal value for sales of combination Fovista products. Such royalties are subject to customary deductions, credits, and reductions for lack of patent coverage or market exclusivity. Novartis's obligation to pay such royalties will continue on a licensed product-by-licensed product and country-by-country basis until Novartis's last actual commercial sale of such licensed product in such country. We will continue to be responsible for royalties we owe to third parties on sales of Fovista products.

Novartis has agreed to pay our manufacturing costs plus a specified percentage margin for commercial supplies of the bulk API for Fovista that we supply to Novartis. If we or Novartis exercise our respective rights to obtain access to study data from clinical trials conducted by the other party, the party exercising the option will be obligated to pay the other party's associated past development costs and share with such other party any future associated development costs. If we exercise our option to obtain Novartis-controlled rights to develop, manufacture and commercialize any co-formulated Fovista product in the United States, we will be obligated to pay a specified percentage of Novartis's associated past development costs and share with Novartis any future associated development costs. 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 agent to which Novartis has rights in the Novartis Territory, for use in our ongoing Phase 3 clinical trials and ongoing Phase 2 trials and future Phase 2 and Phase 3 trials in the Novartis Territory following the effective date of the Novartis agreement. Novartis will have control over, and will be responsible for the costs of, all other clinical trials that may be required to obtain marketing approvals in the Novartis Territory for licensed products under the agreement. Novartis is also responsible for costs associated with co-formulation development, pre-filled syringe development and other development costs in the Novartis Territory, but excluding regulatory filing fees in the European Union for the standalone Fovista product, for which we will be responsible.

Table of Contents

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

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

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

Manufacturing and Supply Agreements with Agilent Technologies, Inc.

Clinical Supply Agreement

In May 2014, we entered into a Clinical Manufacturing and Supply Agreement with Agilent Technologies, Inc. pursuant to which Agilent has agreed to manufacture and supply to us, and we have agreed to purchase from Agilent, a specified percentage of our clinical requirements in specified jurisdictions of 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 Supply Agreement

On September 3, 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 specified jurisdictions of Fovista API. The commercial supply agreement has an initial term that runs for

Table of Contents

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 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 September 30, 2015:

	Payments Due by Period				
	Total	Less than 1 year	1-3 years	3-5 years	More than 5 years
			(in thousands)		
Operating Leases (1)	$ 9,913	$ 2,127	$ 5,521	$ 2,265	$ —
Purchase Obligations (2)	16,510	16,510	—	—	—
Total (3)	$ 26,423	$ 18,637	$ 5,521	$ 2,265	$ —

(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 September 30, 2015, 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 acquisition agreement with OSI (Eyetech), Inc., which agreement is now held by OSI Pharmaceuticals, LLC., or OSI Pharmaceuticals, a subsidiary of Astellas US, LLC, for rights to particular anti-PDGF aptamers, including Fovista, we are obligated to pay to OSI Pharmaceuticals future one-time payments of $12.0 million in the aggregate upon marketing approval in the United States and the European Union of a covered anti-PDGF product. We also are obligated to pay to OSI Pharmaceuticals a royalty at a low single-digit percentage of net sales of any covered anti-PDGF product we successfully commercialize.

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

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

- Under a license, manufacturing and supply agreement with Nektar Therapeutics, or Nektar, for specified pegylation reagents used to manufacture Fovista, we are obligated to make future payments to Nektar of up to an aggregate of $6.5 million if we achieve specified clinical and regulatory milestones, and an additional payment of $3.0 million if we achieve a specified commercial milestone with respect to Fovista. We are obligated to pay Nektar tiered royalties at low to mid-single-digit percentages of net sales of any licensed product we successfully commercialize, with the royalty percentage determined by our level of licensed product sales, 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.

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 2015 annual meeting of stockholders, as filed with the SEC on April 30, 2015.

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 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.

Table of Contents

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 $426.0 million as of September 30, 2015, consisting of cash, money market funds that invest in U.S. Treasury securities 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 September 30, 2015, substantially all of our total liabilities were denominated in the U.S. dollar.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2015. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

In July 2015, we identified a material weakness in our internal control over financial reporting as of December 31, 2014. As described below, during the three months ended September 30, 2015, we implemented changes to our internal controls related to accounting for income taxes. These new internal controls were operational as of September 30, 2015. However, because the identified material weakness in our internal control over financial reporting will not be considered fully remediated until sufficient time has elapsed to provide evidence that the new controls are operating effectively, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures were not effective at the reasonable assurance level as of September 30, 2015.

Changes in Internal Control over Financial Reporting

As discussed in Item 9A in our Annual Report on Form 10-K/A for the year ended December 31, 2014, filed with the Securities and Exchange Commission on July 28, 2015, in July 2015, our management identified a material weakness in our internal control over financial reporting as of December 31, 2014, which material weakness was unchanged as of March 31, 2015 as reported in our Quarterly Report on Form 10-Q/A for the quarter ended March 31, 2015, filed with the Securities and Exchange Commission on July 28, 2015, and remained unchanged as of June 30, 2015 as reported in our

Quarterly Report on Form 10-Q for the quarter ended June 30, 2015, filed with the Securities and Exchange Commission on August 10, 2015. During the three months ended September 30, 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. The identified material weakness in internal control over financial reporting will not be considered fully remediated until sufficient time has elapsed to provide evidence that the new controls are operating effectively. Other than the remediation steps described above, no change in our internal control over financial reporting (as defined in Rules 13a-15(d) and 15d-15(d) under the Exchange Act) occurred during the three months ended September 30, 2015 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Table of Contents

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

We are not currently subject to any material legal proceedings.

Item 1A. Risk Factors.

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

Risks Related to Our Financial Position and Need for Additional Capital

Our history of operating losses 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 incur losses for at least the next several years and may never achieve or maintain profitability.

Since inception, we have experienced significant cash outflows in funding our operations. As of September 30, 2015, we had an accumulated deficit of $369.9 million. Our net loss was $70.1 million for the nine months ended September 30, 2015, and $116.8 million for the year ended December 31, 2014 and we expect to continue to incur significant operating losses in 2015 and in the future. To date, we have not generated any revenues from product sales and have financed our operations primarily through private placements of our preferred stock, venture debt borrowings, funds received under our royalty purchase and sale agreement with Novo A/S, which we refer to as the Novo Agreement, our initial public offering, which we closed in September 2013, our follow-on public offering, which we closed in February 2014 and funds we received under the Novartis Agreement, which we entered into in May 2014.

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

Our product candidates, Fovista and Zimura, are in clinical development. We expect our expenses to continue to increase, particularly as we continue the development of Fovista in our Phase 3 clinical program. We initiated our pivotal Phase 3 clinical program for Fovista in August 2013. We plan to enroll a total of approximately 1,866 patients for this program. In addition, we also expect our expenses to increase as we further evaluate the potential benefit of Fovista when administered in combination with anti-VEGF therapy 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,

Table of Contents

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 also exploring the potential of an ophthalmic formulation for tivozanib, an anti-VEGF compound 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.

We are party to agreements, specifically an asset acquisition agreement with OSI (Eyetech), Inc., which agreement is now held by OSI Pharmaceuticals, LLC, a subsidiary of Astellas US, LLC, and license agreements with Archemix Corp., or Archemix, and Nektar Therapeutics, or Nektar,

that impose significant milestone payment obligations on us in connection with our achievement of specific clinical, regulatory and commercial milestones with respect to Fovista 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.

Furthermore, we are incurring and expect to continue to incur additional costs associated with operating as a public company, hiring additional personnel and expanding our facilities. These costs include significant legal, compliance, accounting and investor and public relations expenses as well as increased insurance premiums. Moreover, additional rules and regulations applicable to public companies have increased our legal and financial compliance costs and have made, and will continue to make, some activities more time-consuming and costly.

Our expenses also 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, for us 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 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, including personnel to support our product development and planned future commercialization efforts.

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 revenue from product sales 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 ability to commercialize our product candidates, in particular Fovista, will require us to be successful in a range of challenging activities, including:

- obtaining favorable results from our Phase 3 clinical program for Fovista;
- obtaining favorable results, especially with respect to safety, in our other clinical trials involving Fovista, including the Fovista Expansion Studies;
- subject to obtaining favorable results from our Phase 3 clinical program, applying for and obtaining marketing approval for Fovista;

Table of Contents

- establishing sales, marketing and distribution capabilities to effectively market and sell Fovista in the United States with our own specialty sales force targeting retinal specialists;
- successfully maintaining our arrangement with Novartis to commercialize Fovista in markets outside the United States;
- obtaining adequate coverage and reimbursement for our product candidates, if approved, from governmental and third-party payors;
- securing, protecting and enforcing our rights to our intellectual property portfolio related to Fovista;
- ensuring the manufacture of commercial quantities of Fovista; 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 profitability. In addition, our profitability 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. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or continue our operations. A decline in the value of our company would also cause our stockholders to lose all or part of their investment.

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

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

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

As of September 30, 2015, we had cash, cash equivalents, and marketable securities of $426.0 million. We also had $367.6 million in total liabilities, $339.7 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 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 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 or the scale-up of manufacturing activities 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.

Table of Contents

Moreover, our current Phase 3 clinical program for Fovista is expected to continue through at least 2017, 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. Furthermore, we are at the early stages of clinical development for Zimura, which we expect will continue for at least the next several years. At this time, we cannot reasonably estimate the remaining costs necessary to complete the clinical development of either Fovista or Zimura, complete manufacturing validation activities associated with Fovista and 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 therapy, and potentially in other ophthalmic diseases and conditions with unmet need;
- the scope, progress, costs and results of our Zimura clinical programs, including our Phase 2/3 Zimura GA trial, our Phase 2a Zimura wet AMD trial and our Phase 2a Zimura PCV trial, 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 review of Fovista and Zimura;
- the timing, scope and cost of commercialization activities for Fovista or Zimura if we receive, or expect to receive, marketing approval for either product candidate, including the costs and timing of expanding our outsourced manufacturing activities, including activities 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 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.

Table of Contents

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.

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 stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our existing stockholders' rights as holders of our common stock. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Our pledge of assets, including intellectual property rights, as collateral to secure our obligations under the Novo Agreement may limit our ability to obtain debt financing.

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

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

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

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

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

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 therapy 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, wet AMD and PCV. 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 therapy 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

Table of Contents

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.

We do not expect to have initial, top-line data from our Phase 3 clinical program for Fovista until the fourth quarter of 2016, at which time we expect to have initial top-line data from the two Fovista Phase 3 Lucentis Trials only. The timing of the availability of initial, top-line data from the Fovista Phase 3 Eylea/Avastin Trial 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 becomes 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 currently anticipate that we will 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, assuming positive data 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. Additionally, 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 remains a significant risk that we will fail to successfully develop Zimura. We have very limited data from our completed Phase 2a clinical trial evaluating the safety and effectiveness of Zimura for the treatment of dry AMD and our completed Phase 2a clinical trial evaluating the safety and effectiveness of Zimura administered in combination with Lucentis for the treatment of wet AMD. These trials enrolled 47 patients and 60 patients, respectively, and neither trial included a control arm.

Although our current development plans call for us to initiate multiple clinical trials over the next several months, we may not initiate or complete these 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 in our planned trials 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 generate revenues from product sales, which we do not expect will occur before the end of 2017, if ever, is dependent on our obtaining marketing approval for and commercializing our product candidates, and in particular, Fovista and Zimura. The success of these product candidates will depend on several factors, including the following:

- obtaining favorable results from clinical trials;

Table of Contents

- for Fovista, receipt of marketing approvals from applicable regulatory authorities for the use of Fovista in combination with anti-VEGF therapy 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;
- for Zimura, receipt of 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;
- the scope of the label that may be approved by applicable regulatory authorities, including the specific indication for which the product may be approved;
- 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;
- launching commercial sales of the product candidate, if and when approved, whether alone or in collaboration with others, including Novartis for Fovista;
- acceptance of the product candidate, if and when approved, by patients, the medical community and third-party payors;
- for Fovista, continued, widespread use of anti-VEGF therapies in the treatment of wet AMD in combination with which Fovista will be used;
- effectively competing with other therapies, including the existing standard of care, and other forms of drug delivery;
- maintaining a continued acceptable safety profile of the product candidate following approval;

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

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

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

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

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

Our Phase 2b clinical trial evaluated a combination of Fovista and Lucentis. In this trial, patients treated with a combination of 0.3 mg of Fovista and Lucentis did not achieve statistically significant superiority compared to Lucentis monotherapy based on the pre-specified primary endpoint of mean change in visual acuity from baseline at the 24 week time point. Although a combination of 1.5 mg of Fovista and Lucentis demonstrated statistically significant superiority in this trial compared to Lucentis monotherapy based on the pre-specified primary endpoint of mean change in visual acuity from baseline at the 24 week time point, we may nonetheless fail to achieve success in our 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 timepoint is substantially longer than 24 weeks after the first dose of Fovista, which was the timepoint at which

Table of Contents

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 studies 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 timepoint for the primary endpoint, have a greater number of patients (approximately 1,866), have a greater number of sites (more than 225), which encompass a much larger geographical recruitment area, and result in chronic exposure to a higher rate of intraocular pressure due to an increased injection volume. Consequently, there is potential for an increase in cumulative side effects resulting from two separate 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 study and monotherapy anti-VEGF studies which may be reflected in an increase in adverse events and/or serious adverse event rates (either ocular, systemic or both) in patients receiving Fovista combination therapy. For example, there may be, among others, an increase in the rates of intraocular infections, or endophthalmitis, intraocular pressure, glaucoma, retinal tears, cataracts, retinal detachment, intraocular inflammation, retinal and/or choroidal circulation compromise, cardiovascular disease such as myocardial infarctions, stroke, blood clots or emboli, or hospitalizations in the Fovista combination therapy patients.

In general, the FDA and similar regulatory authorities outside the United States require two adequate and well controlled clinical trials demonstrating safety and effectiveness for marketing approval 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 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

Table of Contents

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, and initiated the three trials in our Phase 3 clinical program in the United States without waiting for any such comments. The FDA or other regulatory authorities may request additional information, require us to conduct additional 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 made regulatory submissions in selected countries to initiate Phase 3 clinical trials of Fovista. For the two Fovista Phase 3 Lucentis Trials, we obtained all of the necessary country approvals to proceed with the trials in these selected countries outside the United States. For the Fovista Phase 3 Eylea/Avastin Trial, we have obtained all of the necessary country approvals to proceed with the trial in the selected countries in the European Union and substantially all of the necessary country approvals to proceed with the trial in the selected countries outside of the European Union. 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 Phase 2/3 Zimura GA trial 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 the interim analysis, a determination will be made based on safety and efficacy parameters whether to expand the trial and enroll 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 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 Phase 2/3 Zimura GA trial 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 access to all of the data from the Phase 2/3 trial and based solely on the determination to expand the Phase 2/3 trial 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 Phase 2/3 trial, may

Table of Contents

change based on feedback we may receive from regulatory authorities during development, including during the Phase 2/3 trial, 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 or distribution restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

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

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

Patient enrollment is affected by other factors, including:

- severity of the disease under investigation;
- the ability of current technology to adequately define the disease state;
- eligibility criteria for the study in question;
- perceived risks and benefits of the product candidate under study;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- proximity and availability of clinical trial sites for prospective patients.

The Novartis Agreement provides for an additional milestone payment by Novartis upon our achievement of a defined level of patient enrollment in our ongoing Phase 3 clinical program for Fovista. We will not be entitled to receive the remaining enrollment-based milestone payment unless and until we enroll the specified number of patients. In addition, our inability to locate and enroll a sufficient number of patients for our clinical trials would result in significant delays in our clinical trials, could require us to abandon one or more clinical trials altogether and could delay or prevent our receipt of necessary regulatory approvals. Enrollment delays in our clinical trials also may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

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

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

Table of Contents

- 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.

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

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

Fovista administered in combination with Lucentis was generally well tolerated in our Phase 1 clinical trial and our Phase 2b clinical trials. However, we have clinical data for Fovista administered in combination with Lucentis from only two 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 timepoint for the primary efficacy endpoint, have a greater number of patients (approximately 1,866), have a greater number of sites (more than 225), which encompass a much larger geographical recruitment area, and result in chronic exposure to a higher rate of intraocular pressure due to an increased injection volume. Consequently, there is potential for an increase in cumulative side effects resulting from two separate 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 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 third-some party clinical trials, may be associated with a greater risk of serious adverse events or undesirable side effects as compared to Lucentis.

We have very limited data regarding the safety, tolerability and efficacy of Zimura for the treatment of GA. We have no preclinical or clinical data on the effects of Zimura when administered in combination with an anti-VEGF drug for the treatment of wet AMD, including PCV. Our clinical trials for Zimura may 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.

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

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

- efficacy and potential advantages compared to alternative treatments, including the existing standard of care;

- any restrictions on the use of our products in combination with other medications, such as a Fovista label requiring a waiting period after the intravitreal injection of the anti-VEGF drug and prior to the intravitreal injection of Fovista;

- any restrictions on the use of our products to a subgroup of patients, such as by excluding from the Fovista label patients with pure occult subtype wet AMD;

- restrictions in the label on the use of Fovista with a particular anti-VEGF drug;

- any changes in the dosing regimen of, or the means of administering or delivering, an anti-VEGF drug with which Fovista will be used;

- our and our 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.

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 therapy limits further enhancement of visual outcome with anti-VEGF monotherapy.

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

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

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

Table of Contents

to our approach, and others are based on entirely different approaches. We also will face similar competition with respect to any other products or product candidates that we may seek to develop or commercialize in the future for the treatment of wet AMD, dry AMD or other diseases.

There are also a number of products in preclinical research and clinical development by third parties to treat wet AMD, including product candidates that inhibit the function of PDGF, which is the molecule whose function Fovista inhibits, product candidates that inhibit the function of both VEGF and PDGF that could obviate the separate use of an anti-PDGF agent, such as Fovista, and anti-VEGF and/or anti-PDGF gene therapy products that may substantially reduce the number and frequency of intravitreal injections when treating wet AMD. These companies include pharmaceutical companies, biotechnology companies, and specialty pharmaceutical and generic drug companies of various sizes, such as Regeneron Pharmaceuticals, Inc., which is working in collaboration with Bayer HealthCare and has announced that it initiated a Phase 2 clinical trial of its combination anti- VEGF/anti-PDGF clinical candidate in the second quarter of 2015. Novartis's Alcon division, Quark Pharmaceuticals, Inc., PanOptica, Inc. Allergan, Inc., Ohr Pharmaceutical, Inc., Xcovery Vision LLC, Santen, Neurotech Pharmaceuticals, Inc., Avalanche Biotechnologies, Inc., Somalogic, Inc. and others all have either anti-VEGF or gene therapy treatments in various phases of clinical development. Furthermore, we are aware of at least one company, Tyrogenex Inc., that is developing an orally-administered dual inhibitor of VEGF and PDGF, for which it recently announced the initiation of a Phase 2 trial. Several companies are pursuing the manipulation of stem cells to provide a novel approach to treating retinal diseases, including wet AMD. 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 therapies that are currently in use. If such technologies are successfully developed and approved for use, we may need to conduct additional clinical trials of Fovista using a less frequent dosing schedule than the dosing schedule we are currently using in our ongoing Phase 3 clinical program. Any such trials may not be successful.

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

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.

Table of Contents

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, also referred to as drug substance, for both Fovista and Zimura and a different, single third-party manufacturer to provide fill-finish services for both Fovista and Zimura. Other than our 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 lots of Fovista and Zimura to date. Fovista has been manufactured at commercial scale only on a limited basis, and Zimura has never been manufactured at commercial scale. 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.

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

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

We have no track record as a company 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 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

Table of Contents

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.

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 therapy, including off-label use by intravitreal injection of Avastin provided by us, we also face an inherent risk of product liability exposure related to the testing of such anti-VEGF drugs. If we become subject to or otherwise cannot successfully defend ourselves against claims that our product candidates, anti-VEGF drugs administered in combination with our product candidates or our products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop or in-license;

- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;

Table of Contents

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

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

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations and may not perform their obligations as expected;
- collaborators may deemphasize or not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus, changes in product candidate priorities or available funding or changes in priorities as a result of a merger, acquisition or other corporate restructuring;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- we could grant exclusive rights to our collaborators, which would prevent us from collaborating with others;
- disagreements or disputes with collaborators, including disagreements or disputes over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of products or product candidates, might lead to additional responsibilities for us with respect to

Table of Contents

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.

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

We will depend heavily on our commercialization arrangement with Novartis for 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.

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

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

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product

Table of Contents

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 drug substance for Fovista for clinical and commercial supply.

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

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

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

If our third-party manufacturers for Fovista drug substance, Zimura drug substance or the PEG reagent we use for Zimura fail to fulfill our purchase orders, if Nektar breaches its obligations to us under our supply agreement, or if any of these manufacturers should become unavailable to us for any reason, 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 drug substance 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 drug substance, 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 drug substance for use in any other FDA approved drug. Furthermore, with respect to the potential replacement of Nektar, the replacement manufactuerer’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 drug substance, 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 drug substance made using the Nektar PEG reagent and the Fovista drug substance made using any replacement PEG reagent we propose to use or between the Nektar PEG reagent itself and any replacement PEG reagent we propose to use to make Fovista, or, in the case of a replacement of Agilent, equivalence between the Fovista drug substance made by Agilent and the Fovista drug substance made by the alternative manufacturer. We ultimately may be unable to demonstrate such equivalence.

Table of Contents

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

We also have diligence and development obligations under our acquisition agreement with OSI Pharmaceuticals and our license agreements with Archemix and Nektar. Generally, these diligence obligations require us to use commercially reasonable efforts to develop, seek regulatory approval for and commercialize our products in the United States, the European Union and, in some cases, certain other specified countries. Although the Novartis Agreement provides that Novartis will be responsible for performing certain of these obligations with respect to specified countries 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 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

Table of Contents

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 one of these U.S. patents, 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 would be in 2030.

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

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

The U.S. patent rights covering Zimura as a composition of matter are expected to expire in 2025. Such expiration date may be prior to the date by which we would be able to commercialize Zimura in the United States if we seek and obtain marketing approval. The U.S. patent rights covering methods of treating certain complement protein mediated disorders with Zimura are expected to expire in 2026. As a result, if we obtain marketing approval for Zimura,

we may not be able to exclude competitors from commercializing products similar or identical to ours if such competitors do not use or promote our claimed methods of treatment or do use or promote our methods of treatment after our patents expire. Depending on potential delays in the regulatory review process for Zimura, we may be able to obtain a patent term extension for one of these patents in the United States, but we can provide no assurances that such an extension will be obtained.

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

The issuance of a patent is not conclusive as to its inventorship, ownership, scope, term, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. For example, if we receive marketing approval for our product candidates, other pharmaceutical companies may seek approval of generic versions of our products with the FDA or regulatory authorities in other jurisdictions. We may then be required to initiate proceedings against such companies in an attempt to prevent them from launching such generic versions. The risk of being involved in such proceedings is likely to increase if our products are commercially successful. In any such proceedings, the inventorship, ownership, scope, term,

Table of Contents

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, 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 and as we gain the greater visibility associated with being a public company. Third parties may assert infringement claims against us or our collaboration or commercialization partners based on existing or future intellectual property rights. We or they may not be aware of all such intellectual property rights potentially relating to our product candidates and their manufacture and uses. Thus, we do not know with certainty that Fovista, Zimura or any other product candidate, or our intended commercialization thereof, does not and will not infringe or otherwise violate any third party's intellectual property.

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

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

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

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 Other Legal Compliance Matters

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

Our product candidates, including Fovista and Zimura, and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to

comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries.

