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 June 30, 2017

OR

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

Commission file number: 001-36080

Ophthotech Corporation
(Exact name of registrant as specified in its charter)

Delaware	**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 ☐

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 ☐

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

Large accelerated filer x	Accelerated filer ☐	Non-accelerated filer ☐	Smaller reporting company ☐	Emerging growth company ☐

(Do not check if a smaller reporting company)

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

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

As of July 28, 2017 there were 35,942,879 shares of Common Stock, $0.001 par value per share, outstanding.

TABLE OF CONTENTS

PART I—FINANCIAL INFORMATION

PART II—OTHER INFORMATION

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 potential benefits of our updated business plan and strategy to initiate new development programs and potentially expand our product pipeline;
- our ability to in-license or acquire additional products, product candidates or technologies to treat ophthalmic diseases and the timing, costs, conduct and outcome of preclinical development or clinical trials we undertake for these newly acquired assets;
- our expectations related to our use of available cash;
- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;
- the timing, costs, conduct and outcome of our clinical trial of Zimura® (avacincaptad pegol) as a monotherapy for the treatment of geographic atrophy, or GA, a form of dry AMD, our planned clinical trial of Zimura in combination with an anti-VEGF drug for the treatment of wet AMD, our planned clinical trial of Zimura in combination with an anti-VEGF drug for the treatment of idiopathic polypoidal choroidal vasculopathy, our planned clinical trial of Zimura as a monotherapy for Stargardt disease, and our planned clinical trial of Zimura as a monotherapy for non-infectious intermediate and posterior uveitis, 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 remaining Phase 3 clinical trial of Fovista® (pegpleranib) administered in combination with Eylea® (aflibercept) or Avastin® (bevacizumab) for the treatment of wet age-related macular degeneration, or AMD, and the National Eye Institute-led trial of Fovista administered in combination with an anti-VEGF drug for the treatment of the retinal capillary hemangiomas associated with the orphan disease Von-Hippel-Lindau Syndrome, including statements regarding the timing and the availability of, and the costs to obtain, initial, top-line results from, and the completion of, such trials;
- the timing of and our ability to obtain marketing approval of our product candidates, and the ability of our product candidates 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 our product candidates;
- the rate and degree of potential market acceptance and clinical utility of our product candidates, if approved;
- our estimates regarding the potential market opportunity for our product candidates;
- the potential receipt of revenues from future sales of our product candidates, if approved;
- our sales, marketing and distribution capabilities and strategy;
- our ability to establish and maintain arrangements for the manufacture of our product candidates;
- our intellectual property position;

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

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and our stockholders should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this 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.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements

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

	June 30, 2017	December 31, 2016
Assets		
Current assets		
Cash and cash equivalents	$ 140,917	$ 133,930
Available for sale securities	55,525	155,348
Due from Novartis Pharma AG	674	3,531
Prepaid expenses and other current assets	2,738	3,078
Total current assets	199,854	295,887
Property and equipment, net	1,888	3,281
Other assets	46	462
Total assets	$ 201,788	$ 299,630
Liabilities and Stockholders' Deficit		
Current liabilities		
Accrued research and development expenses	$ 12,687	$ 47,240
Accounts payable and accrued expenses	6,268	12,032
Deferred revenue	6,646	6,646
Total current liabilities	25,601	65,918
Deferred revenue, long-term	200,007	203,330
Royalty purchase liability	125,000	125,000
Total liabilities	350,608	394,248
Stockholders' deficit		
Preferred stock - $0.001 par value, 5,000,000 shares authorized, no shares issued or outstanding	$ —	$ —
Common stock - $0.001 par value, 200,000,000 shares authorized, 35,932,179 and 35,733,276 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	36	36
Additional paid-in capital	515,615	504,517
Accumulated deficit	(664,285)	(598,959)
Accumulated other comprehensive loss	(186)	(212)
Total stockholders' deficit	(148,820)	(94,618)
Total liabilities and stockholders' deficit	$ 201,788	$ 299,630