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

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

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

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

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

In the United States, our lead product candidate, Fovista, received fast track designation and may be eligible for priority review status. If a drug is intended for the treatment of a serious or life-threatening disease or condition and the drug demonstrates the potential to address unmet medical needs for this disease or condition, the drug sponsor may apply for FDA fast track designation. If a drug offers major advances in treatment, the drug sponsor may apply for FDA priority review status. The FDA has broad discretion whether or not to grant fast track designation or priority review status, so even if we believe a particular product candidate is eligible for such designation or status the FDA could decide not to grant it. Even though Fovista has received fast track designation for the treatment of wet AMD and may be eligible for priority review status, we may not experience a faster development process, review or

approval compared to conventional FDA procedures. The FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program.

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

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

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

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

In order to market and sell Fovista, Zimura and any other product candidate that we may develop in the European Union and many other jurisdictions, we or our third-party commercialization partners, including Novartis, 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.

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

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

The FDA may also impose requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the product, including the adoption and implementation of risk evaluation and mitigation strategies. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling and regulatory requirements. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we do not restrict the marketing of our products only to their approved indications, we

may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws.

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

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions and warnings in the labeling and marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing clinical trials;
- warning or untitled letters;
- field alerts;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recalls of products;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

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

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

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of any product candidates, including Fovista, for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us and our commercialization partners to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial

arrangements and relationships through which we and our commercialization partners market, sell and distribute any products for which we or they obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

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

- the federal False Claims Act imposes criminal and civil penalties, including civil whistleblower or *qui tam* actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for

Table of Contents

payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

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

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

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

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

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

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

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

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

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of Fovista, Zimura or any other product candidate that we may develop, restrict or regulate post-approval activities and affect our and 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.

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

Table of Contents

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

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

- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements to report financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

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

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

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

The pricing of prescription pharmaceuticals is also subject to governmental control outside of the United States. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we or 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.

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

We and our third-party manufacturers are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and produce hazardous waste products. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for

Table of Contents

any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

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

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

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

Risks Related to Employee Matters and Managing Growth and Our Operations

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

We are highly dependent on David R. Guyer, M.D., our Chief Executive Officer, Samir Patel, M.D., our President, and Michael G. Atieh, our Chief Financial and Business Officer, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into 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 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 and manufacturing development. During the 12-month period ending September 30, 2015, we hired approximately half of our over 100 employees. We also expect to continue to hire additional employees and expand the scope of our operations in the area of clinical development and, as we approach potential marketing approval for any of our product candidates, in the area of sales, marketing and distribution. To manage our growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the inherent challenges associated with managing such rapid growth, we may not be able to manage effectively the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

We have identified a material weakness in our internal control over financial reporting.

As discussed in a Current Report on Form 8-K filed with the Securities and Exchange Commission on July 28, 2015, we determined in July 2015 that we overstated the net deferred tax asset recorded on our balance sheets and understated the income tax provision on our statements of operations as of and for the periods ending June 30, 2014, September 30, 2014, December 31, 2014 and

March 31, 2015. As discussed in Item 9A, "Controls and Procedures," of our Annual Report on Form 10-K/A, also filed on July 28, 2015, management believes that this situation revealed a material weakness in internal controls related to technical accounting expertise over the accounting for deferred tax assets and income tax accounting in general. A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the company's annual or interim financial statements will not be prevented or detected on a timely basis. The 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.

We have discussed the identified control deficiency in our financial reporting and the remediation of such deficiency with the audit committee of our board of directors and will continue to do so as necessary. During the three months ended September 30, 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. The identified material weakness in internal control over financial reporting will not be considered fully remediated until sufficient time has elapsed to provide evidence that the new controls are operating effectively. Moreover, while we have taken steps to remediate this deficiency in controls, 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 maintain sensitive data on our 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. The secure maintenance of this information is critical to our business and reputation. Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. 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. In the recent past, cyber-attacks have become more prevalent and much harder to detect and defend against. Our 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.

Risks Related to Our Common Stock

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

As of September 30, 2015, 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.

Table of Contents

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 September 30, 2015, we had outstanding 34,903,881 shares of common stock. Of these shares, approximately 6,301,000 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 5,560,000 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 September 30, 2015, we had outstanding stock options to purchase an aggregate of approximately 3,224,000 shares of our common stock, of which options to purchase approximately 995,000 shares were vested, as well as approximately 285,000 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
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our certificate of incorporation or by-laws.

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

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

Our stock price may be volatile. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular

Table of Contents

companies. As a result of this volatility, our stockholders may not be able to sell their shares of common stock at or above the price at which they purchased their shares. The market price for our common stock may be influenced by many factors, including:

- the success of 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 now required to devote substantial time to new compliance initiatives and corporate governance practices.

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

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to furnish with our periodic Exchange Act reports a report by our management on our internal control over financial reporting. We are also required to include with our annual report an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404, we 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

Table of Contents

and improvement process for internal control over financial reporting. These efforts will need to increase following management’s conclusion that our accounting for net deferred tax assets in 2014 and early 2015 revealed a material weakness in internal control over financial reporting related to technical accounting expertise over the accounting for deferred tax assets and income tax accounting in general. Despite our ongoing efforts, 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.

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

Recent Sales of Unregistered Securities

On August 31, 2015, we issued to an employee an option to purchase 120,000 shares of our common stock at an exercise price of $44.03 per share. This option was an inducement grant issued outside our existing equity compensation plan in accordance with NASDAQ listing rule 5635(c)(4). We intend to file a registration statement on a Form S-8 to register the shares of common stock underlying this option prior to the time at which this option becomes exercisable.

Purchase of Equity Securities

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

Use of Proceeds from Registered Securities

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

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

As of September 30, 2015, we have used approximately $39.3 million of the net proceeds from initial public offering as follows:

- approximately $27.6 million to fund certain costs of our Phase 3 clinical program for Fovista administered in combination with anti-VEGF therapy for the treatment of wet AMD, which costs consists of external research and development expenses and clinical development related employee expenses; and

- approximately $11.7 million for working capital and other general corporate purposes.

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

Item 5. Other Information.

None.

Table of Contents

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: November 5, 2015

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

EXHIBIT INDEX

Exhibit Number	Description of Exhibit
10.1*	Amendment No. 1 to Clinical Manufacturing and Supply Agreement by and between the Registrant and Agilent Technologies, Inc. dated September 3, 2015
10.2*	Commercial Manufacturing and Supply Agreement by and between the Registrant and Agilent Technologies, Inc. dated September 3, 2015
10.3	Letter Agreement with Todd N. Smith executed July 20, 2015
31.1	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS**	XBRL Instance Document
101.SCH**	XBRL Taxonomy Extension Schema Document
101.CAL**	XBRL Taxonomy Extension Calculation Linkbase Document
101.LAB**	XBRL Taxonomy Extension Label Linkbase Database
101.PRE**	XBRL Taxonomy Extension Presentation Linkbase Document
101.DEF**	XBRL Taxonomy Extension Definition Linkbase Document

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

** Submitted electronically herewith.

Attached as Exhibit 101 to this report are the following formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheet at September 30, 2015 and December 31, 2014 (unaudited), (ii) Statement of Operations (unaudited) for the three and nine month periods ended September 30, 2015 and 2014, (iii) Statement of Cash Flows (unaudited) for the nine month period ended September 30, 2015 and 2014 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.

Exhibit 10.1

Confidential Materials omitted and filed separately with the
Securities and Exchange Commission. Double asterisks denote omissions.

AMENDMENT NO. 1

TO THE CLINICAL MANUFACTURING AND SUPPLY AGREEMENT

BY AND BETWEEN

AGILENT TECHNOLOGIES, INC. AND OPHTHOTECH CORPORATION

This Amendment No. 1 (this "Amendment") amends the Clinical Manufacturing and Supply Agreement (the "Agreement") by and between Agilent Technologies, Inc. ("Agilent") and Ophthotech Corporation ("Customer") dated May 2, 2014 ("Effective Date").

WHEREAS, Agilent and Customer entered into the Agreement as of the Effective Date to specify the terms and conditions under which Agilent will manufacture and supply to Customer the E10030 molecule described in Exhibit A of the Agreement, and perform Manufacturing Services for Customer with respect thereto, solely for clinical purposes and not for commercial purposes; and

WHEREAS, Agilent and Customer desire to amend the Agreement to modify certain terms relating to the license of Agilent and Third Party Know-How to Customer and certain other terms as set forth in this Amendment.

NOW, THEREFORE, in consideration for the mutual promises and covenants contained herein, the parties agree as follows:

1. Terms. Capitalized terms in this Amendment shall have the same meaning as those in the Agreement, unless specifically defined in this Amendment. All section and paragraph references refer to sections or paragraphs, as applicable, in the Agreement. References to the term "Agreement" in the Agreement shall be deemed to give effect to this Amendment.

2. Amendments. The parties agree to amend the Agreement as set forth below.

a. Sections 9.3.2.3, 9.3.2.4 and 9.3.2.5 are hereby deleted in their entirety.

b. A new Section 9.3.2.3 is added as follows:

"With respect to the licenses set forth in Sections 9.3.2.1 and 9.3.2.2, the Parties hereby agree that the licenses for the Product and Finished Product under this Agreement are hereby extended to the ARC1905 molecule (Zimura)."

c. Section 9.4, Reservation of Rights, is hereby deleted and replaced with the following:

"9.4 Reservation of Rights. Except as expressly provided herein, no license to any Agilent Intellectual Property or Customer Intellectual Property is granted, conveyed or implied. All rights not conferred are expressly reserved."

d. Section 9.6.3, Third Party Know-How, is hereby deleted and replaced with the following:

"9.6.3 Third Party Know-How. Agilent has not and shall not incorporate into the Process any Third Party Know-How unless (A) Agilent has the right to incorporate such

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Third Party Know-How into the Process and (B) the Parties have agreed to incorporate such Third Party Know-How into the Process pursuant to the Change Management process".

e. At the beginning of the first sentence of Section 13.2(a), the words "This Agreement or a Statement of Work" are replaced with the following: "Subject to Section 13.2(c), this Agreement or a Statement of Work".

f. Section 13.2(c) is hereby deleted and replaced with the following:

"(c) In the event of either Party's material breach of its confidentiality obligations under Article 14, the Parties shall refer the matter for resolution under the escalated dispute resolution process set forth in Section 16(b). For the avoidance of doubt, [**]"

g. Section 14.1.6 is hereby amended by deleting all references to Section 9.3.2.3 from the fifth sentence. In addition, the final sentence of Section 14.1.6 is hereby deleted in its entirety.

h. For the sake of clarity, the Parties agree that the weights in Exhibit J, Product Pricing, refer to oligonucleotide weight.

3. Entire Agreement. This Amendment constitutes the entire agreement between the parties with respect to the subject matter therein and incorporates all prior agreements and amendments by reference. Except as expressly modified herein, the Agreement shall remain in full force and effect in accordance with its terms. To the extent there are any inconsistencies or ambiguities between this Amendment and the Agreement, the terms of this Amendment shall supersede the Agreement.

In witness whereof, the parties have executed this Amendment effective as of September 3, 2015.

AGREED:

AGILENT TECHNOLOGIES, INC.	OPHTHOTECH CORPORATION
By: /s/ Nelson Thune	By: /s/ Barbara A. Wood
Name: Nelson Thune	Name: Barbara A. Wood
Title: General Manager	Title: SVP & General Counsel
Date: 03 September 2015	Date: September 3, 2015

Confidential Materials omitted and filed separately with the
Securities and Exchange Commission. Double asterisks denote omissions.

COMMERCIAL MANUFACTURING AND SUPPLY AGREEMENT

This Commercial Manufacturing and Supply Agreement (this "Agreement") is entered into by and between AGILENT TECHNOLOGIES, INC., a Delaware corporation, having a principal office at 5301 Stevens Creek Blvd., Santa Clara, CA 95051 ("Agilent") and OPHTHOTECH CORPORATION, a Delaware corporation, having a principal office at One Penn Plaza, Suite 1924, New York, NY 10119 ("Customer") effective as of September 3, 2015 (the "Effective Date"). Agilent and Customer are each referred to herein as a "Party" and together as the "Parties".

In consideration of the mutual covenants and promises set forth herein, the Parties hereby agree as follows:

1. **SCOPE OF AGREEMENT**

This Agreement, together with the Quality Agreement (as defined below) specifies the terms and conditions under which Agilent will manufacture and supply the Product (as defined below) to Customer and perform Manufacturing Services (as defined below) for Customer for commercial purposes.

2. **DEFINITIONS**

The following capitalized terms will have the meanings given for the purposes of this Agreement:

2.1 "Affiliate" means any business entity which directly or indirectly controls, is controlled by, or is under common control with any Party to this Agreement. A business entity shall be deemed to "control" another business entity if (i) it owns, directly or indirectly, at least fifty percent (50%) of the issued and outstanding voting securities, capital stock, or other comparable equity or ownership interest of such business entity, or (ii) it has the de facto ability to control or direct the management of such business entity. If the laws of the jurisdiction in which such entity operates prohibit ownership by a Party of fifty percent (50%) or more, "control" shall be deemed to exist at the maximum level of ownership allowed by such jurisdiction; provided, however, that there is a de facto ability to direct or control its management.

2.2 "Anti-PDGF Aptamer" means (i) an Aptamer that binds to a platelet-derived growth factor (PDGF) and (ii) intermediates thereof.

2.3 "Active Pharmaceutical Ingredient (API)" has the meaning set forth in the Quality Agreement.

2.4 "Aptamer" means (i) any pegylated or unpegylated naturally or non-naturally occurring oligonucleotide that binds to a Target and (ii) any pegylated or unpegylated oligonucleotide Derived from an oligonucleotide of clause (i) that binds to a Target.

2.5 "Batch" means a specific quantity of Product with a specified yield mutually agreed upon between Customer and Agilent as set forth in a Purchase Order that (a) is intended to have uniform character and quality within specified limits, (b) is Processed according to a single manufacturing order during the same cycle of manufacture, and (c) has a minimum yield of at least [**] of Product.

2.6 "Batch Packet" has the meaning set forth in the Quality Agreement.

2.7 "Certificate of Analysis" has the meaning set forth in the Quality Agreement.

2.8 "Certificate of Compliance" has the meaning set forth in the Quality Agreement.

2.9 "Change Management" means the procedure set forth in the Quality Agreement.

2.10 "Confidentiality Agreement" has the meaning set forth in Section 14.1.

2.11 "Derived" means identified, obtained, developed, created, synthesized, designed or resulting from, based upon, containing or incorporating or generated from or conjugated to or complexed with (whether directly or indirectly or in whole or in part).

2.12 "Facility" means Agilent's manufacturing facility located at 5555 Airport Boulevard, Boulder, Colorado 80301, or such other manufacturing site agreed to by the Parties in writing.

2.13 "FDA" means the United States Food and Drug Administration or any successor organization.

2.14 "Finished Product" means Customer's biological or pharmaceutical drug product that includes the Product.

2.15 "GMP" has the meaning set forth in the Quality Agreement.

2.16 "Good Condition" means that at the time of delivery to Customer's carrier the Product supplied shall: (i) be the right Product; (ii) be in the right quantity in accordance with the manifest; (iii) be in the packaging agreed to by the Parties; (iv) be labeled in accordance with the Product registration; and (v) have no visible defect in the packaging or seal.

2.17 "Independent Laboratory" means a laboratory independent of each Party, mutually agreed in writing between the Parties and competent to determine the matters referred to in Section 8.2.3.

2.18 "E10030" means the Product.

2.19 "Initial Term" has the meaning set forth in Section 13.1.

2.20 "Intellectual Property" means, collectively, Patents, Marks, copyrights, Know-How, and any other intellectual property owned or licensed by a Party.

2.21 "Kilo" means a kilogram of oligonucleotide weight.

2.22 "Know-How" means all non-patented and proprietary: information, inventions, developments, techniques, materials, processes, manufactures, compositions of matter or methods of use and trade secrets, whether or not patentable or copyrightable. Know-How excludes (i) Patents and (ii) any of the foregoing which would be excluded from the definition of Proprietary Information under Section 5 of the Confidentiality Agreement.

2.23 "Latent Defect" means (a) a failure of the Product to meet the Specification, or (b) Agilent's failure to manufacture the Product in accordance with GMP, which failure or defect is (x) present at the time of delivery to Customer's carrier; (y) non-obvious and not reasonably susceptible to discovery upon receipt of the Product from Customer's carrier and (z) subsequently detected by Customer. Latent Defect excludes a failure or defect that (i) is attributable to a defect in the PEG delivered to Agilent by Customer for use in the Product, provided that such defect in the PEG was not discoverable by Agilent in the course of testing in accordance with Agilent's Standard Operating Procedure and provided that such defect in the PEG is not otherwise caused by Agilent's negligence; (ii) is attributable to a fundamental chemical or stability defect in the Product and results in a change in the Product after delivery by Agilent; or (iii) is the result of further processing, storage, handling or use of the Product after delivery by Agilent.

2.24 "Licensed Patent(s)" means any Patent owned by Agilent or its Affiliates as of the Effective Date or during the Term claiming or covering the Process.

2.25 "Manufacturing Services" means services, other than testing and other services that are performed as part of the manufacture and supply of Product, set forth in a Statement of Work to be performed by Agilent with respect to Product and Finished Product, including stability testing.

2.26 "Manufacturing Standards" has the meaning set forth in Section 5.2.

2.27 "Marks" means the trademarks, service marks, trade dress, trade names, logos, insignia, symbols, designs or other marks identifying either Party or its products.

2.28 "Master Batch Record" has the meaning set forth in the Quality Agreement.

2.29 "Patents" means patents, patent applications and any issued divisions, continuations, continuations-in-part, re-issues, re-examinations, renewals or extensions thereof and any foreign counterpart of any of such U.S. patents.

2.30 "Person" means any individual, partnership, corporation, limited liability company, unincorporated organization or association, any trust or any other business entity.

2.31 "Process" or "Processing" means the combination of materials, procedures, test methods and controls used by Agilent to manufacture the Product under this Agreement, that includes the following unit operations: [**].

2.32 "Product" means the Aptamer described in Exhibit A and intermediates thereof.

2.33 "Proprietary Information" has the meaning set forth in Section 14.1.1.

2.34 "Purchase Order" means a written purchase order, in substantially the form agreed in good faith based on customary arrangements in the biotechnology industry, between Agilent and Customer, to be delivered by Customer to Agilent for the manufacture and supply of Product (including testing and other services performed as part of the manufacture and supply of Product) pursuant to this Agreement.

2.35 "Quality Agreement" means the agreement by and between Agilent and Customer, executed by duly authorized representatives of each Party, setting forth the obligations of the Parties with respect to quality matters applicable to the manufacturing and supply of the Product and Customer's drug product testing under this Agreement, attached hereto as Exhibit C.

2.36 "Regulatory Authority" has the meaning set forth in the Quality Agreement.

2.37 "Renewal Term" has the meaning set forth in Section 13.1.

2.38 "Specification" means the specification for the Product as set forth in Exhibit M, which specification may be amended from time to time in accordance with this Agreement.

2.39 "Statement of Work" means any statement of work as mutually agreed to by the Parties for the performance of Manufacturing Services under this Agreement.

2.40 "Supply Deficiency" has the meaning set forth in Section 5.8.1.

2.41 "Supply Failure" has the meaning set forth in Section 5.8.1.

2.42 "Target" means a protein, cytokine, enzyme, receptor, transducer, transcription factor, antigen or any other non-nucleic acid molecule.

2.43 "Term" has the meaning set forth in Section 13.1.

2.44 "Third Party" means any Person who is not a Party or an Affiliate of a Party.

3. OBLIGATIONS OF THE PARTIES; STATEMENTS OF WORK

3.1 Obligations of Agilent. Agilent will manufacture and supply the Product to Customer and perform the Manufacturing Services at the Facility in accordance with the terms of this Agreement, the Quality Agreement, any applicable Purchase Order or Statement of Work and in accordance with GMP and all laws and regulations applicable to the manufacture and supply of the Product at the Facility and the performance of the Manufacturing Services. Agilent shall perform any testing and other services performed as part of the manufacture and supply of

Product as well as the Manufacturing Services in a professional and workmanlike manner consistent with industry standards. Agilent will deliver the Product in accordance with the delivery dates set forth in each accepted Purchase Order and will perform the Manufacturing Services in accordance with the delivery dates set forth in each applicable Statement of Work.

3.2 Obligations of Customer. Customer will provide Agilent with polyethylene glycol ("PEG") in accordance with Section 3.3.1 and information and cooperation reasonably necessary for the manufacture and supply of the Product in accordance with this Agreement and the Quality Agreement.

3.3 PEG.

3.3.1 During the Term, Customer is responsible for the supply of PEG in sufficient amounts to enable Agilent to manufacture Product ordered by Customer hereunder. Customer shall use commercially reasonable efforts to maintain the License, Manufacturing and Supply Agreement between Customer and Nektar Therapeutics, Corporation ("Nektar"), dated September 30, 2006, as amended, (the "PEG Supply Agreement"), or enter into an agreement with Nektar or another Third Party to obtain supply of PEG for the E10030 molecule. For purposes of clarity, Customer may terminate the PEG Supply Agreement with Nektar; provided that Customer has obtained an alternative source of supply of PEG. Notwithstanding any other provision herein, Agilent shall not be liable for any delays or supply failures associated with (i) Customer's failure to supply PEG in sufficient amounts to enable Agilent to manufacture Product ordered by Customer or (ii) termination of Customer's PEG Supply Agreement with Nektar and retention of an alternative source of supply of PEG. Customer shall reimburse Agilent for any reasonable direct costs incurred by Agilent to qualify any alternative source of supply of PEG. Customer shall immediately notify Agilent in writing if Customer reasonably anticipates any delay or shortfall in the supply of PEG in sufficient amounts to enable Agilent to manufacture Product ordered by Customer hereunder. In the event of cancellation or deferment of any Purchase Order due to Customer's failure to supply sufficient amounts of PEG to enable Agilent to manufacture Product ordered by Customer hereunder, the cancellation and deferment fees set forth in Section 4.7 shall apply.

3.3.2 PEG delivered shall be held by Agilent on behalf of Customer on the terms and conditions contained in this Agreement and in accordance with any mutually agreeable instructions provided by Customer (it being understood that Agilent shall be deemed to have agreed to any written instruction provided to Agilent regarding the handling of PEG if it has not objected to such instruction upon delivery of the PEG to Agilent). Customer shall provide any such written instructions to Agilent at least [**] days prior to delivery of the PEG. Upon receipt of the PEG, Agilent will promptly do a visual inspection of the PEG container to ensure it has not been compromised. Agilent will, promptly, but in no event later than [**] business days of receiving the PEG, notify Customer in writing in accordance with the Quality Agreement in the event that such visual inspection revealed that the PEG was compromised at the time of delivery or was, at the time of delivery, otherwise unusable to manufacture Product. Agilent will conduct raw material release testing of the PEG in accordance with the specification for the PEG consistent with the Quality Agreement. Agilent will provide Customer notice in writing in accordance with the Quality Agreement in the event that the PEG does not comply with the specification for the PEG. In the event that Agilent provides a notification in accordance with this Section 3.3.2, Agilent shall not be liable for any delays or supply failures associated with delivered PEG that, at the time of delivery, was compromised or, at the time of delivery, was otherwise unusable to manufacture Product ordered by Customer hereunder. Agilent acknowledges that all PEG delivered shall remain the property of Customer and Agilent

shall clearly identify all such PEG in storage and in its books as goods belonging to Customer. Agilent shall not use any such PEG for any purpose other than for Customer under this Agreement. Agilent shall use first in — first out and first expiry — first out methods of usage.

3.3.3 Subsequent to the completion of the validation Batches for the New Drug Application submission for the Finished Product, Agilent shall reimburse Customer for [**] percent ([**]%) of the invoiced cost of any PEG (which in no event shall be greater than [**] dollars ($[**]) per kilogram) and any associated freight fees in the event that such PEG cannot be used in the Processing of the Product as a result [**]. Agilent shall immediately inform Customer of any loss or damage to the PEG and promptly provide in writing all explanations and evidence relating thereto.

3.3.4 Subsequent to the completion of the validation Batches for the New Drug Application submission for the Finished Product, Agilent shall reimburse Customer for [**] percent ([**]%) of the Cost of the PEG used in the Processing of Product that (i) fails to conform to the Specification, (ii) is not manufactured in accordance with GMP, (iii) is not in Good Condition or (iv) is not free from Latent Defects. For the purposes of calculating the cost of the PEG under this Section 3.3.4, the Parties agree that the cost of the PEG is [**] dollars ($[**]) per Batch in the [**] Process (the "Cost"). Agilent's reimbursement obligation under this

Section 3.3.4 shall be equitably adjusted with respect to any Batch where a portion of the Product produced in such Batch (i) conforms to the Specification, (ii) has been manufactured in accordance with GMP, (iii) is in Good Condition and (iv) is free from Latent Defects.

3.3.5 Agilent shall maintain up-to-date records of all PEG held in inventory and, within the [**] or at such other frequency as the Parties may agree, shall provide to Customer a complete and accurate list of all PEG held by it on the [**]. Such inventory list shall in particular specify the inventory balance of PEG at the relevant date. Agilent shall also provide Customer with written reports on a [**] basis reconciling the quantities of the PEG provided to and held by Agilent, the consumption of the PEG and the estimated yield losses in the Processing.

3.3.6 In addition to the reports set forth in Section 3.3.5, Agilent shall provide to Customer the result of an inventory count to be carried out, under the joint supervision of Agilent and Customer, in accordance with Agilent's usual year end audit procedures, of all the PEG held by Agilent as of [**] (or such other date as the Parties may agree) of each calendar year, such report to be delivered on or before [**] after such date, or within such other time as the Parties may agree. Agilent shall be responsible for all discrepancies (such as, for example, missing quantities) not accounted for during such yearly inventory count or in connection with the monthly inventory report without regard to the reason for the discrepancy. Each Party shall pay for its own fees under this Section.

3.4 Statements of Work.

3.4.1 During the Term, Customer may request that Agilent perform Manufacturing Services. As mutually agreed by the Parties in a Statement of Work, each Party shall perform the obligations set forth in each Statement of Work. In the event of any inconsistency between this Agreement and a Statement of Work, the terms and conditions of this Agreement shall prevail. Each Party shall retain appropriately qualified and trained personnel with the requisite knowledge and experience to perform such obligations in accordance with this Agreement and any applicable Statement of Work.

3.4.2 Customer may make changes to a Statement of Work (other than cancellation or deferment) at any time by a written request. Agilent shall notify Customer within [**] business days of its receipt of such request of any proposed modifications to the price or delivery dates incurred as a direct result of the change. Upon Customer's written acceptance of such proposal, the Statement of Work shall be deemed so modified.

3.5 Exclusivity.

3.5.1 Except as otherwise provided in Section 13.3.1, Agilent agrees that during the Term and for a two (2) year period after the Term (the "Exclusivity Period"), Agilent and its Affiliates shall only supply Anti-PDGF Aptamer APIs or Finished Product to Customer and any Affiliate of Customer or Third Party designated by Customer.

3.5.2 Customer agrees that during the Term, Agilent shall be Customer's supplier of at least [**] percent ([**]%) of Customer's commercial manufacturing requirements of Product on an annual basis for use in the United States, European Union, and any additional future jurisdictions as mutually agreed to by the Parties in writing or such lesser amount in the event Agilent is unable to supply such [**]% percent. If Customer does not meet such purchase requirement for any such calendar year during the Term, Customer shall have the right to cure such deficiency by ordering the missing quantity of Product during the [**] month period following the end of such calendar year. Provided that Customer has cured such deficiency by ordering the missing quantity of Product during the [**] month period following the end of such calendar year, such deficiency shall not be deemed a breach of this Agreement and Agilent shall not have the right to terminate this Agreement as a result of such deficiency. Upon [**] days prior written notice to Customer, Agilent may, at its own expense, appoint an independent auditor to audit and examine Customer's books and records solely for the purpose of confirming Customer's compliance with the exclusivity commitment in this Section 3.5.2. Such audit may be made no more than [**].

4. SUPPLY

4.1 [RESERVED]

4.2 Forecasts. Within [**] days of the commencement of the [**] following the Effective Date and every [**] months thereafter, Customer shall submit to Agilent a written rolling forecast of the quantity of Product which Customer expects to order from Agilent for delivery during the next [**] months (the "Forecast"). The Forecast shall constitute a non-binding, good faith estimate provided by Customer solely to assist Agilent in production planning, and shall not represent any purchase commitment by Customer or a supply commitment by Agilent. It is understood and agreed by the Parties that Agilent will not hold inventory of Product. However, Agilent shall deliver such quantities of Product that are ordered in accordance with a Purchase Order that has been accepted by Agilent.

4.3 Purchase Orders.

4.3.1 Issuance of Purchase Orders and Lead Times. Within [**] days of the commencement of the [**] following the Effective Date, and within [**] days of the commencement of each [**] thereafter, Customer may submit to Agilent a Purchase Order for Product to be delivered within the lead time set forth herein. Each Purchase Order shall be based upon the then-current Forecast and represent the quantity of Batches to be manufactured in a single campaign. Customer may decrease or increase the quantity of Product ordered using validated Batch size increments, provided that any such increase falls within the [**] campaigns per year allotted to Customer. Each Purchase Order shall set forth the

requested delivery date(s) for the Product ordered. The lead time for all Purchase Orders shall be [**] months from the date of Purchase Order acceptance to the requested delivery date. Customer may, at its discretion, have a longer lead time in a Purchase Order. For each accepted Purchase Order, Agilent will employ campaigning for Facility efficiency.

4.3.2 Acceptance of Purchase Orders. Agilent shall notify Customer as to whether any Purchase Order delivered pursuant to Section 4.3.1 has been accepted or rejected within [**] business days following Agilent's receipt of such Purchase Order, unless such Purchase Order exceeds Customer's then current credit limit, in which case the [**] business day period shall be extended to [**] business days. Customer's credit limit shall be reviewed by Agilent consistent with Agilent's corporate policy, and adjusted as necessary, [**] or upon any material adverse change specific to Customer that would affect Customer's ability to perform its obligations hereunder. Agilent may only reject a Purchase Order as follows: (i) the Purchase Order is not in compliance with this Agreement; (ii) the Purchase Order does not have a delivery address; (iii) the Purchase Order does not comply with Agilent's credit limit standards; provided that if Customer agrees to make an up-front pre-payment representing all or a portion of the amount due with respect to such Purchase Order as may be requested by Agilent, Agilent may not reject the Purchase Order on the basis that it does not comply with Agilent's credit limit standards, which pre-payment shall be refunded to Customer by Agilent in the event Agilent fails to deliver Product for which the pre-payment was made; (iv) the Purchase Order does not comply with the lead times set forth in Section 4.3.1; (v) during the period beginning on the date on which Customer files a New Drug Application for the Finished Product with the FDA and ending [**] months from the date of Customer's first commercial sale of the approved Finished Product, the Purchase Order specifies a quantity of Product that is greater than [**] percent ([**]%) of the then-current Forecast rounded up to the nearest validated Batch size increment; (vi) at any time during the Term of this Agreement other than during the period described in (v) above, the Purchase Order specifies a quantity of Product that is greater than [**] percent ([**]%) of the then-current Forecast rounded up to the nearest validated Batch size increment; (vii) Customer has previously submitted, and Agilent has accepted, a Purchase Order within the applicable [**] pursuant to Section 4.3.1; or (viii) the Purchase Order specifies delivery dates that would require more than a single campaign every [**] months. Notwithstanding the foregoing, Agilent will use commercially reasonable efforts to accept more than [**] within each [**] and to accept Purchase Orders in excess of the quantities set forth in (v) and (vi) above. Agilent's failure to affirmatively reject a Purchase Order within the [**] business day period or [**] business day period, as applicable, shall be deemed an acceptance of such Purchase Order. In the event that Agilent rejects a Purchase Order hereunder, Agilent shall notify Customer in writing within the [**] business day period or [**] business day period, as applicable, of the reasons why such Purchase Order was rejected by Agilent. Customer may, at its option and at any time after such rejection, submit a revised Purchase Order.

4.3.3 Details for Purchase Orders. Each Purchase Order shall specify the quantity of Product ordered and the time, manner and address of delivery, all of which shall be subject to this Article 4.

4.3.4 Fulfillment of Purchase Orders. Upon acceptance of a Purchase Order by Agilent, Agilent will deliver the Product (including without limitation delivery of any Batch) by the delivery dates set forth in the applicable Purchase Order. The Parties acknowledge

and agree that Agilent shall have the right to deliver Product up to [**] business days prior to the applicable delivery date set forth in the applicable Purchase Order.

4.4 Delivery and Acceptance. Subject to Section 8.2.2, Agilent will deliver the Product to the carrier selected by Customer. Shipment terms are FCA Agilent's Dock Boulder (Incoterms 2010). Title and risk of loss will pass to Customer when the Product is delivered to Customer's carrier. Customer is responsible for payment of all shipment costs, including any insurance necessary to guard against loss or damage during shipment. Acceptance shall occur upon delivery of the Product to Customer's carrier. Agilent shall not be entitled to deliver partial shipments of Product unless expressly authorized by Customer in writing.

4.5 Certificates. An appropriate Certificate of Analysis (which shall include a material safety data sheet) and Certificate of Compliance shall be provided with the shipment of each Batch delivered to Customer.

4.6 Shipping Instructions. Customer will provide Agilent with packaging and shipping instructions including temperature requirements, temperature monitoring instructions and packaging specifications. Notwithstanding any other provision of this Agreement, Agilent will not be liable for any loss or damage caused by Agilent's compliance with Customer's packaging and shipping instructions or any loss or damage caused by Customer's carrier.

4.7 Cancellation and Deferment of Purchase Orders.

4.7.1 Cancellation. Customer may cancel any Purchase Order or part thereof by providing Agilent with written notice thereof prior to the scheduled delivery date. In the event that Customer cancels a Purchase Order or a part thereof, Customer shall pay the cancellation fees set forth below. In addition, Customer shall reimburse Agilent for all material and labor costs incurred prior to the effective date of cancellation. All cancellation fees and reimbursements of incurred material and labor costs are due within [**] days from the date of Customer's receipt of an invoice. In the event that Agilent is able to use any material for another Purchase Order or customer within [**] months of the effective date of cancellation, Agilent will issue a credit to Customer for such reimbursed material cost. Partial cancellations shall be made using validated Batch size increments.

Cancellation fees are as follows:

[**] percent ([**]%) of the total price of the cancelled Purchase Order or part thereof if Customer provides Agilent with not less than [**] months written notice of the cancellation prior to the scheduled delivery date;

[**] percent ([**]%) of the total price of the cancelled Purchase Order or part thereof if Customer provides Agilent with written notice of the cancellation within [**] months of the scheduled delivery date but not less than [**] days prior to the scheduled delivery date; and

[**] percent ([**]%) of the total price of the cancelled Purchase Order or part thereof if Customer provides Agilent with less than [**] days prior written notice of the cancellation.

4.7.2 Deferment. Customer may defer delivery of any Purchase Order or part thereof by providing Agilent with written notice thereof at least [**] months prior to the scheduled delivery date. Delivery dates for deferred Purchase Orders or parts thereof will be as mutually agreed to by the Parties, subject to the limitation of [**] campaigns per year. In the event that Customer defers delivery of a Purchase Order or a part thereof, Customer shall not be subject to any deferment fees unless Customer defers a Purchase Order or a part thereof for more than [**] months in which case Customer will pay a deferment fee

of [**] percent ([**]%) of the total price of the deferred Purchase Order or part thereof. The deferment fee is due within [**] days from the date of Customer's receipt of an invoice. Partial deferments shall be made using validated Batch size increments. Any deferment fee paid under this Section will be credited against any cancellation fee that may become due with respect to the same Purchase Order under Section 4.7.1. Any deferment of a Purchase Order beyond the next [**] scheduled campaigns, or [**] months from the scheduled delivery date set forth in the Purchase Order, shall be deemed a cancellation subject to the fees set forth in Section 4.7.1.

5. PROCESSING OF PRODUCT

5.1 Storage and Handling. Agilent shall store and handle the raw materials (including PEG) and packaging components under appropriate conditions and temperature, humidity, light and cleanliness to avoid any material adverse effect on the identity, strength, quality and purity of such materials and components. Agilent shall store and handle the Product in accordance with the Specification and under appropriate conditions as defined by Customer in accordance with the Product stability studies and temperature, humidity, light and cleanliness to avoid any material adverse effect on the identity, strength, quality and purity of the Product.

5.2 Manufacturing Standards. Agilent shall manufacture the Product in conformity with the Process, Master Batch Record, GMP, the Quality Agreement and the Specification (the "Manufacturing Standards").

5.3 Shortage of Supply. Agilent shall notify Customer immediately upon becoming aware of an event of force majeure under Article 12 or any other event that would render Agilent unable to supply any quantity of the Product required to be supplied hereunder.

5.4 Risk Management.

5.4.1 Safety Stock; Deposit.

5.4.1.1 Except with respect to the supply of PEG, Agilent shall maintain a mutually agreed upon inventory level of critical raw materials and supplies ("Safety Stock") required for Agilent to manufacture and supply the Product for Customer based upon the then-current Forecast. The list of such critical raw materials and supplies is set forth in Exhibit O, which list (i) will be updated by the Parties within [**] days of the execution of this Agreement to include the agreed upon quantities of such raw materials and supplies and (ii) may otherwise be updated from time to time as mutually agreed to by the Parties. Agilent will acquire, maintain and store the Safety Stock [**]. The levels of Safety Stock shall be adjusted after each [**] based upon Customer's Product requirements set forth in the then-current Forecast.

5.4.1.2 Upon mutual agreement regarding the initial inventory level of the Safety Stock, Agilent shall invoice Customer, and Customer shall pay Agilent a one-time deposit (the "Deposit") in an amount [**] of the actual cost incurred by Agilent to acquire such raw materials that comprise the initial Safety Stock. In the event the then-current Forecast necessitates a Safety Stock level increase, Agilent will invoice Customer an additional amount to increase Customer's Deposit so that it is [**] of the cost of the inventory of such Safety Stock. In the event the Parties mutually agree to decrease the Safety Stock, once the excess raw materials have been consumed in the manufacture of Product, Agilent will credit Customer's account in an amount [**] of the value of the raw materials that were consumed and such credit shall be applied against the next invoice sent by Agilent to

Customer. All adjustments to Customer's Deposit with Agilent will be made [**] and will be memorialized in writing by Agilent with supporting documentation to be provided to Customer upon request.

5.4.1.3 Agilent will use commercially reasonable efforts to manage and use the Safety Stock in a manner that minimizes the risk that any amount of the Safety Stock expires before it can be used to manufacture Product hereunder, including, but not limited to, using the Safety Stock on a first in, first out basis. In the event that any raw materials that comprise Safety Stock expire due to Customer submitting Purchase Orders at levels materially below the then-current Forecast, an amount equal to the value of such expired raw materials in Safety Stock will be deducted from the Deposit for such Safety Stock and the expired material will be shipped to a destination of Customer's choosing or disposed of by Agilent upon Customer's direction.

5.4.1.4 Agilent shall at all times retain title to the Safety Stock and assumes the risk of loss of any or all of such Safety Stock. In the event any or all of the Safety Stock is damaged or destroyed, Agilent shall, [**], replace the damaged or destroyed Safety Stock with replacement raw materials of similar type and quality.

5.4.2 Risk Management Plan. Agilent shall maintain and comply with the risk management plan set forth in Exhibit K, which shall be updated [**] subject to Customer's prior written approval, which shall not be unreasonably withheld, conditioned or delayed.

5.5 Capacity. During the Term, Agilent will provide capacity to manufacture [**] Batches in 2016, [**] Batches in 2017 and [**] Batches of Product per calendar year thereafter in accordance with Purchase Orders that are issued by Customer in accordance with Section 4.3.1 and accepted by Agilent in accordance with Section 4.3.2. The Parties will negotiate in good faith increased capacity to be dedicated to Customer. If the Parties agree on such increased capacity, Agilent shall use commercially reasonable efforts to increase its capacity to the agreed upon units of Product within a reasonable time and Customer shall use commercially reasonable efforts to use the increased capacity built by Agilent.

5.6 Alternative Supplier. Agilent acknowledges that (i) it is the intent of Customer to establish one or more alternative suppliers to manufacture Product and (ii) in the process of establishing such an alternative supplier Customer will discuss the Product and Customer's Know-How, subject to Article 14. Customer shall be free to disclose to any such actual or proposed alternative supplier the Process overview set forth in Exhibit H.

5.7 Process Changes or Improvements. The Parties shall collaborate in good faith to evaluate and implement potential process improvements to increase Processing efficiencies and Agilent Product capacity. Agilent agrees that no change to the Process shall be made without the prior written approval of Customer. Notwithstanding the foregoing, any such change to the Process shall be subject to the agreed upon Change Management process as set forth in the Quality Agreement and the prior mutual agreement of the Parties with respect to the costs and expenses associated with the agreed upon change.

5.8 Supply Deficiency; Supply Failure; Technology Transfer

5.8.1 Definitions of Supply Deficiency and Supply Failure. A "Supply Deficiency" means Agilent has delivered less than [**] percent ([**]%) of the aggregate minimum yield for the Batch(es) of Product specified in any accepted Purchase Order in accordance with the delivery date set forth therein (the undelivered amount being referred to herein as the "Deficiency Amount"), unless such failure results from a delay or default by Customer

under the Agreement (including but not limited to a failure to supply sufficient amounts of PEG to enable Agilent to manufacture Product ordered by Customer hereunder). A "Supply Failure" means Agilent has failed to cure a Supply Deficiency by (i) delivering the full Deficiency Amount of Product within [**] days from the delivery date specified in the accepted Purchase Order or such other date as mutually agreed to by the Parties in writing; or (ii) issuing, at the time of the invoice, a credit to Customer for the Deficiency Amount, provided that the Deficiency Amount is equal to or less than [**] percent ([**]%) of the aggregate yield for the quantity of Product ordered. In the event that Agilent has delivered [**] percent ([**]%) or more of the aggregate yield for the Product ordered but less than [**]% of the amount of Product ordered in any accepted Purchase Order, Agilent will issue a credit to Customer for the difference. For the avoidance of doubt, a shortfall where Agilent has delivered at least [**] percent ([**]%) of the aggregate minimum yield for the Batch(es) of Product specified in any accepted Purchase Order in accordance with the delivery date set forth therein shall not constitute a Supply Deficiency.

5.8.2 Procedure to Address Supply Deficiency. In the event of a Supply Deficiency, Agilent will use commercially reasonable efforts to take one (1) or more of the following steps, as mutually agreed with Customer, in the following order of preference whenever practicable (i.e., with highest preference given to the remedy in (a) and the lowest preference given to the remedy in (d)): [**].

5.8.3 Alternative Supply for Supply Failure. In the event of a Supply Failure, notwithstanding the exclusivity provisions set forth in Section 3.5.2, Customer will have the right to purchase from one or more alternative suppliers all of its Product requirements, provided that Customer will use commercially reasonable efforts to limit any order of Product with alternative suppliers to the extent of and for the anticipated duration of the Supply Failure. Customer will resume purchase of its Product requirements under Section 3.5.2 from Agilent as soon as (a) Agilent reasonably demonstrates that Agilent is able to resume supplying Product to fulfill Customer's requirements and (b) Customer has fulfilled all obligations or commitments, if any, undertaken by Customer in connection with Customer's arrangement(s) with the alternative supplier(s). Any purchases of Product by Customer from an alternative supplier during a Supply Failure will, in addition to Customer's purchases from Agilent under this Agreement, count towards Customer's [**] percent ([**]%) purchase commitment from Agilent under Section 3.5.2.

5.8.4 Technology Transfer. In the event of (i) a Supply Failure, or (ii) termination of this Agreement by Customer pursuant to Section 13.2(a) or (b), and provided that Customer does not have a validated and approved alternative supplier of Product, Agilent and its Affiliates shall use their best efforts to co-operate, and cause their approved subcontractors to co-operate, in good faith and in accordance with a technology transfer plan to be agreed in good faith with Customer, to bring about a smooth and orderly transition to an alternative supplier(s) of Product as further specified in this Section. For the sake of clarity, the licenses granted in Sections 9.3.2.1 and 9.3.2.2 shall survive and remain in full force and effect. Agilent shall transfer to Customer or its designee the technology and materials agreed upon by the Parties in the technology transfer plan necessary for an alternative supplier(s) to develop, manufacture and supply the Product, including providing Customer or its designee sufficient information (including manufacturing documentation, testing methodologies and quality protocols). As agreed upon by the Parties in the technology transfer plan, Agilent shall transfer to Customer or its designee the Safety Stock and Customer shall pay Agilent an amount equal to the

remaining [**] cost of the inventory of such Safety Stock transferred that is not already covered by the Deposit. Agilent shall further provide the necessary technical assistance and required documentation necessary for transferring such development, manufacture and supply responsibilities to an alternative supplier(s) as agreed upon by the Parties in the technology transfer plan. If

applicable and to the extent requested by Customer and permitted thereunder, Agilent shall assign to Customer or its designee any Third Party agreements to which Agilent or its Affiliate is a party that relate solely to the development, manufacture and supply of the Product. In addition, Agilent will also assist Customer or its designee in obtaining other items necessary for the development, manufacture and supply of the Product. Agilent and its Affiliates and approved subcontractors shall be responsible for their costs and expenses in connection with this Section. Notwithstanding any other provision of this Agreement, the Parties acknowledge and agree that the obligations of Agilent, its Affiliates and their subcontractors under this Section 5.8.4 shall terminate [**] months after regulatory approval of the Finished Product.

6. PRICE AND PAYMENT

6.1 Pricing. Pricing for the Product shall be as set forth in each Purchase Order; provided that such pricing shall not exceed the pricing for Product set forth in Exhibit J except to the extent that the Manufacturing Standards as of the Effective Date for Product ordered are materially modified pursuant to the Change Management provisions set forth in the Quality Agreement; provided that any increase in pricing shall be proportionate to the increase in Agilent's costs to manufacture Product based on such modified Manufacturing Standards. Pricing for the Manufacturing Services shall be as set forth in each Statement of Work.

6.2 Payment. Agilent shall invoice Customer at the time of, as applicable, shipment of the Product in accordance with this Agreement or completion of the Manufacturing Services, unless otherwise agreed to by the Parties in a Statement of Work. Payment of an undisputed invoice for Product is due [**] days from the date of invoice. Payment of a disputed invoice for Product is due [**] days from the date of resolution of the dispute. Payment terms are subject to change if Customer's financial condition or payment record merits such change.

6.3 Taxes. Prices are exclusive of any sales, use, service, value added or other similar taxes. Any tax, duty, custom, insurance or other fee of any nature imposed on Product or services by any federal, state, local or foreign governmental authority shall be paid by Customer. If Agilent is required to pay any such tax or fee, Customer will reimburse Agilent promptly upon invoice by Agilent. If Customer claims exemption from any taxes, Customer will provide Agilent with an appropriate exemption certificate for the delivery jurisdiction. Each Party will be responsible for its own income, employment and property taxes.

6.4 Remedies. Agilent may temporarily discontinue its performance of the manufacture and supply obligations under this Agreement if Customer fails to pay any sum when due and Customer has not cured such failure within [**] days after receipt of written notice from Agilent identifying such failure.

6.5 Manufacturing Cost Reductions. The Parties will work together during the Term to identify opportunities to reduce the cost of manufacturing Product. Any such cost reductions shall be subject to Section 5.7 and the Change Management process set forth in the Quality Agreement. With respect to all other reductions in costs, the Parties shall negotiate in good faith decreases to the price for the Product.

6.6 Manufacturing Cost Increases. The Parties agree that Agilent shall have, after good faith negotiations with Customer, the right to increase Product prices provided that Agilent can

demonstrate that such increases are based upon unavoidable increases in material costs. Any increases in labor costs will cause the Parties to negotiate in good faith increases to the price of the Product, provided that such increases shall not exceed the Producer Price Index percentage [**] as published by the United States Department of Labor Producer Price Index for Chemical Manufacturing Series ID:PCU325—325—, or any such replacement or substitute index published by the United States government in the event the Producer Price Index is discontinued. Price increases under this Section may not be made more than [**].

7. REPRESENTATIONS AND WARRANTIES

7.1 General Representations and Warranties. Each Party represents and warrants to the other Party that (i) it has the right and authority to enter into this Agreement and to carry out its obligations hereunder; (ii) it is validly existing in each jurisdiction in which it is incorporated and is authorized to do business under the laws of each jurisdiction in which it engages in business activities; and (iii) it is not aware of any legal, contractual or other restriction, limitation or condition that might adversely affect its ability to perform its obligations hereunder.

7.2 Warranties by Agilent. Agilent warrants to Customer that (i) all Product supplied under this Agreement shall conform to the Specification at the time of delivery to Customer's carrier; (ii) all Product delivered under this Agreement shall be manufactured in accordance with GMP; (iii) all Product delivered hereunder shall be free from Latent Defects; and (iv) all Product delivered hereunder shall be delivered to Customer free and clear of all liens and security interests. Agilent warrants to Customer that all Manufacturing Services shall be performed in a professional and workmanlike manner consistent with industry standards. The warranties set forth in this Section (i) survive acceptance of the Product or Manufacturing Services by Customer (including any Batch Packet acceptance); and (ii) are for the sole benefit of Customer.

7.3 IP Warranty by Agilent. Except as otherwise provided in this Section 7.3, Agilent warrants to Customer that, as of the Effective Date, to the best of Agilent's knowledge, the Process does not (i) infringe any Third Party patents issued as of the Effective Date or (ii) infringe or misappropriate any other intellectual property rights of any Third Party existing as of the Effective Date ("IP Warranty"). [**]. In the event of breach of the foregoing IP Warranty, Customer's sole and exclusive remedy, and Agilent's sole liability shall be as follows: (i) Agilent will defend or settle any Third Party claim against Customer, its officers, directors, and employees in accordance with Section 9.8, Third Party Infringement Claims, and (ii) in the event that a court of competent jurisdiction determines that the Process infringes the Third Party's intellectual property rights [**] Agilent, at its cost and sole option, will either (a) with Customer's written consent, which consent shall not be unreasonably conditioned, withheld or delayed, modify the Process so that it is non-infringing or (b) obtain any necessary license.

7.4 Warranties by Customer. Customer warrants to Agilent that (i) as of the Effective Date, to the best of Customer's knowledge, it owns or has the necessary rights, title and interest in and to the Product, including the right under Patents owned or controlled by Customer to have

Product made for Customer, and (ii) as of the Effective Date, Customer has not received any written notification alleging that the Product infringes or misappropriates the intellectual property rights of any Third Party.

7.5 Remedies. In the event that (a) the Product supplied under this Agreement fails to conform to the warranties set forth in Section 7.2 of this Agreement, (b) the Parties agree that the Product has a Latent Defect in accordance with Section 8.2.3.4 or an Independent Laboratory so determines in accordance with Section 8.2.3.5, or (c) the Product is not in Good Condition for any reason other than that the Product is not in the right quantity in accordance with the manifest, (i) Agilent may elect either to collect and dispose of the affected Product, at Agilent's expense, or to reimburse

Customer for any reasonable costs incurred by Customer to collect and dispose of the affected Product; (ii) Agilent shall reimburse Customer for all reasonable costs incurred by Customer in connection with delivery of the affected Product, including freight, clearance, duty and storage charges; and (iii) Agilent shall promptly, at no additional cost to Customer (subject to Section 3.3.3 and 3.3.4, as applicable), (y) replace the affected Product as soon as reasonably practicable with Product that meets the requirements of Section 3.1 or (z) rework the affected Product, subject to mutual agreement of the Parties. In the event that Agilent fails to replace or rework the affected Product within the timeframe mutually agreed to by the Parties, (1) Agilent will refund to Customer any amounts paid for such Product, [**]. In the event that Manufacturing Services performed under this Agreement fail to conform to the warranty set forth in Section 7.2 of this Agreement, Agilent will promptly, at Agilent's cost, re-perform such Manufacturing Services, provided that Agilent receives notice from Customer within [**] business days after such Manufacturing Services were completely delivered. [**].

7.6 No Warranty to Third Parties. The warranties set forth in Section 7.2 and Section 7.3 are solely for the benefit of Customer. Agilent makes no warranty to Customer's end user customers or any other Third Party. Customer will not pass on to any end user customer or any other Third Party any warranty or representation on behalf of Agilent.

7.7 DISCLAIMER. THE WARRANTIES SET FORTH IN THIS ARTICLE 7 ARE EXCLUSIVE. THE REMEDIES SET FORTH IN SECTION 7.3 ARE EXCLUSIVE WITH RESPECT TO THE WARRANTIES IN SECTION 7.3. EXCEPT AS EXPRESSLY PROVIDED HEREIN, NEITHER PARTY MAKES NOR RECEIVES ANY WARRANTY OF ANY KIND, EXPRESS, IMPLIED, STATUTORY OR OTHERWISE, INCLUDING WARRANTIES OF DESIGN, SUITABILITY OF QUALITY, OR ARISING FROM A COURSE OF DEALING OR USAGE OF TRADE PRACTICE, WITH REGARD TO THE PRODUCT. AGILENT SPECIFICALLY DISCLAIMS THE IMPLIED WARRANTIES OF MERCHANTABILITY, NON-INFRINGEMENT AND FITNESS FOR A PARTICULAR PURPOSE.

8. QUALITY

8.1 Quality Agreement. Each Party will comply with the terms of the Quality Agreement in the performance of its obligations hereunder including record retention, audits and inspections, change control, adverse events and product recall. The Parties will conduct periodic Product quality reviews in accordance with the terms of the Quality Agreement.

8.2 Quality Assurance.

8.2.1 Testing by Agilent. Agilent shall perform quality testing using assays mutually agreed to by the Parties in order to assure that Product complies with the Specification, and shall retain samples of Product as required by applicable law and produce records of the tests made on each Batch. Agilent shall provide Customer a Certificate of Analysis and Certificate of Compliance confirming the performance of such testing. Customer may elect, at its sole discretion, to attend and observe any testing conducted by Agilent in accordance with this Section 8.2.1. In addition, no Product shall be delivered until such Product has been Processed in accordance with the agreed upon testing specifications; provided, however, that the foregoing shall not relieve Agilent of its obligation under this Section 8.2. With respect to PEG, Agilent shall perform quality testing of PEG in accordance with Agilent's approved standard operating procedures and report results to Customer within [**] days of Agilent's receipt of PEG.

8.2.2 Records. Capitalized terms used in this Section 8.2.2 that are not defined in this Agreement shall have the meaning set forth in the Quality Agreement. Agilent shall maintain records, including Master Batch Records and Batch Production Records, with

respect to the manufacturing and quality testing of the Product and shall deliver the Executed Batch Record (in electronic PDF format) to Customer prior to Agilent providing the complete Batch Packet to Customer. Agilent shall provide a point of contact, familiar with the Executed Batch Record, through which information about the Executed Batch Record can be obtained in a timely and organized manner to improve and expedite the Customer review. In addition, Agilent will provide to Customer the completed, executed batch records for each manufacturing step as such step is completed and approved by Agilent's Manufacturing and Quality groups, on a rolling basis (and in any event, within [**] days of the completion of the manufacturing step) in order to expedite completion of the final Batch Packet review. Customer will have [**] business days following receipt of the complete Executed Batch Record to advise Agilent of any deficiencies or corrections needed with the Executed Batch Record. Agilent will subsequently provide Customer a complete Batch Packet (in electronic PDF format). Agilent shall not ship Product hereunder unless and until: (i) Agilent has provided to Customer the Batch Packet for such Product and under the condition that all opened deviations, investigations or other anomalous events related to such Batch have been resolved, and (ii) Customer has reviewed the Batch Packet for such shipment and authorized such shipment in writing. Upon receipt of a complete copy of the Batch Packet, Customer will use commercially reasonable efforts to review such Batch Packet, and advise Agilent of any deficiencies or corrections needed within [**] business days ("Target Review Period"). Upon Customer's request, during the Target Review Period [**] Agilent will make available for up to [**] business days the necessary personnel for in-person, on-site meetings at the Facility (or such other location as the Parties may agree) to facilitate Customer review of the Batch Packet. If Customer is to exceed the Target Review Period, [**]. Following Agilent's resolution (to Customer's satisfaction) of any issues raised by Customer's review of the Batch Packet, Agilent shall ship the Batch of Product. Notwithstanding the foregoing, in the event that

(a) Customer fails to provide such authorization within [**] business days after Customer's receipt of the Batch Packet, and (b) Customer has not within such [**] business day period submitted to Agilent any questions or requests for information and (c) Customer does not within such [**] business day period find fault or anomaly with the balance of the Batch Packet documentation, then Agilent may ship the associated Batch of Product and Customer shall be deemed to have accepted the Batch Packet, [**].

8.2.3 Non-Conforming Product. Notwithstanding any prior acceptance of Product (including any Batch Packet acceptance) by Customer, the following shall apply with respect to non-conforming Product:

8.2.3.1 Inspection/Testing. Upon receipt of each delivery of Product from Agilent under this Agreement, Customer shall report to Agilent within [**] business days of Customer's receipt of Product from Customer's carrier if the Product does not conform to the quantity specified in the Purchase Order, or if the Product is otherwise not in Good Condition.

8.2.3.2 Failure to Conform to Good Condition. In the event Customer notifies Agilent pursuant to Section 8.2.3.1 that the Product is not in Good Condition, Agilent shall have the right to inspect and analyze the Product within [**] business days of Agilent's receipt of the Product or Agilent's receipt of visual evidence demonstrating that the Product is not in Good Condition. In the event that the Parties agree that the Product was not in Good Condition at the time of delivery to Customer's carrier, Customer shall have the remedies as set forth in Section 8.2.3.6. If the Parties cannot agree as to whether the Product was in Good

Condition at the time of delivery, the matter shall be escalated in accordance with Section 16.b, Escalated Dispute Resolution.

8.2.3.3 Latent Defect. In the event Customer discovers that the Product has a Latent Defect, Customer shall promptly notify Agilent in writing providing specific details about the nature of the Latent Defect.

8.2.3.4 Notification from Customer. In the event Customer notifies Agilent pursuant to Section 8.2.3.3 that the Product has a Latent Defect, (i) Agilent shall have the right to inspect and analyze the Product within [**] business days of Agilent's receipt of a suitable quantity of such Product and (ii) the Parties shall work together in good faith to reach agreement as to whether the Product has a Latent Defect. In the event the Parties agree that the Product has a Latent Defect, Customer shall have the remedies set forth in Section 7.5.

8.2.3.5 Independent Laboratory. In the event the Parties fail to agree whether the Product has a Latent Defect, the matter shall be referred to an Independent Laboratory. Agilent shall forward a sample of retained Product from the Batch in question to the Independent Laboratory for testing and control purposes. Customer may also forward a sample of the affected Product to the Independent Laboratory for such evaluation. The Parties shall mutually agree to the controls and procedures used by the Independent Laboratory to test the Product. Each Party shall have the right to audit the Independent Laboratory to determine whether there was any departure from the established controls and procedures used to test the Product. In the event a Party determines that there was a departure from the established controls and procedures, the Party shall notify the other Party in writing within [**] business days and the Parties shall resolve the matter in accordance with Section 16.b. In the absence of such determination, the decision of the Independent Laboratory shall be final and binding on the Parties. If the Independent Laboratory determines that the Product has a Latent Defect, then the Independent Laboratory's fees shall be borne by Agilent. If the Independent Laboratory determines that the Product does not have a Latent Defect, then Customer shall bear the Independent Laboratory's fees and reimburse Agilent for any reasonable direct costs incurred by Agilent in connection with the Independent Laboratory's analysis of the Product.

8.2.3.6 Customer's Remedies. In the event that the Product is not in Good Condition because the Product is not in the right quantity in accordance with the manifest, Sections 5.8.1-5.8.4 shall apply. In the event that the Product is not in Good Condition for any other reason, or in the event that the Product has a Latent Defect, the remedies set forth in Section 7.5 shall apply.

8.2.4 Audit Rights. Customer shall have the right to conduct audits and inspections of the Facility, Agilent's manufacturing operations and Agilent's records relating to this Agreement as provided in the Quality Agreement. Agilent shall cooperate with Customer in conducting such audits and inspections, including scheduling any requested audit to take place in accordance with the Quality Agreement.

8.2.5 Observation by Customer. During the Term, Customer shall have the right, at Customer's sole cost and expense, during normal business hours and upon reasonable notice, to visit the Facility in order to ensure that the Processing complies with applicable legal requirements and the Specification, as applicable. Agilent shall reasonably cooperate with Customer to permit Customer such access in connection with such visits. At all times while in attendance at the Facility, Customer agrees to comply

with all Agilent health and safety protocols and other policies and procedures applicable to visitation of the Facility as notified by Agilent to Customer prior to or during such attendance. Such visits shall not interfere with Agilent's operations.

8.2.6 Recalls and Voluntary Withdrawals. If either Party becomes aware of information about the Product or Finished Product indicating that it may be non-conforming Product or Finished Product or that there is potential adulteration, misbranding and/or any potential issues regarding the safety or effectiveness of the Product or Finished Product, it shall within [**] hours provide

notice to that effect to the other Party. Customer will initiate an investigation and assessment of such circumstances and shall promptly notify Agilent of its findings and any proposed course of action. The Parties shall meet to discuss such circumstances and to consider appropriate courses of action. Customer shall bear all costs associated with a recall of the Finished Product except to the extent such recall is caused by (i) the negligence or willful misconduct [**]; (ii) [**]; or (iii) Agilent's failure to manufacture the Product in accordance with GMP, in which cases Agilent shall pay Agilent's share of such costs associated with the recall. The determination as to each Party's [**]. For purposes of this Section, the costs associated with a recall of the Finished Product are defined [**]. In the event of a dispute with respect to responsibility for costs associated with a recall, such dispute shall be escalated for resolution in accordance with Section 16.b.

9. INTELLECTUAL PROPERTY

9.1 Background Property. Each Party retains all right, title and interest in and to all Intellectual Property owned, licensed or developed by or on behalf of such Party prior to the Effective Date or independent of this Agreement, and without reliance on the other Party's Proprietary Information.

9.2 Ownership of Developed Intellectual Property.

9.2.1 Customer shall be the sole owner of all right, title and interest in and to all Intellectual Property relating specifically to the Product, including the Specification and all improvements to the Product and Specification that are (i) jointly developed by Customer or its employees or consultants on the one hand and Agilent or its Affiliates, employees or consultants on the other hand, during the course of performing or receiving services hereunder or (ii) developed by Agilent or its Affiliates, employees or consultants during the course of performing Manufacturing Services under a Statement of Work or during the course of performing services for Customer under any Purchase Order, ((i) and (ii) collectively, "Product Improvements"). Agilent hereby assigns to Customer all of its right, title and interest in Product Improvements. Agilent agrees to execute such assignments and other documents and to take such other actions as may be reasonably requested by Customer from time to time, at Customer's expense, in order to effect the ownership provisions of this Section 9.2.1. For avoidance of doubt, intellectual property relating to the Processing of nucleic acids and the Processing of modified nucleic acids, including pegylated nucleic acids, which is not sequence or Product specific shall not be considered to be a "Product Improvement" but shall be considered to be Intellectual Property relating to the Process, as provided in Section 9.2.2 below and not subject to the obligation to assign provided in this Section 9.2.1.

9.2.2 Agilent shall be the sole owner of all right, title and interest in and to all Intellectual Property relating to the Process, including all improvements thereto, that are developed by Agilent or its Affiliates, employees or consultants. In addition, Agilent shall be the sole owner of all right, title and interest in and to all Intellectual Property relating to the Process, including all improvements thereto, that are jointly developed by Customer or

its Affiliates, employees or consultants and Agilent or its Affiliates, employees or consultants, during the course of performing or receiving services hereunder ("Joint Process Improvements"). Customer hereby assigns to Agilent all of its right, title and interest in Joint Process Improvements, except as otherwise provided in Section 9.3.2.1 below. Customer agrees to execute such assignments and other documents and to take such other actions as may be reasonably requested by Agilent from time to time, at Agilent's expense, in order to effect the ownership provisions of this Section 9.2.2.

9.3 License Grants.

9.3.1 License to Agilent.

9.3.1.1 During the Term, Customer hereby grants to Agilent a fully paid, non-exclusive, non-sublicensable (except as otherwise permitted under Section 9.5), non-transferable (except to a permitted assignee in accordance with Section 16(e) ("Permitted Assignee")) license under any and all Customer Intellectual Property that is necessary for Agilent to perform its obligations under this Agreement, for the sole and limited purpose of Agilent's performing its obligations under this Agreement.

9.3.2 Licenses to Customer.

9.3.2.1 Agilent hereby grants to Customer a worldwide, fully paid-up, royalty-free, perpetual, non-sublicensable (except in accordance with this Section 9.3.2.1), non-transferable and non-assignable (except to a Permitted Assignee), (x) non-exclusive license under Joint Process Improvements; and (y) non-exclusive license under analytical methods that are developed by Agilent or its Affiliates, employees or consultants in the performance of a Statement of Work or a Purchase Order, ("Analytical Methods") (together with Joint Process Improvements, collectively, "Licensed Technology") to manufacture, have manufactured, produce, have produced, develop, have developed, use, have used, offer for sale, have offered for sale, sell, have sold, import, and have imported the Product and Finished Product, subject to the following: (i) any sublicense granted by Customer to a Third Party manufacturer or a Third Party that Customer has granted a license under Customer Intellectual Property to develop or manufacture Product ("Customer Licensee") and/or Finished Product shall be restricted to using the Licensed Technology for the sole purpose of performing services (including development and manufacturing services) for the Product or Finished Product exclusively for Customer, Customer's Affiliates, Customer Licensees or a Permitted Assignee and (ii) prior to disclosing any Licensed Technology to any Third Party, Customer shall enter into a valid written confidentiality agreement with such Third Party that (a) requires the Third Party to maintain the confidentiality of Agilent Proprietary Information contained in the Licensed Technology under terms no less restrictive than those set forth in Article 14 of this Agreement and (b) restricts the Third Party from using the Licensed Technology for any purpose other than to perform services for the Product or Finished Product exclusively for Customer, Customer's Affiliates, Customer Licensees or a Permitted Assignee in accordance with this Section 9.3.2.1. In addition, any sublicense to Analytical Methods granted by Customer under this Section 9.3.2.1 to a

Third Party manufacturer of Finished Product shall be restricted to those Analytical Methods that are necessary to manufacture and release the Finished Product. In addition to the non-exclusive license granted above, and solely with respect to Joint Process Improvements,

Agilent hereby grants to Customer a worldwide, fully paid-up, royalty-free, perpetual, non-sublicensable (except in accordance with this Section 9.3.2.1), non-transferable and non-assignable (except to a Permitted Assignee) license to use Joint Process Improvements on a non-exclusive basis to manufacture, have manufactured, produce, have produced, develop, have developed, use, have used, offer for sale, have offered for sale, sell, have sold, import, and have imported products controlled by Customer, provided that (i) any sublicense granted by Customer to a Third Party manufacturer or a Customer Licensee shall be restricted to using the Joint Process Improvements for the sole purpose of performing services (including development and manufacturing services) exclusively for Customer, Customer's Affiliates, Customer Licensees or a Permitted Assignee and (ii) prior to disclosing any Joint Process Improvements to any Third Party, Customer shall enter into a valid written confidentiality agreement with such Third Party that (a) requires the Third Party to maintain the confidentiality of Agilent Proprietary Information contained in the Joint Process Improvements under terms no less restrictive than those set forth in Article 14 of this Agreement and (b) restricts the Third Party from using the Joint Process Improvements for any purpose other than to perform services exclusively for Customer, Customer's Affiliates, Customer Licensees or a Permitted Assignee in accordance with this Section 9.3.2.1. Except as expressly provided herein, no license to any Licensed Technology is granted, conveyed or implied. [**].

9.3.2.2 Agilent hereby grants to Customer, for the life of the Licensed Patents, a non-exclusive, fully paid-up, royalty-free, non-sublicensable (except in accordance with this Section 9.3.2.2), non-transferable and non-assignable (except to a Permitted Assignee) license, under the Licensed Patents, to make, have made, use, have used, import, have imported, offer for sale, have offered for sale, sell and have sold the Product and Finished Product, subject to the following: any sublicense granted by Customer to a Third Party manufacturer or Customer Licensee shall be restricted to developing and manufacturing the Product or Finished Product exclusively for Customer, Customer's Affiliates, Customer Licensees or a Permitted Assignee and shall contain a provision identifying Agilent as an intended third party beneficiary of, and entitled to enforce, any such sublicense. Except for any future licenses that may be granted by Agilent pursuant to Section 7.3 in a written amendment to this Agreement in accordance with Section 16.j, no other license is granted by Agilent under this Agreement, either directly or by implication, under any Patent other than the Licensed Patents. [**].

9.3.2.3 With respect to the licenses set forth in Sections 9.3.2.1 and 9.3.2.2, the Parties hereby agree that the licenses for the Product and Finished Product under this Agreement are hereby extended to the ARC1905 molecule (Zimura).

9.4 Reservation of Rights. Except as expressly provided herein, no license to any Agilent Intellectual Property or Customer Intellectual Property is granted, conveyed or implied. All rights not conferred are expressly reserved.

9.5 Subcontracting. Agilent shall only engage those Affiliates and Third Parties approved by Customer in writing to manufacture the Product and shall not sub-license the rights under any Customer Intellectual Property other than to such approved Affiliates and Third Parties and solely for the purpose of manufacturing and supplying Product to Customer and provided that any such approved Affiliate or Third Party shall be subject to Agilent's obligations contained in this

Agreement. Agilent shall be responsible for any breach of this Agreement by any such Affiliates and Third Parties subject to Article 15.

9.6 Process Patents, Process Overview and Know-How.

9.6.1 Process Patents. All Patents owned or licensed by Agilent or its Affiliates that cover or claim the Process are set forth in Exhibit F. During the Term, upon the reasonable request of Customer, no more than [**], Agilent shall update Exhibit F. Agilent shall not incorporate into the Process any claims covered by Patents unless the Parties have agreed to incorporate such claims into the Process pursuant to the Change Management process.

9.6.2 Process Overview. An overview of the Process is attached to this Agreement as Exhibit H. Agilent acknowledges and agrees that Exhibit H does not contain any Agilent Proprietary Information and that Customer may disclose Exhibit H, or any information contained therein, to any Third Party to the extent such Third Party has a reasonable need to know such information.

9.6.3 Third Party Know-How. Agilent has not and shall not incorporate into the Process any Third Party Know-How unless (A) Agilent has the right to incorporate such Third Party Know-How into the Process and (B) the Parties have agreed to incorporate such Third Party Know-How into the Process pursuant to the Change Management process.

9.7 Licenses to Use the Process. Agilent is responsible for the procurement of any licenses to Intellectual Property necessary to use the Process to manufacture the Product under this Agreement. Agilent shall have full responsibility for the determination of whether and from which Third Party it requires any such license to Intellectual Property claiming or covering the Process for the manufacture of the Product under this Agreement and for the procurement of any such license. For purposes of clarity, nothing in this Section 9.7 shall limit or prevent Customer, in its sole discretion, from obtaining any license or other rights to any Third Party Intellectual Property it considers necessary or useful to manufacture the Product.

9.8 Third Party IP Existing as of the Effective Date. Agilent will defend or settle any Third Party claim against Customer, its officers, directors, and employees that (i) Agilent's use of the Process to manufacture the Product under this Agreement or (ii) any Manufacturing Services provided by Agilent under this Agreement (a) infringes any Third Party patents issued as of the Effective Date or (b) infringes or

misappropriates any other intellectual property rights of any Third Party existing as of the Effective Date. Agilent shall not settle or compromise any action or proceeding under this Section 9.8 that adversely affects Customer's rights and interests without the written consent of Customer, which consent shall not be unreasonably conditioned, withheld or delayed. The Parties shall comply with the indemnification process set forth in Section 10.3 with respect to any such Third Party claims. Agilent will pay infringement defense costs, settlement amounts and court awarded damages in connection with infringement claims under this Section 9.8. Agilent shall have no obligation under this Section 9.8 for any claim of infringement arising from Product use prohibited by this Agreement. This Section 9.8 states Customer's sole and exclusive remedy and Agilent's sole liability with respect to any such Third Party claim.

9.9 Third Party IP Arising after the Effective Date. In the event of a claim or allegation that the Process or the Manufacturing Services (a) infringes any Third Party patents issued after the Effective Date or (b) infringes or misappropriates any other intellectual property rights of any Third Party that first came into existence after the Effective Date, the Party first having notice of the claim or assertion shall promptly notify the other Party and the Parties shall negotiate in good faith and jointly determine (i) any necessary or desirable action to remediate the same, which may include modifying the Process so that it is non-infringing, obtaining any necessary license and/or opposing the claim or allegation, and (ii) the allocation of related costs.

10. INDEMNITIES AND INSURANCE

10.1 Agilent's Indemnity Obligations. Agilent will indemnify, defend and hold harmless Customer, its officers, directors, and employees, from and against any and all claims, losses, damages, demands, expenses or other liability arising out of a Third Party claim to the extent caused by (i) failure of the Product to conform to the Specification at the time of delivery to Customer's carrier; (ii) Agilent's failure to manufacture the Product in accordance with GMP; or (iii) the negligence or willful misconduct of Agilent or its officers, directors, employees, Affiliates, subcontractors or suppliers. Agilent's obligations under this Section 10.1 do not apply with respect to any claim to the extent such claim is subject to indemnification under Section 10.2.

10.2 Customer's Indemnity Obligations. Customer will indemnify, defend and hold harmless Agilent, its officers, directors, and employees, from and against any and all claims, losses, damages, demands, expenses or other liability arising out of a Third Party claim to the extent (i) arising from the sale, marketing or distribution of the Product or Finished Product, or use of the Product or Finished Product, by Customer or its officers, directors or employees or any Third Party including death or injury to any person; or (ii) caused by the negligence or willful misconduct of Customer or its officers, directors or employees, Affiliates, subcontractors or suppliers. Customer's obligations under this Section 10.2 do not apply with respect to any claim to the extent such claim is subject to indemnification under Section 10.1.

10.3 Process. Each Party agrees to notify the other Party promptly upon receipt of any claim for which indemnification is sought. The Party seeking indemnification will provide the indemnifying Party with such information and assistance as the indemnifying Party may reasonably request, at the expense of the indemnifying Party. In no event may either Party compromise or settle any claim or suit in a manner that adversely affects the rights and interests of the other Party (or any indemnitee) without the prior written consent of the other Party, which consent shall not be unreasonably conditioned, withheld or delayed. The indemnifying Party shall have no liability under this Article 10 with respect to claims or suits settled or compromised by the indemnified Party (or any indemnitee) without the indemnifying Party's prior written consent, which shall not be unreasonably withheld, conditioned or delayed. The indemnified Party may, at its own expense, participate in the defense of any claim. In the event that the indemnifying Party fails to assume control of the defense of any claim, the indemnified Party may assume control at the expense of the indemnifying Party.

10.4 Insurance. During the Term, Agilent will maintain insurance coverage in accordance with the Memorandum of Insurance attached hereto as Exhibit D.

11. COMPLIANCE WITH LAWS AND REGULATORY MATTERS

11.1 Compliance with Laws. Each Party shall comply with all applicable laws and regulations governing the performance of such Party's obligations under this Agreement. Without limiting the foregoing, Agilent shall ensure that the Facility and Product conform to GMP and the requirements of all applicable Regulatory Authorities and Customer shall ensure that the Finished Product conforms to GMP and the requirements of all applicable Regulatory Authorities.

11.2 Regulatory Filings. Customer, at its expense, shall be solely responsible for the preparation, filing and maintenance of all regulatory documents and all governmental permits, licenses and other approvals as may be necessary with respect to the formulation, marketing, distribution, sale and use of the Product and Finished Product. Upon Agilent's request, Customer will provide Agilent with a copy of such regulatory documents to the extent they relate to the manufacture of Product under this Agreement.

11.3 Codes of Conduct. Agilent agrees to maintain, in accordance with industry practices, and comply with Agilent's Standards of Business Conduct available at http://www.agilent.com/supplier/downloads/StandardsBusinessConduct.pdf.

11.4 Permits and Commercial Readiness Plan.

11.4.1 Permits. Agilent at its expense shall be solely responsible for, and has the obligation to prepare, file and maintain all licenses, permits and approvals as may be necessary with respect to the manufacture of the Product and performance of Manufacturing Services at the Facility, including all regulatory approvals required to import raw materials and packaging components. Upon Customer's request, Agilent will provide Customer with a copy of such documents to the extent they relate to the manufacture of the Product or the performance of Manufacturing Services under this Agreement.

11.4.2 Commercial Readiness Plan. Agilent's Commercial Readiness Plan is attached hereto as Exhibit N. [**]. Agilent's obligations under this Section 11.4.2 shall terminate [**] months after regulatory approval of the Finished Product.

11.5 Hazardous Waste. Any hazardous waste generated during the manufacture of the Product under this Agreement will be disposed of by Agilent in accordance with Agilent procedures at Agilent's cost and expense.

11.6 Export Controls. Each Party shall comply with applicable US and other laws, rules and regulations that govern the import, export and re-export of the Product, including the U.S. Export Administration Regulations, and will obtain any required export and import authorizations.

11.7 Record Retention. Agilent shall maintain the records and documentation relating to the manufacture of the Product in accordance with ICH guidance, Agilent's Standard Operating Procedure and the Quality Agreement.

11.8 Technical Support.

11.8.1 Upon notification to Agilent that Customer has received a complaint or inquiry regarding the safety, efficacy or quality of the Product or Finished Product, Agilent shall, within a reasonable period, supply Customer with a chemical analysis of a number of retained samples, maintained in accordance with the Quality Agreement, of the Batch(es) of the Product in question.

11.8.2 Upon notification to Customer that Agilent has received a complaint or inquiry regarding or discovery by Customer of any issues relating to the safety, efficacy or quality of the Product or Finished Product, Customer shall, within a reasonable period, provide technical support as reasonably requested by Agilent, which may include, but shall not be limited to, technical advice and chemical analysis of retained samples of the Product, maintained in accordance with the Quality Agreement.

11.8.3 Except as set forth in Section 8.2.6, all technical support provided by Agilent under this Section 11.8 shall be subject to the pricing and payment terms for technical and regulatory support as set forth in a Statement of Work agreed upon by the Parties.

11.9 Regulatory Support.

11.9.1 Agilent agrees to cooperate with, and provide regulatory assistance to, Customer to support existing, pending or new Product or Finished Product registrations and marketing approvals, in each case, with any relevant governmental authority. The foregoing assistance rendered by Agilent may include: (i) assisting Customer in completing and submitting changes to any regulatory submissions related to the Product; (ii) cooperation in connection with pre-approval inspections carried out by governmental

authorities; and (iii) providing information to Customer that may be required by a relevant governmental authority to support the Product or Finished Product, including the manufacturing and exportation related thereto. Except as set forth in Section 8.2.6 and except for the general requirements for API manufacturers set forth in the GMP and all laws and regulations applicable to the manufacture and supply of API, all Product-specific regulatory support provided by Agilent under this Section 11.9 shall be subject to the pricing and payment terms for technical and regulatory support as set forth in a Statement of Work agreed upon by the Parties.

11.10 FDA Debarment Statement. Agilent hereby certifies that neither Agilent nor any employee engaged by Agilent to perform services under this Agreement has been debarred under section 306 of the Federal Food, Drug and Cosmetic Act in connection with the performance of services under this Agreement or any comparable law or regulation outside of the United States. In the event that Agilent becomes aware of any such debarment, Agilent will provide Customer with written notice thereof. Agilent will request that all GMP manufacturing and testing subcontractors utilized pursuant to Section 2.4 of the Quality Agreement provide Customer with a certification that is substantially similar to the certification provided by Agilent in this Section 11.10. In the event that any such subcontractor fails to provide the certification, Customer may withdraw its approval for such subcontractor and Agilent shall cease using such subcontractor to provide services under this Agreement.

12. FORCE MAJEURE

Neither Party will be liable for any failure or delay in performance of its obligations under this Agreement to the extent such failure or delay is caused by any event beyond such Party's reasonable control, including fire, flood, explosion, unavailability of utilities or raw materials, labor difficulties, war, riot, act of God, export control regulation, or other laws or regulations, action or failure to act of any governmental authority, or any judgment, injunction or order of a court, administrative agency or regulatory authority having the effect of preventing or adversely affecting either Party's performance under this Agreement.

13. TERM AND TERMINATION

13.1 Term. Unless otherwise terminated under this Article 13, this Agreement will commence as of the Effective Date and will continue for seven (7) years from the date of Customer's first commercial sale of the approved Finished Product (the "Initial Term"). Unless otherwise terminated in accordance with this Article 13, this Agreement shall be automatically extended for one two-year period (the "Renewal Term" and together with the Initial Term, the "Term"). Notwithstanding any of the foregoing, either Party may terminate this Agreement at the end of the Initial Term or during the Renewal Term provided, however, that if Customer desires to terminate it has given Agilent at least eighteen (18) months prior written notice of termination and if Agilent desires to terminate it has given Customer at least twenty-four (24) months prior written notice of termination.

13.2 Termination.

(a) Subject to Section 13.2(c), this Agreement or a Statement of Work may be terminated by either Party upon [**] days written notice in the event of a material breach of any provision of this Agreement or such Statement of Work; provided, however, that the breaching Party will have an opportunity to (i) cure the breach during the [**], or (ii) provide the non-breaching Party with a plan to remedy the breach

within the [**], and if so cured, no termination will be deemed to have occurred as long as the breaching Party diligently pursues the plan to remedy the breach and completes such plan in accordance with the time frame mutually agreed to by the Parties (such time frame not to exceed an additional [**] days).

(b) This Agreement may be terminated by either Party immediately upon written notice to the other Party (i) if the other Party makes an assignment for the benefit of creditors; (ii) if proceedings in voluntary or involuntary bankruptcy are initiated by, on behalf of or against the other Party (and, in the case of any such involuntary proceeding, not dismissed within ninety (90) days); (iii) if the other Party is adjudicated bankrupt, files a petition under insolvency laws, is dissolved or has a receiver appointed for substantially all of its property; or (iv) if the other Party ceases operation of its business as its business has normally been conducted, or terminates substantially all of its employees.

(c) In the event of either Party's material breach of its confidentiality obligations under Article 14, the Parties shall refer the matter for resolution under the escalated dispute resolution process set forth in Section 16(b). For the avoidance of doubt, [**].

(d) In the event that Customer fails, from the date of Customer's first commercial sale of the approved Finished Product, under this Agreement for a period of thirty-six (36) months (i) to place a Purchase Order for a minimum of one hundred (100) oligonucleotide grams of Product and (ii) to take delivery of such Product within the lead times as set forth in Section 4.3.1 of this Agreement, Agilent shall have the right to terminate this Agreement upon written notice to Customer without further opportunity to cure.

(e) In the event that Agilent has failed to cure any Supply Failure under this Agreement by delivering the full amount of Product ordered within [**] months of such Supply Failure, Customer may terminate this Agreement upon written notice to Agilent.

(f) Customer may terminate this Agreement immediately if any Regulatory Authority issues a final order or determination that prevents Customer from supplying the Product or Finished Product or exporting, purchasing or selling the Product or Finished Product. Additionally, Customer shall have the right to terminate this Agreement immediately if the Product or Finished Product cannot be reasonably commercialized for medical, scientific or legal reasons, including reasons arising out of clinical trials.

13.3 Effect of Termination or Expiration.

13.3.1 Section 3.5.1 shall survive termination or expiration of this Agreement unless this Agreement is terminated by Agilent pursuant to Section 13.2(a), (b) or (d), or by Customer pursuant to Section 13.2(f). Notwithstanding the foregoing, if this Agreement is terminated by Customer pursuant to Section 13.2(a), (b) or (e) during the Initial Term, the obligations under Section 3.5.1 shall survive such termination for a period of five (5) years after the effective date of such termination.

13.3.2 Termination or expiration of this Agreement or any Statement of Work shall not release either Party from any liability, right of action or other obligation which has arisen prior to such termination or expiration, including Agilent's obligation to deliver to Customer such quantity of Product under any Purchase Order accepted by Agilent prior to the effective date of termination or expiration, and Customer's obligation to pay Agilent the amount set forth in such Purchase Order. In the event of termination of any Statement of Work under Section 13.2, Customer shall only pay Agilent for all work performed under such Statement of Work prior to the termination date. In the event a Supply Failure is not cured and this Agreement is terminated under Section 13.2(e), Agilent will refund to Customer any amounts paid for the Product that was not delivered.

13.3.3 In the event of expiration or termination of this Agreement for any reason other than (i) termination by Agilent pursuant to Section 13.2(a) for Customer's failure to pay amounts owed hereunder or (ii) termination by Agilent pursuant to Section 13.2(b), Customer shall be entitled, subject to Section 4.3.2, to place Purchase Orders in accordance with

the lead times set forth in Section 4.3.1 in order to build inventory of the Product. Customer shall be entitled to place such Purchase Orders for a period of [**] months following the date of notification for expiration or termination. Customer shall, subject to the terms of this Agreement, including the procedures set forth in Article 8, take delivery of all Product ordered under this Section 13.3.3 within [**] months following the date of such notification for expiration or termination. All Purchase Orders placed by Customer under this Section 13.3.3 shall be subject to the terms and conditions of this Agreement, notwithstanding any prior termination or expiration thereof.

13.4 Surviving Provisions. Notwithstanding any expiration or termination of this Agreement, the following provisions shall survive: 3.3.3, 3.3.4, 4.7, 5.8.4, 6.2, 6.3, 7, 8.2.3, 8.2.6, 9, 10, 11, 12, 13, 14, 15 and 16.

14. CONFIDENTIAL INFORMATION

14.1 Proprietary Information. The terms and conditions of the Confidentiality Agreement dated March 22, 2011, by and between Customer and Agilent, as amended ("Confidentiality Agreement"), are attached hereto as Exhibit G and incorporated herein by this reference. Capitalized terms used in this Article 14 and not defined in this Agreement shall have the meanings ascribed to them in the Confidentiality Agreement. The terms and conditions of the Confidentiality Agreement shall apply to information exchanged under this Agreement; provided that:

14.1.1 with respect to information exchanged pursuant to this Agreement, the "Purposes" as defined in Section 1 of the Confidentiality Agreement shall be amended to mean the conduct of activities and exercise of rights granted pursuant to this Agreement;

14.1.2 notwithstanding Section 3 of the Confidentiality Agreement, the Confidentiality Agreement shall apply to all Proprietary Information disclosed between the Parties pursuant to the Confidentiality Agreement and/or this Agreement from March 22, 2011 until the end of the Term plus any additional time needed to complete Agilent's obligations under Section 13.3.3;

14.1.3 notwithstanding Section 8(c) of the Confidentiality Agreement, the Confidentiality Agreement, as it applies to information exchanged under this Agreement, shall be construed and interpreted in accordance with the laws of the State of New York as provided in Section 16(k);

14.1.4 notwithstanding Section 8(e) of the Confidentiality Agreement, the obligations of confidentiality and non-use under the Confidentiality Agreement shall apply until the [**] anniversary of the expiration or termination of this Agreement;

14.1.5 the restrictions on disclosure and use set forth in the Confidentiality Agreement shall not apply to the disclosure of this Agreement or the disclosure of Proprietary Information to governmental authorities (i) that is required by applicable law or regulation to be submitted by Customer in connection with the issuance or maintenance of marketing approvals for the Product or Finished Product; (ii) that is submitted by either Party to comply with requests for information from any governmental authority; or (iii) that is submitted by either Party to comply with applicable governmental regulations (including the rules and regulations of any stock exchange); provided that, (x) to the extent permitted by applicable law, Customer or Agilent, as the case may be, will give reasonable advance notice to the other Party of such disclosure requirement in order to allow the other Party the opportunity to seek appropriate legal relief to prevent or limit disclosure of its Proprietary Information; (y) reasonable measures shall have been taken by the Party seeking to disclose the other Party's Proprietary Information to ensure confidential treatment of such Proprietary Information; and (z) any disclosure shall be

limited to such portion of the other Party's Proprietary Information that is legally required to be disclosed.

14.1.6 notwithstanding anything to the contrary in the Confidentiality Agreement, but subject to Section 14.1.1, in the event that the Recipient wishes to disclose this Agreement or the Disclosing Party's Proprietary Information to actual or potential investors, lenders, acquirers, merger partners, or professional advisors who have a reasonable need to know such information, the Recipient shall provide prior written notice thereof to the Disclosing Party, and the Parties shall promptly meet (in person or via telephone) and confer prior to any such disclosure for the purpose of avoiding any inappropriate disclosure of the Disclosing Party's Proprietary Information. Following such meeting, if the Disclosing Party has provided its express prior written consent to such disclosure, which consent shall not be unreasonably withheld or delayed, the Recipient may disclose the Disclosing Party's Proprietary Information to such Third Party; provided that (i) the Recipient shall only disclose such amount of the Disclosing Party's Proprietary Information as is reasonably necessary; and (ii) the Recipient has entered into a confidentiality agreement, with terms of confidentiality at least as restrictive as the terms and conditions set forth in this Article 14 and the Confidentiality Agreement, with such Third Party (other than attorneys and accountants of Recipient who are bound to confidentiality under applicable ethical and professional rules) before disclosing any of the Disclosing Party's Proprietary Information. In the event that the Disclosing Party has not consented to such disclosure, the Recipient may engage an independent Third Party consultant reasonably acceptable to the Disclosing Party and subject to confidentiality obligations at least as restrictive as the terms and conditions set forth in this Article 14 and the Confidentiality Agreement, to evaluate the Parties' rights and obligations hereunder and such independent Third Party consultant shall be permitted to disclose to such Third Party confirmation solely regarding the adequacy of such rights and obligations and the performance hereunder. For the avoidance of doubt, the independent Third Party consultant shall not be permitted to disclose any Proprietary Information of the Disclosing Party to any Third Party. The Parties agree that the process set forth in this Section 14.1.6 shall not apply to Customer's use or exercise of the license rights under Sections 9.3.2.1 or 9.3.2.2, provided that Customer complies with the provisions of the applicable Sections 9.3.2.1 or 9.3.2.2.

14.2 Remedies. Each Party shall be entitled, in addition to any other right or remedy it may have, at law, in equity or under this Agreement, to seek temporary, preliminary and permanent injunctions, enjoining or restraining the other Party and its Affiliates from any violation or threatened violation of this Article 14.

15. LIMITATION OF LIABILITY

15.1 EXCEPT IN CONNECTION WITH (A) A BREACH OF ARTICLE 14; (B) THIRD PARTY CLAIMS UNDER ARTICLE 10; AND (C) DAMAGES ARISING FROM GROSS NEGLIGENCE OR WILLFUL MISCONDUCT, IN NO EVENT WILL EITHER PARTY OR ITS AFFILIATES, SUBCONTRACTORS OR SUPPLIERS BE LIABLE FOR ANY INDIRECT, INCIDENTAL, SPECIAL OR CONSEQUENTIAL DAMAGES OR LOST PROFITS, LOST OPPORTUNITY OR LOST GOODWILL, ARISING OUT OF THIS AGREEMENT, REGARDLESS OF WHETHER SUCH DAMAGES ARE BASED ON TORT, WARRANTY, CONTRACT OR ANY OTHER LEGAL THEORY, EVEN IF ADVISED OF THE POSSIBILITY OF SUCH DAMAGES. THIS EXCLUSION IS INDEPENDENT OF ANY OTHER REMEDY SET FORTH IN THIS AGREEMENT. NOTWITHSTANDING THE FOREGOING, AGILENT SHALL PAY ALL SETTLEMENT AMOUNTS AND COURT AWARDED DAMAGES IN ACCORDANCE WITH SECTION 9.8, PROVIDED THAT THE

PARTIES HAVE COMPLIED WITH THE INDEMNIFICATION PROCESS SET FORTH IN SECTION 10.3.

15.2 EXCEPT IN CONNECTION WITH (A) A BREACH OF ARTICLE 14; (B) THIRD PARTY CLAIMS UNDER SECTION 9.8; (C) DAMAGES CAUSED BY AGILENT'S OR ITS OFFICERS', DIRECTORS', EMPLOYEES', AFFILIATES', SUBCONTRACTORS' OR SUPPLIERS' GROSS NEGLIGENCE OR WILLFUL MISCONDUCT; (D) AGILENT'S REIMBURSEMENT OBLIGATIONS UNDER SECTIONS 3.3.3 AND 3.3.4; (E) THE REMEDIES SET FORTH IN SECTION 7.5; (F) RECALL COSTS UNDER SECTION 8.2.6; AND (G) OBLIGATIONS OF AGILENT TO REFUND OR CREDIT AMOUNTS TO

CUSTOMER UNDER SECTIONS 4.3.2, 4.7.1, 5.4.1.2, 5.8.1, 7.5 AND 13.3.2, TO THE FULLEST EXTENT PERMITTED BY LAW, AGILENT'S AGGREGATE LIABILITY TO CUSTOMER DURING ANY GIVEN 12-MONTH PERIOD FOR CLAIMS FOR DAMAGES UNDER THIS AGREEMENT SHALL NOT EXCEED THE SUM OF THE TOTAL NUMBER OF BATCHES MANUFACTURED BY AGILENT FOR CUSTOMER UNDER THIS AGREEMENT DURING THE PREVIOUS TWELVE (12) MONTH PERIOD MULTIPLIED BY FIVE HUNDRED THOUSAND DOLLARS ($500,000.00).

16. **MISCELLANEOUS**

a. Notices. All notices required or permitted to be given under this Agreement must be in writing and delivered to the other Party as set forth below. Notices are validly given upon the earlier of confirmed receipt by the receiving Party or three (3) days after dispatch by a reputable courier or certified mail, return receipt requested. Either Party may change its designated contact and address for purposes of notice by giving notice to the other Party in accordance with these provisions.

Agilent Technologies, Inc. 5555 Airport Blvd. Suite 100 Boulder, CO 80301 Attn: General Manager	Ophthotech Corporation One University Square Drive Suite 280 Princeton, NJ 08540 Attn: Chief Business Officer

With a copy to:

Agilent Technologies, Inc. 5301 Stevens Creek Blvd. Santa Clara, CA 95051 Attn: General Counsel	Ophthotech Corporation One Penn Plaza, Suite 1924 New York, NY 10119 Attn: General Counsel

b. Escalated Dispute Resolution. In the event that the Parties are unable to agree upon any disputes arising under this Agreement, including without limitation any claims of breach that may give rise to termination, the Parties' relationship managers agree to negotiate in good faith to resolve any such disputes. If such negotiations and meetings do not resolve the dispute within [**] days after notice of the dispute, then a senior executive from each Party will meet face to face within [**] days or as mutually agreed between them to attempt to resolve such dispute. If the dispute is not resolved to the satisfaction of these executives within [**] days, then either Party may, subject to the provisions of this Agreement, pursue all available legal remedies. Notwithstanding the foregoing, either Party may seek injunctive relief with respect to any disputed matter without following the dispute resolution procedure set forth above.

c. Exhibits. The following Exhibits attached to this Agreement are deemed a part of this Agreement and incorporated by reference herein:

EXHIBIT A	PRODUCT
EXHIBIT B	[RESERVED]
EXHIBIT C	QUALITY AGREEMENT
EXHIBIT D	MEMORANDUM OF INSURANCE
EXHIBIT E	[RESERVED]
EXHIBIT F	LIST OF PATENTS
EXHIBIT G	CONFIDENTIALITY AGREEMENT
EXHIBIT H	PROCESS OVERVIEW
EXHIBIT I	[RESERVED]
EXHIBIT J	PRODUCT PRICING
EXHIBIT K	RISK MANAGEMENT PLAN
EXHIBIT L	[RESERVED]
EXHIBIT M	SPECIFICATION
EXHIBIT N	COMMERCIAL READINESS PLAN
EXHIBIT O	CRITICAL RAW MATERIALS

d. Independent Contractors. The relationship of the Parties established under this Agreement is that of independent contractors and neither Party is a partner, employee, agent or joint venturer of or with the other.

e. Assignment. Except as otherwise provided in this Section 16(e), neither this Agreement nor any part hereof may be assigned or transferred by either Party, whether by operation of law or otherwise, without the other Party's prior written consent. Either Party shall have the right to assign this Agreement, without the other Party's consent, in the event of a sale or transfer of the business as to which this Agreement relates,

whether such sale or transfer occurs by merger, reorganization, asset and/or stock purchase, or by any other means, provided that the assignee agrees in writing to assume all of the assignor's obligations under this Agreement. The assigning Party shall notify the non-assigning Party in writing as soon as possible of any sale or transfer of its business. Any assignment or purported assignment in violation hereof shall be void. This Agreement will be binding upon and inure to the benefit of the Parties and their permitted successors and assigns.

f. Headings; Construction; Interpretation. Headings used herein are for convenience only and shall not in any way affect the construction of or be taken into consideration in interpreting this Agreement. The terms of this Agreement represent the results of negotiations between the Parties and their representatives, each of which has been represented by counsel of its own choosing, and neither of which has acted under duress or compulsion, whether legal, economic or otherwise. Accordingly, the terms of this Agreement shall be interpreted and construed in accordance with their usual and customary meanings, and each of the Parties hereto hereby waives the application in connection with the interpretation and construction of this Agreement of any rule of law to the effect that ambiguous or conflicting terms or provisions contained in this Agreement shall be interpreted or construed against the Party whose attorney prepared the executed draft or any earlier draft of this Agreement. Any reference in this Agreement to an Article, Section, subsection,

paragraph, clause or Exhibit shall be deemed to be a reference to any Article, Section, subsection, paragraph, clause or Exhibit, of or to, as the case may be, this Agreement. Except where the context otherwise requires, (i) any definition of or reference to any agreement, instrument or other document refers to such agreement, instrument or other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein or therein); (ii) any reference to any law refers to such law as from time to time enacted, repealed or amended; (iii) the words "herein," "hereof" and "hereunder," and words of similar import, refer to this Agreement in its entirety and not to any particular provision hereof; (iv) the words "include," "includes," "including," "exclude," "excludes," and "excluding," shall be deemed to be followed by the phrase "but not limited to," "without limitation" or words of similar import; and (v) all references in this Agreement to "days" will, unless otherwise specified herein, mean calendar days.

g. No Third Party Beneficiaries. No provisions of this Agreement are intended to confer or give, or will be construed to confer or give, to any person or entity other than Agilent and Customer any rights, remedies or other benefits under or by reason of this Agreement.

h. Severability. If any provision of this Agreement is determined by a court of competent jurisdiction to be invalid or unenforceable in any respect, such determination will not impair or affect the validity, legality or enforceability of the remaining provisions hereof, and each provision is hereby declared to be separate, severable and distinct. To the extent that any such provision is found to be invalid, illegal or unenforceable, the Parties will negotiate in good faith to substitute for such provision, to the extent possible, a new provision that most nearly effects the Parties' original intent in entering into this Agreement or to provide an equitable adjustment in the event no such provision can be added. The other provisions of this Agreement will remain in full force and effect.

i. Hierarchy Of Documents. Unless otherwise specifically agreed to by the Parties, in the event of any conflict between the terms of this Agreement and its Exhibits, and a Purchase Order, the order of precedence is as follows: (i) the terms of this Agreement; (ii) its Exhibits; and (iii) the terms of the accepted Purchase Order. The Parties acknowledge and agree that the pre-printed provisions on any Purchase Order will be deemed deleted and of no effect whatsoever.

j. Entire Agreement. This Agreement together with any Purchase Orders and Statements of Work constitutes the entire agreement between the Parties with respect to the subject matter hereof and supersedes all prior communications, representations or agreements, whether oral or written. No modifications, amendments, or waiver of any term, condition or provision of this Agreement or any Purchase Order or Statement of Work will be binding on either Party unless in writing and signed by an authorized representative of each Party.

k. Governing Law. This Agreement is made under and will be construed in accordance with the laws of New York without giving effect to that jurisdiction's choice of law rules. The United Nations Convention on Contracts for the International Sale of Goods will not apply to this Agreement or to transactions processed under this Agreement.

l. Announcements. Neither Party shall make any public disclosure relating to this Agreement without the prior consent of the other Party, except as otherwise permitted under Article 14.

m. Subcontractors. Agilent shall not, without the prior written approval of Customer, subcontract or delegate its obligations under this Agreement or a Statement of Work. Agilent shall be responsible for ensuring that any approved subcontractor, including Agilent's Affiliates, shall be subject to Agilent's obligations contained in this Agreement or any applicable Statement of Work. Agilent shall be responsible for any breach of this Agreement by any subcontractors subject to Article 15.

n. Counterparts. This Agreement or any Purchase Order or a Statement of Work may be executed in counterparts each of which, when executed and delivered, shall be original, but all such

counterparts shall constitute one and the same document. The Parties agree that signatures transmitted via portable document format (PDF) shall be deemed originals until originals replace such copies.

APPROVED AND AGREED TO:

AGILENT TECHNOLOGIES, INC.	OPHTHOTECH CORPORATION
By: /s/ Nelson Thune	By: /s/ Michael G. Atieh
Typed Name: Nelson Thune	Typed Name: Michael G. Atieh
Title: General Manager	Title: EVP, Chief Financial and Business Officer
Date: 03 September 2015	Date: September 3, 2015

EXHIBIT A

PRODUCT

Fovista (pegpleranib sodium, [**])

[**].

EXHIBIT B

[RESERVED]

EXHIBIT C

QUALITY AGREEMENT

OPHTHOTECH

Agilent Technologies

Quality Agreement

Use as an exhibit to service and supply agreement

Customer:	Ophthotech Corporation One Penn Plaza New York, NY 10119
Supplier:	Agilent Technologies, Inc. 5555 Airport Boulevard Boulder, Colorado 80301
Product(s):	E10030 (PEGylated oligonucleotide) CSN API
Services:	Laboratory Testing · Manufacturing Support and Finished API Release and Stability (CTX) · Finished Drug Product Release and Stability Testing (CTL)
Version:	00

Approvals:

/s/ Nelson Thune	11/4/13
Agilent Technologies General Manager	Date
/s/ illegible	06 Nov 13

Agilent Manufacturing	Date
/s/ Celeste O'Connor	08 Nov 13
Agilent Technologies Quality Assurance	Date
/s/ Douglas Brooks	06-Nov-2013
Ophthotech Manufacturing	Date
/s/ Douglas Kollmorgen	05-Nov-2013
Ophthotech Quality Assurance	Date
Other	Date

Sections: (aligned to May 2013 FDA DRAFT Guidance)

1. Purpose and Scope

1.1. This Quality Agreement ("Agreement") for the clinical and commercial stage manufacturing of Active Pharmaceutical Ingredients (API), and testing of API and Drug Product under Good Manufacturing Practice (GMP) is being executed by and between:

- **Ophthotech Corporation** hereafter referred to as "Ophthotech".
- And, **Agilent Technologies, Inc**. hereafter referred to as "Agilent".

1.2. Agilent and Ophthotech are parties to Manufacturing and Supply Agreements as set forth in Attachment 2 (the "Supply Agreements"), pursuant to which Agilent is to supply Ophthotech API and perform certain Manufacturing and Laboratory Services with respect to API and Drug Product. This Agreement will become effective as of the date of the last signatory herein.

1.3. Agilent shall operate in accordance with GMP for manufacturing of GMP APIs and the performance of Manufacturing and Laboratory Services and such other applicable regulatory requirements as described in this Agreement and the applicable Supply Agreement. The purpose of this Agreement is to clearly define the roles and responsibilities of Agilent and Ophthotech with regard to quality and GMP compliance issues concerning the production of GMP API molecules and the performance of certain Manufacturing and Laboratory Services, including testing of API and Drug Product.

1.4. The scope of this Agreement includes GMP and quality compliance associated with the clinical and commercial stage manufacturing of GMP API molecules and the performance of certain Manufacturing and Laboratory Services, including testing of API and Drug Product.

2. Terms

2.1. **(3) Definitions**

2.1.1. **Analytical (Test) Methods** — Methods used for analytical testing, including Standard Test Methods and Compendial Methods.

2.1.2. **Active Pharmaceutical Ingredient (API),—**Any substance or mixture of substances intended to be used in the manufacture of a drug (medicinal) product and that, when used in the production of a drug, becomes an active ingredient of the Drug Product as defined in ICH Q 7. Such substances are intended to furnish pharmacological activity or other direct effect on the diagnosis, cure mitigation, treatment, or prevention of disease or to affect the structure and function of the body.

2.1.3. **Approval —** The term "Approval" is defined as concurrence between Ophthotech and Agilent, as evidenced in writing and signed by both companies' Authorized Quality Representatives. In certain cases, Approvals may be obtained electronically or verbally, followed by a written confirmation.

2.1.4. **Authorized Quality Assurance Representative —** An individual named within this Agreement with the authority to resolve any disputes or conflicts relating to this Agreement in a timely and equitable manner and in compliance with all applicable quality and regulatory requirements.

2.1.5. **Batch —** A specific quantity of material produced in a process or series of processes that is expected to be homogeneous, within specified limits, and that is produced by Agilent in the same cycle of manufacture as defined by the applicable batch record and which shall be packaged and released with a single release and lot number.

2.1.6. **Batch Packet —** Relevant documentation to be transferred by Agilent to Ophthotech to support the release of a Batch. This packet includes, but is not limited to, copies of:

2.1.6.1. Executed Batch Records
2.1.6.2. all Deviations, including proposed CAPA's where appropriate, associated with the manufactured API
2.1.6.3. OOS investigations associated with analysis of the API
2.1.6.4. In-process results
2.1.6.5. Certificate of Analysis (COA)
2.1.6.6. Certificate of Compliance (COC)
2.1.6.7. QA disposition

2.1.7. **Batch Production Record** — An accurate reproduction of a Master Batch Record used as instruction for and documentation of production activities.

2.1.8. **CAPA**-Corrective action, preventative action.

2.1.9. **Certificate of Analysis (COA)** — A document, signed by an authorized representative of Agilent, describing (i) the Specification; (ii) the testing methods applied to the API in order to verify compliance with the Specification, and (iii) the results thereof.

2.1.10. **Certificate of Compliance (COC)** — A document, signed by an authorized quality assurance representative of Agilent, attesting that a particular Batch was manufactured in accordance with cGMP, and the Specification. The Certificate of Compliance may be included

within the Certificate of Analysis, or separately, if required by Ophthotech.

2.1.11. **cGMP or GMP** — Current Good Manufacturing Practices pursuant to (i) the U.S. Federal Food, Drug, and Cosmetic Act as amended (21 USC 301 et seq.), (ii) relevant U.S. regulations found in Title 21 of the U.S. Code of Federal Regulations (including but not limited to Parts 11, 210, 211, 600 and 610), (iii) Commission Directive 2003/94/EEC of 08 October 2003, (iv) the EC Guide to Good Manufacturing Practice for Medicinal Licensed Products, including respective guidance documents; (v) any comparable laws, rules or regulations of other jurisdictions as mutually agreed to by Ophthotech and Agilent, as each may be amended from time to time; and (vi) the relevant current International Conference on Harmonization (ICH) guidance documents,

including the ICH Guidance Q7 Good Manufacturing Practice Guide for Active Pharmaceutical Ingredients and ICH Guidance Q11

2.1.12. **Controlled Documents -** Paper or electronic documents that are part of the quality system and contain data/information required by cGMPs. These documents may also be referred to as GMP documents. Such documents must be initiated and revised through document control and/or change control procedures. Examples of controlled documents are: SOPs, analytical test methods, specifications, batch records, validation protocols, forms, etc.

2.1.13. **Critical Raw Material** — A material (starting materials, reagents and solvents) whose intended use is in the production of intermediates or APIs and whose attributes must be controlled within predetermined criteria to ensure that the API meets its specification.

2.1.14. **Deviation** — A departure from written standard where any of the following is true: requires investigation and root cause analysis, has the potential for product, process, or equipment impact, requires CAPA for prevention of future recurrence, requires a Change Control, or presents a potential non-conformance with a regulatory filing, specification, or validated parameter, or requires customer notification.

2.1.15. **Disposition** — The action of assigning a status of release, quarantine, reject etc. to a material.

2.1.16. **Drug Product** — The dosage form in the final immediate packaging intended for human clinical or commercial use.

2.1.17. **Executed Batch Record** — A completed Batch Production Record.

2.1.18. **Intermediate**- A material produced during steps of the processing of an API that undergoes further molecular change or purification before it becomes an API. Intermediates may or may not be isolated.

2.1.19. **Master Batch Record** (**MBR**) — The document that defines the manufacturing methods, materials, and other procedures, directions and controls associated with the manufacture and testing of the API.

2.1.20. **Out-of-Specification (OOS)** — A result derived from testing that is valid but does not comply with the established specification. In this case, "result" is defined as the final reportable value as determined according to the test method. Such a reportable value may be comprised of multiple individual determinations (i.e., replicates) as per the test method. Only reportable values are compared to specifications; therefore only a reportable value may constitute an OOS.

2.1.21. **Product** — Any a) API, or (b) Drug Product comprised of API, or (c) intermediate(s) of (a) or (b), in each case as specified in the applicable Scope.

2.1.22. **Qualified Supplier —** A supplier who has met minimum approval standards and been qualified by Agilent, to provide required items or services that may impact API quality.

2.1.23. **Raw Material** — A general term used to denote starting materials, reagents, and solvents intended for use in the production of intermediates or APIs.

2.1.24. **Regulatory Authority**-means any competent authority of the US, Europe, Japan, or other regulated region which regulates the manufacture of the API in accordance with ICH guidelines.

2.1.25. **Significant Change —** Any change that: has the potential to (a) impact the quality, safety, identity, strength, efficacy, potency or purity of the API; (b) impact the regulatory commitments and/or reporting requirements of the API; (c) require re-qualification or re-validation of the process, methods, reference standards approved by Ophthotech; and/or (d) result in changing or modifying Ophthotech's approved Specifications, test methods or any document approved by Ophthotech.

2.1.26. **Significant Deviation** — A deviation that has been shown to adversely impact final API, stability study, drug product or a critical raw material.

2.1.27. **Specification** — The Specification for the Product as set forth in the Statement of Work, which Specification may be amended from time to time in accordance with this agreement.

2.1.28. **Subcontractor -** Any manufacturer, packager, or other API support service provider who performs processing, packaging, or testing of an API or any intermediate step of manufacture, or other API support service on behalf of Agilent.

2.2. (21) Audit and Inspections

2.2.1. Agilent agrees to allow the FDA and any other Regulatory Authority to conduct any inspection related to the manufacture of the API which the FDA or such Regulatory Authority requires and Agilent agrees to reasonably cooperate with the FDA or such Regulatory Authority in connection with such inspection. Agilent agrees to promptly notify Ophthotech of any inspections or actions by a Regulatory Authority which could potentially impact the production or distribution of the GMP API; provided that Agilent shall provide notice to Ophthotech of any such inspection or action that relates to the API or Product testing within [**] hours. Ophthotech may be present during any regulatory inspections involving their Product. Agilent agrees to provide

Ophthotech (i) copies of any report issued and notice of any regulatory actions resulting from such inspections within [**] business days of any written action from such Regulatory Authority and (ii) within [**] days after Agilent's receipt of such regulatory action, a plan to make corrective actions to remedy such regulatory action (which plan Agilent shall promptly implement and diligently pursue).

2.2.2. Ophthotech reserves the right to conduct compliance audits of Agilent's records and relevant areas of the Agilent facility that are involved in the production, testing, or storage of the API and Intermediates. Agilent requires a minimum of [**] business day notice for compliance audits. During audits, Agilent shall provide Ophthotech with all relevant documentation for the sole purpose of assuring API quality and compliance with agreed-upon manufacturing procedures.

2.2.3. Ophthotech is entitled to one routine on-site GMP audit per [**] period provided active manufacturing occurs during this period. A request for audit due to a specific issue ('for-cause' audit) may be conducted at any time with a minimum of a [**] business day notice and must be focused only on the subject of the 'for-cause' audit.

2.2.4. During an audit by Ophthotech, any non-conformances will be noted and documented in a report issued by Ophthotech within ([**] business days. Agilent will formally respond in writing within [**] business days following receipt of the report [**].

2.2.5. Ophthotech reserves the right, at Ophthotech's expense, to conduct PAI readiness and mock audit exercises at Agilent.

2.3. (2) Roles and Communication

2.3.1. Ophthotech and Agilent will each appoint a Primary Contact for communications between the two parties and who will jointly be responsible for the coordination and management of the project, including communication of quality and regulatory matters pursuant to this Agreement.

2.3.2. Both primary contacts will be included on all communications between Agilent and Ophthotech. For verbal communications regarding quality and regulatory matters, the initiating party will summarize the discussion in a written record, which will then be distributed by the respective primary contact.

2.3.3. Ophthotech and Agilent will each appoint an Authorized Quality Assurance (QA) Representative who will serve as the primary contact for quality related notifications between the two parties.

2.3.4. Responsible personnel are identified in Attachment 23.1. Either party may change its Project Manager or Authorized Quality Assurance Representative by providing the other party written notice and Attachment 23.1 shall be updated to reflect any such change(s).

2.4. (19) Subcontracting

2.4.1. Agilent shall use approved subcontractors according to internal procedures. Agilent will not subcontract any activities related to the GMP manufacturing or testing using non-approved subcontractors of API without prior approval of Ophthotech.

2.4.2. Agilent shall ensure that any quality impacting changes proposed at a subcontractor site utilized for Ophthotech testing are assessed and Ophthotech notified prior to the change being made.

2.5. (17) Complaints, Returns, and Recalls

2.5.1. Customer Complaints - Agilent agrees to maintain appropriate systems for documenting and investigating any customer complaints associated with the GMP API. Agilent will assist Ophthotech with investigational work to resolve the complaint. Agilent will respond within one business day for any serious or patient safety related API complaints. In the case of an emergency Agilent will rely upon site procedures to respond.

2.5.2. GMP API Returns — Agilent will maintain records for returned products including batch number, quantity and reason.

2.5.3. Recalls — Ophthotech will be responsible for the recall of any marketed Drug Products. Agilent is responsible for notifying

Ophthotech of any GMP API that is the subject of a recall. During a Product recall, withdrawal, or field correction, Agilent shall keep accurate drug accountability and distribution records, fully cooperate with Ophthotech in notifying customers, and conducting the necessary recall and investigational activities. Agilent shall provide assistance in the investigation reasonably required to determine the cause and extent of the problem necessitating the recall.

2.6. (22) Quality Agreement Revisions

2.6.1. Any revision to this Quality Agreement or any related attachments must be approved in advance by both parties. Revisions will be documented as written addendums that are attached to the original Quality Agreement. Each addendum will minimally be approved by the Primary Contact and the Quality Assurance representatives from both companies.

2.6.2. The Quality Agreement shall be updated and will minimally be approved by the Primary contact and the Quality Assurance representatives from both companies at the initiation of Ophthotech every [**] years.

3. Quality Dispute Resolution

3.1. In the event of a dispute as to whether (i) the Product has a Latent Defect or (ii) the Product was not in Good Condition at the time of delivery, the parties shall follow the dispute resolution procedure set forth the applicable Supply Agreement. In the unlikely event a dispute arises regarding any other issue affecting product quality that cannot be resolved, the parties agree to resolve the dispute in the following manner.

3.1.1. The parties agree to establish the basis of the dispute in writing within [**] days of the origin of the dispute.

3.1.2. The parties agree to the description content and detail of the dispute by signing and dating the dispute description document.

3.1.3. The document is escalated to the next higher comparable level in both organizations wherein parties from both companies are tasked with resolving the dispute as written.

3.1.4. In the event the escalation does not resolve the dispute the parties agree to follow the dispute resolution procedure set forth for Escalated Dispute Resolutionin the applicable Supply Agreement.

4. Responsibilities, including communication mechanisms and contacts

4.1. (4) General Responsibilities

4.1.1. Agilent agrees to manufacture, test, and deliver the GMP API in accordance with cGMP and other applicable compliance standards.

4.1.2. Agilent agrees to test and perform stability studies on either API or Drug Product as denoted in applicable Statements of Work.

4.1.3. Agilent agrees to maintain and operate under a quality system consistent with US and EU cGMP, including maintaining standard operating procedures (“SOPs”), training and root cause analysis and corrective & preventive actions.

4.1.4. Agilent agrees to ensure that personnel involved in the manufacture, testing and disposition of the GMP API have the education, training and experience, or any combination thereof, to enable those persons to perform their assigned responsibilities. Training extends to the particular operations that the employee performs and to the applicable GMP’s as they relate to API and Drug Product and the employee’s functions. Training records shall be maintained by Agilent as required by GMP and made readily available for the personnel working on API and Drug Product. All training relative to a specific task will be completed prior to the initiation of the task. Training will be conducted with sufficient frequency to assure familiarity with requirements applicable to the position and function. Agilent will ensure that any necessary GMP or technical training has been performed and is documented.

4.2. Quality Unit Responsibilities

4.2.1. The quality unit shall have the responsibility and authority to approve or reject all components, drug product containers, closures, in-process materials, packaging material, labeling, and drug products, and the authority to review production records to assure that no errors have occurred or, if errors have occurred, that they have been fully investigated. The responsibilities and procedures applicable to the quality unit shall be in writing; and the written procedures shall be followed.

4.2.2. The quality unit shall be responsible to assure adequate testing facilities are available and utilized for the testing of raw materials, components, API containers, closures, packaging materials, in-process materials, and drug products.

4.2.3. The quality unit shall have the responsibility for approving or rejecting all procedures or specifications impacting on the identity, strength, quality, and purity of the drug product.

4.3. (12) Facilities and Equipment

4.3.1. Agilent will manufacture the Ophthotech API only at the Agilent facility located at 5555 Airport Blvd. Boulder, CO 80301. (“Facility”)

4.3.2. All critical measuring and monitoring devices used in processing equipment will be calibrated according to a pre-determined documented schedule. As appropriate, calibrations will be conducted using standards that are traceable to NIST or an appropriate, traceable standard.

4.3.3. All GMP manufacturing operations will occur in equipment and facilities that are fully qualified per the Agilent Validation Master Plan and are subject to formal maintenance, calibration, and cleaning procedures.

4.3.4. The facility will be maintained according to procedure to ensure a state of compliance and maintain a validated state relative to the manufacturing of GMP APIs and in the performance of Manufacturing and Laboratory Services.

4.3.5. Any proposed change in the facility that has the potential to impact the quality of the Ophthotech API will be communicated to Ophthotech prior to the change being made.

4.4. Materials Management

4.4.1. (8) Raw Materials

4.4.1.1. It is the responsibility of Agilent to handle procurement, delivery, inspection, testing and storage of raw materials (including components) that are used to produce the GMP API except as specified in 4.4.1.6. Materials will be tested and/ or examined against approved specifications.

4.4.1.2. Materials of animal origin will be certified BSE/TSE free as per Agilent internal procedures.

4.4.1.3. All Critical Raw Material suppliers will be qualified as appropriate to the stage of development and the regulatory status of the GMP API as per Agilent internal procedures. Agilent will select suppliers for non-critical raw materials and components in accordance with the use and after assessment by Agilent Quality.

4.4.1.4. The testing procedures for the Critical Raw Materials will be performed per compendial methods or other test methods developed by Agilent if a compendial testing is not available or applicable.

4.4.1.5. Agilent shall use only those suppliers of Critical Raw Materials that have been approved by Agilent. If Ophthotech requests a specific supplier that is not a current Agilent qualified supplier, Agilent and Ophthotech will work together to qualify that supplier.

4.4.1.6. Except as otherwise agreed to by the Parties in writing, if Ophthotech supplies material to Agilent for API manufacture, it is Ophthotech's responsibility to qualify that supplier and provide qualification documentation to Agilent, including BSE/TSE certification and such qualification and audit records as agreed to by the Parties.

4.4.1.7. Agilent will maintain a Supplier Qualification program that may be assessed by Ophthotech during a quality audit.

4.4.1.8. Agilent will maintain samples of Critical Raw Materials, API and finished Drug Product in accordance with ICHQ7. All materials shall be handled and stored in accordance with the approved specifications.

4.4.1.9. Under no circumstances shall any materials which may present a potential hazard to the raw materials utilized in API be stored in the Facility, or in proximity to the area where raw materials utilized in API are maintained. If such materials are stored in the Facility, the Parties must agree to their separation and segregation.

4.4.2. (18) Reprocessing and Reworking

4.4.2.1. If either Ophthotech or Agilent determines that reprocessing or reworking of the GMP API is necessary due to OOS, manufacturing deviation, unmet Specifications, or otherwise, the procedure will be documented and approved by Agilent Chemical Development, Agilent Manufacturing, Agilent QA and Ophthotech QA, provided that Agilent shall not reprocess or rework the GMP API without the prior written consent of Ophthotech.

4.5. Product Specific Terms

4.5.1. (10) Manufacturing

4.5.1.1. Master Batch Record (MBR) - GMP APIs will be manufactured in accordance with written MBRs that have been drafted by Agilent and approved by Ophthotech. MBRs will be reviewed and approved by the Agilent QA

department prior to use. Executed Batch Records will be reviewed and approved by the Agilent QA department prior to disposition to Ophthotech.

4.5.1.2. Waste Handling — Any waste generated by the process will be disposed of according to Agilent procedures and in a secure and legal manner which prevents unauthorized use and/or environmental compliance problems.

4.5.2. (11) Qualification and Validation

4.5.2.1. Agilent will be responsible for the qualification and validation of manufacturing and testing equipment and processes, as mutually defined by Agilent and Ophthotech.

4.5.2.2. Agilent will perform qualification and/or validation, when applicable, of any analytical test methods as required by Ophthotech. Agilent will be responsible for generating protocols to qualify/validate the test methods which will be reviewed and approved by both Agilent and Ophthotech, if required. Agilent will provide a final report to Ophthotech for method transfer, qualification, and/or validation.

4.5.2.3. Agilent will not make a Significant Change to any Ophthotech specific test method without prior approval from Ophthotech. Compendial updates to methods are acceptable and will not require Ophthotech pre-approval. Ophthotech will be notified of changes to generic methods (other than compendial methods) used for the Ophthotech process and copies provided on request.

4.5.2.4. Ophthotech is responsible for providing Agilent with sufficient quantities of an appropriately qualified API reference standard along with a reference standard qualification certificate or appropriately tested reference material. Agilent can also be requested to prepare an API reference standard as described in section 4.6.1.2

4.6. Laboratory Controls

4.6.1. (15) Reference Standards/ Materials

4.6.1.1. Any reference standards / materials that are supplied by Ophthotech or obtained from an official source will be stored and used in accordance with established Agilent procedures and any written instructions provided by Ophthotech.

4.6.1.2. Any reference standards/ materials produced in-house at Agilent for Ophthotech will be appropriately documented and tested to ensure appropriate characterization of the material.

4.6.1.3. (Copy of foregoing section) Ophthotech is responsible for providing Agilent with sufficient quantities of an appropriately qualified API reference standard along with a reference standard qualification certificate or appropriately tested reference material. Agilent can also be requested to prepare an API reference standard as described in section 4.6.1.2

4.6.2. (9) Specifications and Test Methods

4.6.2.1. Agilent will follow written quality system procedures for the identification, quarantine, handling, sampling, testing and approval or rejection of materials. Agilent will perform testing per established methods/procedures and review results against the Specifications. Changes to these methods and procedures will be consistent with the Change Management section of this Agreement. Deviations to the test methods and procedures and OOS results will be handled in a manner consistent with the Deviation and OOS sections of this agreement.

4.6.2.2. Critical Raw Materials — Agilent will make recommendations for any change in Critical Raw Material Specifications and test methods as necessary to assure quality and compliance. The establishment of formal Critical Raw Material Specifications and test methods will occur per Agilent's internal procedures.

4.6.2.3. In-Process — Ophthotech and Agilent will agree on in-process Specifications and test methods used during development. The establishment of in-process Specifications and test methods for validation and commercial manufacturing will occur per Agilent's internal procedures and shall be subject to approval by Ophthotech.

4.6.2.4. Analytical Data Reporting Requirements - Copies of all analytical QC raw data (including chromatograms) and reports generated by Agilent will be provided to Ophthotech with the Batch Packet for in-process and final API analysis following manufacture. Copies of data related to method transfer or validation will be available

for on-site review by Ophthotech and provided to Ophthotech as required.

4.6.3. (14) Samples / Reserve

4.6.3.1. Raw Materials — Agilent agrees to sample and retain sufficient amounts and stored under controlled conditions, of all materials used in processing and testing, except water, compressed gasses and any highly volatile compounds and compounds that are not stable. In addition to the above, it is the responsibility of Agilent to retain Critical Raw Material samples with appropriate labeling, storage and duration according to Agilent procedures.

4.6.3.2. In-Process — Agilent will retain in-process samples until the Batch has been approved for release or as requested by Ophthotech in writing.

4.6.3.3. Final GMP API - Agilent will obtain retain samples of the final GMP API in accordance as requested by Opthotech in writing, but at a minimum, in sufficient amount to comply with ICH Q7 guidance for API sample retains. These retention samples will be packaged and stored in accordance with ICH Q7 and the Agilent specification. Agilent will notify Ophthotech prior to disposing of retain samples as per Agilent internal procedures.

4.6.4. (16) Stability

4.6.4.1. Stability testing, both accelerated and long-term, will be conducted as contracted by Ophthotech. Ophthotech will be responsible for determining appropriate retest/expiry dates, storage conditions, and packaging materials.

4.6.4.2. Stability testing will be conducted under protocols written by Agilent and approved by Ophthotech and Agilent QA prior to commencement of the stability study.

4.6.4.3. Both parties agree to inform the other of the results of any stability testing for which they are responsible. This includes notification of any stability results that are deemed OOS or out-of-trend per established specifications and/ or Agilent internal procedures per section 5.2 of this agreement.

4.6.5. (13) Packaging, Labeling, Testing and Release of GMP API

4.6.5.1. The final packaging, labeling, and testing of each GMP API Batch will be conducted in accordance with written procedures, and with packaging and labeling requirements and test specifications provided by Ophthotech.

4.6.5.2. Each batch will be internally released by the Agilent QA department as per established internal procedures which will include a review of associated batch records and analytical data.

4.6.5.3. A Certificate of Analysis (COA) will be issued by Agilent for each Batch of API confirming that the API has been tested in accordance with the Specification using approved methods. The COA will contain results for all API analyses that have a Specification. Agilent will provide an Analytical Data Report Form for any additional analyses not listed on the Specification.

4.6.5.4. A Certificate of Compliance (COC) will be issued by Agilent for each Batch of API confirming that the API has been manufactured, packaged and tested in full compliance with GMP, ICH Q7 and local Regulatory requirements. The COC will attest to the accuracy of the manufacturing records and provide limited detail on the occurrence and resolution of deviations that may have occurred during Batch processing and testing. BSE/TSE certification for any animal derived raw materials, packaging components and processing aids is also provided.

4.6.5.5. Final release authority for shipment of each Batch of GMP API to Ophthotech will reside with Agilent's QA department.

4.7. Documentation

4.7.1. (7) Documentation

4.7.2. The Agilent Primary Contact will provide and receive all controlled documents to and from the Ophthotech Primary Contact.

4.7.3. Agilent will generate any internal Controlled Documents necessary to support GMP API production and will be responsible for the retention and storage of all Batch Packet documentation in a secure QA archive according to Agilent's record retention policy. Ophthotech shall be notified prior to destruction of any Controlled Documents supporting a batch production record and have the option of making

arrangements for continued retention or the return of such documents to Ophthotech.

4.7.4. A Certificate of Analysis (COA), Certificate of Compliance (COC), BSE/ TSE Certification, and Material Safety Data Sheet or Safety Data Sheet (MSDS/SDS) will be provided by Agilent with every GMP API shipment. Copies of completed Batch Packets documents will be provided to Ophthotech as defined in Section (2).

4.7.5. Controlled Documents specific to the manufacture of E10030 will be reviewed and approved by Ophthotech prior to Agilent making the documents effective.

4.8. (20) Regulatory Interactions and Submissions

4.8.1. Regulatory Contacts. Unless otherwise required by applicable law, Ophthotech will be solely responsible for all contacts and communications with any regulatory authorities with respect to matters relating to the API or any of the Manufacturing and Laboratory Services under a Statement of Work. Agilent will notify Ophthotech immediately, and in no event later than [**] days, after Agilent receives any contact or communication from any regulatory authority relating in any way to the API or Product testing or the Manufacturing and Laboratory Services under a Statement of Work and will provide Ophthotech with copies of any such communication within [**] of receipt of such communication by Agilent. Agilent will consult with Ophthotech regarding the response to any inquiry or observation from any regulatory authority relating in any way to the API or Product testing or the Manufacturing and Laboratory Services under a Statement of Work and will allow Ophthotech at Opthotech's discretion to participate in any further contacts or communications relating to such Services. Agilent will comply with all reasonable requests and take into consideration all comments by Ophthotech with respect to all contacts and communications with any regulatory authority relating in any way to the API the Manufacturing and Laboratory Services under a Statement of Work.

4.8.2. Submissions. Agilent will provide to Ophthotech at Ophthotech's expense, input, data and written content regarding the manufacturing and controls for the API as may be required for regulatory submissions. As the drug sponsor, it is the responsibility of Ophthotech to provide an appropriate template and specific content requests to Agilent.

5. Change Control and Revisions

5.1. (5) Change Management

5.1.1. Agilent will utilize a documented change control system as defined by internal procedures to control changes to raw materials, packaging materials, suppliers, equipment, manufacturing procedures, material specifications, facilities, sampling procedures, analytical methods, a process or method validated state or standard operating procedures.

5.1.2. Any Significant Change or other change proposed by Agilent to the MBR, Facility, Utilities, Equipment, Specifications and/or SOPs, including but not limited to the manufacturing process, materials and/or analytical methods which may affect the quality or performance of the API over its shelf-life, acceptance criteria not met for post-validation batches or affect commitments made in regulatory filings (a) shall be made only as permissible under the applicable Supply Agreement and this Quality Agreement; and (b) must be approved by Ophthotech, in writing, prior to implementation for routine production or release of any affected batch.

5.1.3. Ophthotech will use reasonable efforts to respond to any written request for change from Agilent within [**] business days. If the change request is part of an initiated manufacturing campaign, Ophthotech will use reasonable efforts to respond within [**]. No Significant Change shall be implemented by Agilent without the prior written approval of Ophthotech.

5.1.4. Ophthotech initiated requests for changes shall be communicated to Agilent's Quality management in writing using Ophthotech's change control documentation. Agilent will use reasonable efforts to respond to any written request for change from Ophthotech within [**] business days. Such Ophthotech requested changes shall, upon mutual agreement of the Parties, be implemented by Agilent using Agilent's current approved change management procedures. Agilent shall not unreasonably withhold, condition or delay its approval of any such change and any such changes required in order to comply with applicable laws, rules or regulations shall not require such approval, without reasonable justification.

5.2. (6) Deviation Handling and OOS Investigations

5.2.1. Any deviations from approved manufacturing, testing, or storage procedures that occur in the course of batch production will be managed according to Agilent's internal procedures for deviation handling, and the extent of investigation will be determined by Agilent and shall be commensurate with the severity of the deviation and the potential API quality impact. Agilent must notify Ophthotech within [**] business days from the observation of Deviations ([**] with respect to Significant Deviations). All deviations will be investigated and fully documented by Agilent. This documentation will be retained

as part of the batch documentation for the batch affected. When deemed necessary, Ophthotech reserves the right to request additional or more in-depth investigation of the Deviation by Agilent. Ophthotech prior approval shall be obtained in writing for any planned Significant Deviation. Agilent shall not release any Batch which includes a Deviation.

5.2.2. All deviations will be assessed for potential API quality impact according to Agilent internal procedures and will be fully documented by Agilent. Investigations will include appropriate justification, scientific rationale and supporting data.

5.2.3. Agilent will notify Ophthotech of confirmed OOS results within [**] business day of notification to Agilent QA that the OOS has occurred. Agilent will perform the OOS investigation as per Agilent internal procedures. Agilent shall provide Ophthotech written notice of any changes to its SOPs or other internal procedures relating to OOS investigations and shall, upon Ophthotech's request, make such changed procedures available for Ophthotech review

6. (23) Attachments

6.1. Contact Information

6.1.1 **ATTACHMENT 1 — CONTACT INFORMATION**

Ophthotech Mailing Address:

Ophthotech Corporation
One Penn Plaza, 35th Floor
New York, New York 10119

Ophthotech Contact Information

Name	Title	Phone	E-Mail
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]

Agilent Technologies Mailing Address

Agilent Technologies, Incorporated
5555 Airport Blvd.
Boulder, CO 80301

Agilent Technologies Contact Information

Name	Title	Phone	E-Mail
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]

6.2. Attachment 2

6.2.1. Manufacturing and Supply Agreements to be added as they are agreed

EXHIBIT D

MEMORANDUM OF INSURANCE

The following MOI will be updated to add Ophthotech as an "additional insured" and provide a waiver of subrogation in favor of Ophthotech.

Memorandum of Insurance (MOI)

MEMORANDUM OF INSURANCE **DATE**
31-Aug-2015

This Memorandum is issued as a matter of information only to authorized viewers for their internal use only and confers no rights upon any viewer of this Memorandum. This Memorandum does not amend, extend or alter the coverage described below. This Memorandum may only be copied, printed and distributed within an authorized viewer and may only be used and viewed by an authorized viewer for its internal use. Any other use, duplication or distribution of this Memorandum without the consent of [**] is prohibited. "Authorized viewer" shall mean an entity or person which is authorized by the insured named herein to access this Memorandum via [**]. The information contained herein is as of the date referred to above. Marsh shall be under no obligation to update such information.

PRODUCER
[**]

COMPANIES AFFORDING COVERAGE

Co. **A** [**]

INSURED
Agilent Technologies, Inc.
5301 Stevens Creek Blvd.
M/S 1B-08, Santa Clara
California 95051
United States

Co. **B**

Co. **C**

Co. **D**

COVERAGES

THE POLICIES OF INSURANCE LISTED BELOW HAVE BEEN ISSUED TO THE INSURED NAMED ABOVE FOR THE POLICY PERIOD INDICATED. NOTWITHSTANDING ANY REQUIREMENT, TERM OR CONDITION OF ANY CONTRACT OR OTHER DOCUMENT WITH RESPECT TO WHICH THIS MEMORANDUM MAY BE ISSUED OR MAY PERTAIN, THE INSURANCE AFFORDED BY THE POLICIES DESCRIBED HEREIN IS SUBJECT TO ALL THE TERMS, EXCLUSIONS AND CONDITIONS OF SUCH POLICIES. LIMITS SHOWN MAY HAVE BEEN REDUCED BY PAID CLAIMS

CO LTR	TYPE OF INSURANCE	POLICY NUMBER	POLICY EFFECTIVE DATE	POLICY EXPIRATION DATE	LIMITS LIMITS IN USD UNLESS OTHERWISE INDICATED	
A	**GENERAL LIABILITY** Commercial General Liability Occurrence	[**]	[**]	[**]	GENERAL AGGREGATE PRODUCTS -COMP/OP AGG PERSONAL AND ADV INJURY EACH OCCURRENCE FIRE DAMAGE (ANY ONE FIRE)	[**] [**] [**] [**] [**]

					MED EXP (ANY ONE PERSON)	[**]
A	**AUTOMOBILE LIABILITY** Any Auto	[**]	[**]	[**]	COMBINED SINGLE LIMIT BODILY INJURY (PER PERSON) BODILY INJURY (PER ACCIDENT) PROPERTY DAMAGE	[**]
	EXCESS LIABILITY				EACH OCCURRENCE AGGREGATE	
	GARAGE LIABILITY				AUTO ONLY (PER ACCIDENT OTHER THAN AUTO ONLY: EACH ACCIDENT AGGREGATE	
A	**WORKERS COMPENSATION/ EMPLOYERS LIABILITY** THE PROPRIETOR / PARTNERS / EXECUTIVE OFFICERS ARE Included	[**]	[**]	[**]	WORKERS COMP LIMITS EL EACH ACCIDENT EL DISEASE — POLICY LIMIT EL DISEASE — EACH EMPLOYEE	Statutory [**] [**] [**]

The Memorandum of Insurance serves solely to list insurance policies, limits and dates of coverage. Any modifications hereto are not authorized.

MEMORANDUM OF INSURANCE

DATE
31-Aug-2015

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PRODUCER
[**]

INSURED
Agilent Technologies, Inc.
5301 Stevens Creek Blvd.
M/S 1B-08, Santa Clara
California 95051
United States

ADDITIONAL INFORMATION
Work Comp/Employers Liability

 All states coverage except [**]

 Work Comp excludes: [**]

The Memorandum of Insurance serves solely to list insurance policies, limits and dates of coverage. Any modifications hereto are not authorized.

EXHIBIT E

[RESERVED]

EXHIBIT F

LIST OF PATENTS

Pursuant to Section 9.6.1 of the Agreement, the following Patents that cover the Process are [**].

[**]

Country/Treaty	Patent/Application #	Title	Filing Date
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]

[**]

Country/Treaty	Patent/Application #	Title	Filing Date
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]

EXHIBIT G

CONFIDENTIALITY AGREEMENT

OPHTHOTECH

CONFIDENTIALITY AGREEMENT

This Agreement dated 22 March, 2011 (the "Effective Date"), between Ophthotech Corporation, a Delaware corporation ("Ophthotech") with offices at 5 Vaughn Drive, Suite 106, Princeton, New Jersey, 08540, and Agilent Technologies , a Delaware corporation ("Agilent") with office at 5555 Airport Blvd # 100, Boulder, CO 80301-2648

1. Background. Ophthotech and Agilent (hereinafter collectively referred to as the "Parties", respectively as the "Party") intend to engage in discussions relating to the development, manufacture, and testing of Ophthotech Drug Substance and Drug Products including E10030 and ARC1905 and other, as mutually agreed to between the Parties (the "Purposes"). In the course of these discussions it is anticipated that each Party will disclose or deliver to the other Party and to the other Party's contractors and designees,(collectively, the "Representatives") certain of its trade secrets or confidential or proprietary information for the purposes of enabling the other Party to perform its obligations under the Purposes. The Parties have entered into this Agreement in order to assure the confidentiality of such trade secrets and confidential or proprietary information in accordance with the terms of this Agreement. As used in this Agreement, the Party disclosing Proprietary Information (as defined below) is referred to as the "Disclosing Party"; the Party receiving such Proprietary Information is referred to as the "Recipient".

2. Proprietary Information. As used in this Agreement, the term "Proprietary Information" shall mean all trade secrets or confidential or proprietary information designated as such in writing by the Disclosing Party, whether by letter or by the use of an appropriate proprietary stamp or legend, prior to or at the time any such trade secret or confidential or proprietary information is disclosed by the Disclosing Party or the Disclosing Party's Representatives to the Recipient or the Recipient's Representatives. Notwithstanding the foregoing, information which is orally or visually disclosed to the Recipient by the Disclosing Party, or is disclosed in writing without an appropriate letter, proprietary stamp or legend, shall constitute Proprietary Information if (i) it would be apparent to a reasonable person, familiar with the Disclosing Party's business and the industry in which it operates, that such information is of a confidential or proprietary nature the maintenance of which is important to the Disclosing Party or if (ii) the Disclosing Party, within [**] days after such disclosure, delivers to the Recipient a written document or documents describing such Proprietary Information and referencing the place and date of such oral, visual or written disclosure and the names of the Representatives of the Recipient to whom such disclosure was made. In addition, the term "Proprietary Information" shall be deemed to include: (a) any notes, analyses, compilations, studies, interpretations, memoranda or other documents prepared by the Recipient or its Representatives which contain, reflect or are based upon, in whole or in part, any Proprietary Information furnished to the Recipient or its Representatives pursuant hereto; and (b) the existence or status of, and any information concerning, the discussions between the Parties concerning the possible establishment of a business relationship.

3. Scope of Agreement. This Agreement shall apply to all Proprietary Information disclosed between the Parties hereto from the Effective Date until third anniversary of the Effective Date.

4. Use and Disclosure of Proprietary Information. The Recipient and its Representatives shall use Proprietary Information only for the Purposes and such Proprietary Information shall not be used for any other purpose without the prior written consent of the Disclosing Party. The Recipient and its Representatives shall hold in confidence, and shall not disclose Proprietary Information; provided, however, that (i) the Recipient may make any disclosure of such information to which the Disclosing Party gives its prior written consent; and (ii) any of the Proprietary Information may be disclosed by the Recipient to its Representatives who need to know such information in connection with the Purposes and who are informed of the confidential nature of such information and of the terms of this Agreement. In any event, the Recipient shall be responsible for any breach of this Agreement by any of its Representatives, and agrees, at its sole expense, to take reasonable measures to restrain its Representatives from prohibited or unauthorized disclosure or use of the Proprietary Information. Notwithstanding anything contained in this Agreement to the contrary, this Agreement shall not prohibit the Recipient from disclosing Proprietary Information of the Disclosing Party to the extent required in order for the Recipient to comply with applicable laws and regulations, provided that the Recipient provides prior written notice of such required disclosure to the Disclosing Party.

5. Limitation on Obligations. The obligations of the Recipient specified in Section 4 and 7 shall not apply, and the Recipient shall have no further obligations, with respect to any Proprietary Information to the extent that such Proprietary Information:

(a) is generally known to the public at the time of disclosure or becomes generally known without the Recipient or its Representatives violating this Agreement;

(b) is in the Recipient's possession at the time of disclosure;

becomes known to the Recipient through disclosure by sources other than the Disclosing Party without such sources violating any confidentiality obligations to the Disclosing Party; or

(c) is independently developed by the Recipient without reference to or reliance upon Proprietary Information.

6. Ownership of Proprietary Information. The Recipient agrees that it shall not receive any right, title or interest in, or any license or right to use, Proprietary Information or any Disclosing Party's patent, copyright, trade secret, trademark or other intellectual property rights therein, by implication or otherwise. Each of the Parties hereto represents, warrants and covenants that the trade secrets herein which it discloses to the other Party pursuant to this Agreement have not been stolen, appropriated, obtained or converted without authorization.

7. Return of Proprietary Information. The Recipient shall, upon the written request of the Disclosing Party, return to the Disclosing Party all Proprietary Information (and all copies and reproductions thereof). In addition, the Recipient shall destroy: (i) the part of any notes,

reports or other documents prepared by the Recipient which contain Proprietary Information; and (ii) any Proprietary Information (and all copies and reproductions thereof) which is in electronic form or cannot otherwise be returned to the Disclosing Party. Alternatively, upon written request of the Disclosing Party, the Recipient shall destroy all Proprietary Information from the Disclosing Party (and all copies and reproduction thereof) and the part of any notes, reports or other documents prepared by the Recipient which contain Proprietary Information. Notwithstanding the return or destruction of the Proprietary Information, the Recipient and its Representatives will continue to be bound by their obligations specified in Section 4, 5 and 7.

8. Miscellaneous.

(a) This Agreement supersedes all prior agreements, written or oral, between the Patties relating to the subject matter of this Agreement. This Agreement may not be assigned modified, changed or discharged, in whole or in part, except by an agreement in writing signed by the Parties.

(b) This Agreement will be binding upon and inure to the benefit of the Parties and their respective heirs, successors and assigns. Notwithstanding the forgoing, such heirs, successors and assignments shall not release such assigning Party from any of its obligations under this Agreement.

(c) This Agreement shall be construed and interpreted in accordance with the internal laws of the State of New Jersey, without giving effect to the principles of conflicts of law thereof.

(d) The provisions of this Agreement are necessary for the protection of the business and goodwill of the Patties and are considered by the Parties to be reasonable for such purpose. The Recipient agrees that any breach of this Agreement will cause the Disclosing Party substantial and irreparable injury and, therefore, in the event of any such breach, in addition to other remedies which may be available, the Disclosing Party shall have the right to specific performance and other injunctive and equitable relief.

(e) The obligations of the Recipient specified in Section 4, 5 and 7 imposed by this Agreement shall continue until the [**] anniversary of the Effective Date.

(f) For the convenience of the Parties, this Agreement may be executed by facsimile and in counterparts, each of which shall be deemed to be an original, and both of which taken together, shall constitute one agreement binding on both Parties.

EXECUTED as of the day and year first set forth above.

AGILENT TECHNOLOGIES	OPHTHOTECH CORPORATION
By: /s/ James Powell	By: /s/ Richard Everett

Name: James Powell	Name: Richard Everett
Title: General Manager NASD	Title: VP, CMC Operations

AMENDMENT #1
TO
CONFIDENTIALITY AGREEMENT
BY AND BETWEEN
AGILENT TECHNOLOGIES, INC.
AND
OPHTHOTECH CORPORATION

This Amendment # 1 ("Amendment") amends the Confidentiality Agreement by and between Agilent Technologies, Inc. ("Agilent") and Ophthotech Corporation ("Ophthotech") dated as of 22 March 2011 (the "Agreement").

Agilent and Ophthotech hereby agree to amend the Agreement as follows:

1. Section 3, Scope of Agreement, is hereby deleted in its entirety and replaced with the following:

   "This Agreement shall apply to all Proprietary Information disclosed between the Parties hereto from the Effective Date until the tenth anniversary of the Effective Date."

2. Section 8(e) is hereby deleted in its entirety and replaced with the following:

   "The obligations of the Recipient specified in Section 4, 5 and 7 imposed by this Agreement shall continue until the [**] anniversary of the expiration or termination of this Agreement."

3. This Amendment shall take effect as of 22 March 2014.

4. This Amendment constitutes the entire agreement between the parties and incorporates all prior agreements and amendments by reference. Except as; expressly amended by this Amendment, all other terms and conditions of the Agreement shall remain in full force and effect. All capitalized terms used in this Amendment but not otherwise defined herein, shall have the meaning assigned to them in the Agreement.

AGREED:

AGILENT TECHNOLOGIES, INC.		OPHTHOTECH CORPORATION	
By:	/s/ Nelson Thune	By:	/s/ Barbara A. Wood
Name:	Nelson Thune	Name:	Barbara A. Wood
Title:	General Manager	Title:	SVP, General Counsel and Corporate Secretary
Date:	02 May 2014	Date:	14 April 2014

EXHIBIT H

[**] Process Flow

[**]

EXHIBIT I

[RESERVED]

EXHIBIT J

PRODUCT PRICING

Pursuant to Section 6.1 of the Agreement, the following table provides not to exceed Product pricing based on quantities ordered via a single Purchase Order. [**].

Pricing Tiers:	Price per Batch:
[**]	[**]

EXHIBIT K

RISK MANAGEMENT PLAN

[**].

EXHIBIT L

[RESERVED]

EXHIBIT M

SPECIFICATION

[**] SPECIFICATIONS
API Specification

Effective Date:

ADP Number:

1. **GENERAL INFORMATION**

1.1.	Agilent Product Name:	[**]
1.2.	Customer Reference:	E10030
1.3.	Product Type:	PEGylated oligonucleotide
1.4.	Storage Conditions:	[**]
1.5.	Length:	[**]
1.6.	Backbone:	[**]
1.7.	Material State:	[**]
1.8.	Sequence:	[**]
	Legend:	[**]
1.9.	Theoretical Molecular Weight:	[**]

2. **SAMPLE HANDLING**

[**]

3. **SPECIFICATIONS**

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of three pages were omitted. [**]

4. **MATERIALS REQUIREMENTS**

[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]

5. **REVISION HISTORY**

Document Number	Section	Change

[**]	[**]	[**]

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of two pages were omitted. [**]

EXHIBIT N - COMMERCIAL READINESS PLAN

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of seven pages were omitted. [**]

EXHIBIT O

CRITICAL RAW MATERIALS AND SUPPLIES

Raw Materials To Be Considered for Safety Stock Inventory
[**]

OPHTHOTECH

July 17, 2015

Todd N. Smith

Re: Separation Agreement and General Release

Dear Todd:

This letter agreement (the "Letter Agreement") confirms our agreement concerning your separation from Ophthotech Corporation ("Ophthotech" or the "Company"). Subject to the terms of this Letter Agreement, your employment will end effective July 31, 2015 (the "Separation Date"). By signing a copy of this Letter Agreement in the space provided below, you agree to the terms and conditions set forth herein.

A. The Company's Obligations. In exchange for your execution (and non-revocation) of and compliance with this Letter Agreement and subject to its terms and conditions, the Company will provide you with the following payments and benefits on the Separation Date:

1. Pursuant to their terms, the stock option and restricted stock unit awards granted to you by the Company (the "Stock Grants") will terminate effective as of the Separation Date.

2. The Company will provide you with a severance payment ("Severance Payment"). The Severance Payment shall consist of:

   i. a lump sum payment in the amount of $403,500, consisting of twelve (12) months of your current base salary; and

   ii. a lump sum payment in the amount of $105,313, consisting of the pro- rated portion of your Target Bonus (as such term is defined in your March 5, 2015 agreement) ("March 5, 2015 Agreement").

   Section 4(a) of the March 5, 2015 Agreement (Code Section 409A) shall govern any payment under this Letter Agreement.

   Except as provided for in this Paragraph A.2, both the March 5, 2015 Agreement and the letter outlining the terms of your offer of employment with the Company dated September 29, 2014 (the "Offer Letter") are of no further force or effect.

3. Your group medical and dental coverage will continue through the last date of the month in which your Separation Date occurs. You will be given separate information regarding your right to continue your group health/dental/vision coverage, as required by the Consolidated Omnibus Budget Reconciliation Act of

---

1985 ("COBRA"). All COBRA rights are subject to your completion and submission of the proper forms in the times allotted.

Provided you timely elect COBRA continuation coverage, the Company will reimburse you for the monthly premium to continue such coverage for the lesser of (i) the twelve (12) full calendar months immediately following the last day of the calendar month in which your Separation Date occurs; and (ii) the end of the calendar month in which you become eligible to receive group health plan coverage under another employee benefit plan. For the avoidance of doubt, such reimbursement of monthly premiums shall be subject to Section 4(a) of the March 5, 2015 Agreement (Code Section 409A).

4. All payments under this Letter Agreement will be subject to all deductions required by law, including applicable taxes and withholdings. In accordance with its normal payroll practices, the Company will mail to the address listed above (or such other address as you have provided in writing to the Company's Human Resources Department) an IRS Form W-2 (a) following the end of 2015, covering compensation you received in 2015, inclusive of the Severance Payment and any COBRA reimbursement payments received in 2015 and (b) following the end of 2016, covering any COBRA reimbursement payments received in 2016.

B. Your Obligations. In consideration for the Company providing you with the payments and benefits described in Section A, above, to which you are not otherwise entitled, you voluntarily agree to the following:

1. You, for yourself and for your heirs, executors, administrators, successors and assigns (referred to collectively as "Releasor"), forever release and discharge the Company and any and all of the Company's past and present affiliates, parent entities, subsidiaries, divisions, offices, branches, assets, employee benefit plans, funds, investment funds, successors and assigns, and any and all of its and their past and present officers, directors, partners, members, shareholders, agents, attorneys, employees, agents, trustees, fiduciaries, representatives, administrators, successors and assigns (whether acting in such capacity or otherwise) (referred to collectively as the "Releasees"), from any and all claims, demands, causes of action, fees and liabilities of any kind whatsoever, whether known or unknown, which Releasor ever had, now has or may have against Releasees or any of them by reason of any actual or alleged act, omission, transaction, practice, conduct, occurrence or other matter from the beginning of the world up to and including the date you sign this Letter Agreement based on your employment with the Company and the termination of your employment (other than claims you may have based upon your rights under this Letter Agreement).

2. Without limiting the generality of the foregoing general release, by signing this Letter Agreement you agree that you are releasing Releasees from any and all claims arising out of your employment with the Company, the terms and conditions of such, employment and/or the termination of such employment, including but not limited to: (i) any claim under the Employee Retirement Income Security Act of

1974 (“ERISA”), Title VII of the Civil Rights Act of 1964, the Civil Rights Act of 1991, the Civil Rights Act of 1866, the Age Discrimination in Employment Act (including the Older Workers Benefit Protection Act), the Equal Pay Act, the Americans with Disabilities Act, the Family and Medical Leave Act, the National Labor Relations Act, the Sarbanes-Oxley Act, the Dodd-Frank Act, the New York State Human Rights Law, the New York City Human Rights Law, the New York Labor Law (all as amended), and any other applicable federal, state or local statute; (ii) any other claim of discrimination, harassment or retaliation in employment (whether based on federal, state or local law, statutory or decisional); (iii) any claim sounding in tort, common law or contract (express or implied), wrongful discharge, whistleblowing, detrimental reliance, or defamation; (iv) any claim based on the Stock Grants; and (v) any claim for attorney’s fees, costs, disbursements, emotional distress, compensatory and/or punitive damages and/or the like.

3. You acknowledge that you may hereafter discover claims or facts in addition to or different from those which you now know or believe to exist with respect to the subject matter of this Letter Agreement and which, if known or suspected at the time you execute this Letter Agreement, may have materially affected this Letter Agreement and your decision to enter into it. Nevertheless, you hereby waive any right, claim or cause of action that might arise as a result of such different or additional claims or facts.

4. You represent and warrant that you have maintained in the strictest confidence all information relating to the Company and/or the Releasees and their respective business that is not generally known by persons not employed by the Company and that could not easily be determined of learned by someone outside of the Company. All of the foregoing shall be deemed “Confidential Information.” You agree that you will maintain in the strictest confidence all Confidential Information, except as set forth below. In addition, you hereby acknowledge and affirm your post-termination obligations under the Invention, Non-Disclosure, Non-Competition and Non-Solicitation Agreement between you and the Company dated September 29, 2014 (the “Covenant Agreement”), which are expressly incorporated herein.

5. You agree that you have not and in the future will not disclose to any other person or entity (directly or indirectly), Confidential Information, except (a) as may be required pursuant to a valid subpoena, a request by a government agency (including but not limited to the United States Equal Employment Opportunity Commission (“EEOC”) or the Securities and Exchange Commission (“SEC”) in connection with any charge filed, investigation or proceeding or as otherwise required by law; and (b) to your immediate family members, financial advisors and attorneys, provided that you first inform them of the confidentiality of this Agreement and they agree to maintain its confidentiality. Yon further agree that you will not solicit or initiate any demand or request by others for the disclosure of Confidential Information; or encourage or induce any other person to make any statement or disclosure of Confidential Information. In the event that you receive an inquiry from the press or otherwise that could potentially call for the disclosure of Confidential Information,

you will respond to the inquiry, if at all, by stating “I cannot comment” or words to that effect.

6. You will cooperate fully with the Company, and provide assistance to the Company, in connection with (a) the orderly transition of all of your responsibilities and matters, (b) any pending or future litigation, administrative proceeding, or investigatory matter, and (c) any other matters for which you were responsible or with respect to which your knowledge may be of assistance to the Company. You further agree that, in the event you are subpoenaed by any person or entity (including, but not limited to, any government agency) to give testimony (in a deposition, court proceeding or otherwise) which in any way relates to your employment with the Company, you will give prompt written notice of such request to the Company’s Head of Human Resources, at the address above to allow the Company a reasonable opportunity to first contest the right of the requesting person or entity to such disclosure. Nothing in this Letter Agreement shall preclude you from responding truthfully to a valid subpoena. You agree to provide such cooperation and assistance as requested by the Company, subject to the reasonable efforts of the Company to accommodate any new employment obligations you may have, and the Company shall reimburse you for your reasonable out-of-pocket expenses in connection therewith. For the avoidance of doubt, nothing in this Paragraph or elsewhere in the Agreement is intended in any way to prevent you from testifying fully and truthfully in any action or proceeding or in connection with any regulatory matter.

7. You agree that you have not and will not make any disparaging, critical or otherwise detrimental statements (orally or in writing) to any person or entity concerning the Company, its officers, directors, managing members, investors, employees, attorneys, representatives, affiliates, customers, clients, its and their business affairs or financial condition, the circumstances surrounding your employment and separation from the Company. For purposes of this Letter Agreement, the term “disparage” shall mean any oral or written statement or representation which, directly or by implication, tends, in the minds of a reasonable audience, to create a negative impression about the subject of the statement or representation, and includes, without limitation, comments or statements to the press and/or media, including, but not limited to, print journalists, press interviews or statements, newspapers, radio, television, cable, satellite programs, or Internet media (including blogs, web pages, web posts, email, and or “chat programs”), or to the Company, its officers, directors, employees, affiliates, customers, clients, or any person or entity with which the Company has a business relationship which would: (a) adversely affect in any manner the conduct of the business of the Company or the Company’s business relationships; (b) adversely affect in any manner the business reputation of the Company, its officers, directors, managing members, investors, employees, attorneys, representatives, affiliates, customers, clients, or any person or entity with which the Company has a business relationship; (c) induce or encourage others, to disparage the Company, its officers, directors, managing members, investors, employees, attorneys, representatives,

affiliates, customers, clients, or any person or entity with which the Company has a business relationship.

8. Nothing in this agreement shall be construed to prohibit you from reporting possible violations of federal or state law or regulations to any governmental agency or self-regulatory organization, or making other disclosures that are protected under whistleblower or other provisions of any applicable federal or state law or regulations. Nothing contained in this Letter Agreement shall prohibit you from filing a charge with, or participating in any investigation or proceeding conducted by, the EEOC, or other federal, state or local government agency, except

that you understand and agree that you will not be able to recover monetary or equitable relief of any kind from Releasees in connection with any such charged filed by you or on your behalf in connection with any action filed by a third party with respect to the claims you are waiving in this Letter Agreement Additionally, nothing in this Letter Agreement shall constitute a waiver of claims arising after the date you sign it; claims that cannot be waived by law; any right to make any disclosure to or cooperate with the United States Securities and Exchange Commission ("SEC") pursuant to Section 21F(b) of the Securities and Exchange Act or to receive a reward from the SEC in connection therewith; claims for accrued, vested benefits under any employee pension plan of the Company in accordance with the terms of the official plan documents and applicable law; claims for reimbursement through the Company's Flexible Spending Account Program; or claims for benefits under the Company's group medical, vision and dental and disability plans in accordance with the terms of such plans and applicable law.

9. You agree to immediately return to the undersigned all property of the Company and/or any of the other Releasees that you have, including but not limited to records and materials, business and client information and files, cardkey access to Company offices, remote access card, desktop and laptop computer, keys, and corporate credit cards.

10. You acknowledge that apart from the payments and benefits that will be provided to you as set forth in this Letter Agreement, you have received all compensation, wages, bonuses, severance or termination pay, stock options, restricted stock units, equity grants, commissions, notice period, leave and/or benefits to which you may have been entitled to under any law, policy or plan of or sponsored by the Company, or pursuant to any prior agreement with the Company and that no other payments or benefits are due or owing to you except as set forth in this Letter Agreement, including any severance payment or benefits under the March 5, 2015 Agreement or the Offer Letter. You further affirm that you have had no known workplace injuries or occupational diseases.

C. **Mutual Understandings**. The parties mutually agree to the following provisions:

1. Any description by either (a) you or (b) the Company to any inquiring third party or in any internal company-wide communication, in the case of (a) or (b), regarding your status with the Company, shall be consistent with Exhibit A.

2. It is the Company's policy not to provide the reasons for any employee's departure unless required by law. Therefore, any prospective employer who makes an inquiry to the Human Resources Department about your employment shall contact the Company's Head of Human Resources or her designee, who will confirm only the dates of your employment, the positions you held, and your compensation (provided that compensation information will be provided only if you submit written authorization releasing this information to the Company's Head of Human Resources or her designee or to the extent required by subpoena, court order or law).

3. Notwithstanding the foregoing Paragraph C.1 and C2, nothing herein shall limit the Company's ability to make any disclosures required by the securities laws or the rules and regulations of the SEC or of any stock exchange on which the Company's shares are listed, including the filing of a Current Report on Form 8-K to disclose the fact of your resignation and the financial arrangements memorialized hereby, the inclusion of information regarding compensation paid to you as required in any filing with the SEC made by the Company and the filing of this Agreement as an exhibit to the Company's periodic reports filed pursuant to the Securities Exchange Act.

4. Nothing herein is intended to or shall be deemed to constitute an admission that the Company or any of the other Releasees have violated any federal, state or local law (statutory or decisional), ordinance or regulation, breached any contract, or committed any wrongdoing whatsoever against you or otherwise. Neither this Letter Agreement nor any of its terms may be used as an admission or introduced as evidence as to any issue of law or fact in any proceeding, suit or action, other than an action to enforce this Letter Agreement. Moreover, by signing this Letter Agreement you acknowledge that you are not aware of any wrongdoing or fraudulent or unlawful conduct on the part of the Company or the Releasees.

5. In the event that any provision of this Letter Agreement is held to be invalid, illegal or unenforceable, the validity, legality and enforceability of the remaining provisions will not in any way be affected or impaired thereby. Moreover, if any provision contained in this Letter Agreement is held to be excessively broad as to duration, scope, activity or subject, that provision will be construed by limiting and reducing it so as to be enforceable to the maximum extent compatible with applicable law.

6. This Letter Agreement, together with any attachments and exhibits hereto, constitutes the entire agreement between you and the Company with respect to the subject matter hereof and supersedes all prior negotiations, representations or

agreements relating thereto, whether written or oral, with the exception of any agreements or portions thereof expressly described herein as imposing continuing rights and obligations. You represent that in executing this Letter Agreement, you have not relied on any representation or statement not set forth herein. No amendment or modification of this Letter Agreement shall be valid or binding upon the parties unless in writing and signed by both parties.

7. This Letter Agreement will be governed by and construed in accordance with the laws of the State of New York, except as may be preempted by federal law. This Letter Agreement is binding upon, and shall inure to the benefit of, the parties and their respective hens, executors, administrators, successors and assigns.

D. **Obligations Unrelated to This Letter Agreement**. Regardless of whether you sign this Letter Agreement, you and the Releasees will have the following rights and obligations:

1. You will be paid for all accrued vacation days that remain unused as of the Separation Date, with such payment occurring within ten (10) days of the Separation Date.

2. Your participation in the Company's 401(k)/retirement plan(s) will cease on the Separation Date. You will receive any accrued vested benefits under this plan(s) in accordance with the terms of the plan and applicable law. Separate information will be given to you regarding these benefits.

E. **Consideration Period**. By signing this Letter Agreement in the space provided below and returning it to the undersigned, you are confirming your acceptance of the terms and conditions set forth herein, and you are acknowledging the following:

1. The obligations as set out in this Letter Agreement represent a complete waiver and release of all rights and claims that you have or may have against the Releasees, as provided in Paragraph B.1 above. Accordingly, you should review it carefully before signing it.

2. You may take up to twenty-one (21) days from your receipt of this Letter Agreement to consider its meaning and effect and to determine whether or not you wish to enter into it. You are advised to consult with an attorney of your choice before signing this Letter Agreement.

3. To accept this Letter Agreement, you must sign, have notarized, and deliver the Letter Agreement to **Amy Sheehan**, at the address above.

4. By signing this Letter Agreement, you acknowledge that you have carefully read this Letter Agreement in its entirety, you have had an opportunity to consider the terms of this Letter Agreement for at least twenty-one (21) days, you fully understand the significance of all the terms and conditions of this Letter Agreement and have had a reasonable opportunity to discuss them with an attorney of your choice, and you are signing this Letter Agreement voluntarily and of your own free will and agreeing to all the terms and conditions contained herein.

5. In addition, you may take seven (7) days after signing this Letter Agreement to revoke your signature (such period, the "Revocation Period"). This Letter Agreement will not become effective until after you sign this Letter Agreement and the Revocation Period expires without revocation (the "Effective Date"). Any revocation of this Letter Agreement must be in writing and delivered personally or by overnight courier to **Amy Sheehan,** in which event this Letter Agreement will become null and void and your employment with the Company will terminate immediately.

We wish you the best in your future endeavors,

Sincerely yours,

/s/ Amy Sheehan
Amy Sheehan
Vice President, Human Resources
Ophthotech

Agreed to and Accepted by:

/s/ Todd N. Smith
Todd N. Smith

Date: 7/20/2015

State of IL)
) ss.:
County of LAKE)

On this 20th day of July, 2015, before me, a Notary Public of the State of IL, personally appeared Todd N, Smith, who executed the foregoing Letter Agreement and did then and there acknowledge to me that he voluntarily executed the same.

/s/ Wanda Cervantes
Notary Public

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 September 30, 2015 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: November 5, 2015

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

Exhibit 31.2

CERTIFICATIONS

I, Michael G. Atieh, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the quarter ended September 30, 2015 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: November 5, 2015

By: /s/ Michael G. Atieh
Michael G. Atieh
Executive Vice President and Chief Financial and Business 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 September 30, 2015 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, David R. Guyer, M.D., Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 5, 2015

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 September 30, 2015 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Michael G. Atieh, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 5, 2015

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