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Collaboration revenue	$ 1,661	$ 28,198	$ 3,323	$ 43,919
Operating expenses:				
Research and development	15,657	48,262	47,636	86,032
General and administrative	8,552	10,489	21,711	25,185
Total operating expenses	24,209	58,751	69,347	111,217
Loss from operations	(22,548)	(30,553)	(66,024)	(67,298)
Interest income	344	446	722	892
Other loss	(1)	(98)	(22)	(68)
Loss before income tax provision (benefit)	(22,205)	(30,205)	(65,324)	(66,474)
Income tax provision (benefit)	(1)	(260)	2	(228)
Net loss	$ (22,204)	$ (29,945)	$ (65,326)	$ (66,246)
Net loss per common share:				
Basic and diluted	$ (0.62)	$ (0.85)	$ (1.82)	$ (1.88)
Weighted average common shares outstanding:				
Basic and diluted	35,858	35,392	35,831	35,324

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Net loss	$ (22,204)	$ (29,945)	$ (65,326)	$ (66,246)
Other comprehensive income (loss):				
Unrealized gain (loss) on available for sale securities, net of tax	36	(122)	26	355
Other comprehensive income (loss)	36	(122)	26	355
Comprehensive loss	$ (22,168)	$ (30,067)	$ (65,300)	$ (65,891)

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

OPHTHOTECH CORPORATION
Unaudited Statements of Cash Flows
(in thousands)

	Six Months Ended June 30,	
	2017	**2016**
Operating Activities		
Net loss	$ (65,326)	$ (66,246)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation	1,393	352
Amortization of premium and discounts on investment securities	137	331
Deferred income taxes	(8)	22,853
Share-based compensation	11,052	16,641
Changes in operating assets and liabilities:		
Due from Novartis Pharma AG	2,857	(25,808)
Income tax receivable	(52)	(20,863)
Prepaid expense and other current assets	392	187
Accrued interest receivable	275	147
Other assets	416	(3)
Accrued research and development expenses	(34,553)	6,331
Accounts payable and accrued expenses	(5,764)	(4,249)
Deferred revenue	(3,323)	364
Net cash used in operating activities	(92,504)	(69,963)
Investing Activities		
Purchase of marketable securities	(12,014)	(12,003)
Maturities of marketable securities	111,459	50,500
Purchase of property and equipment	—	(149)
Net cash provided by (used in) investing activities	99,445	38,348
Financing Activities		
Proceeds from stock option/employee stock purchase plan exercises	46	3,808
Net cash provided by financing activities	46	3,808
Net change in cash and cash equivalents	6,987	(27,807)
Cash and cash equivalents		
Beginning of period	133,930	221,861
End of period	$ 140,917	$ 194,054
Supplemental disclosures of non-cash information related to investing activities		
Change in unrealized gain (loss) on available for sale securities, net of tax	$ 26	$ 355

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

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 ophthalmic diseases, with a focus on orphan and age-related retinal diseases. The Company currently has two product candidates, Zimura® (avacincaptad pegol), an anti-complement factor C5 aptamer, and Fovista® (pegpleranib), an anti-platelet derived growth factor, or PDGF, aptamer. Prior to 2017, the Company's primary focus was on developing Fovista and Zimura for various types of age-related macular degeneration, or AMD, which is a disorder of the central portion of the retina, known as the macula, that may result in blindness. In December 2016, the Company announced that two of its three pivotal, Phase 3 clinical trials for Fovista in combination with the anti-vascular endothelial growth factor, or anti-VEGF, drug Lucentis® (ranibizumab) for the treatment of wet AMD, failed to meet their primary endpoint. The Company announced in early 2017 that it had engaged a financial advisor to assist it in reviewing the Company's strategic alternatives, including identifying potential business development opportunities. Also beginning in early 2017, the Company undertook a reassessment of its development plans for Zimura and Fovista, which included an evaluation of the scientific rationale for potentially developing these product candidates in one or more other ophthalmic indications for which there is a high unmet need.

On July 26, 2017, the Company announced that it is pursuing a strategy to leverage its clinical experience and retina expertise to identify and develop therapies to treat multiple ophthalmic orphan diseases for which there are limited or no treatment options available. To this end, the Company plans to initiate a randomized, controlled clinical trial of Zimura as a monotherapy for the treatment of Stargardt disease, an inherited retinal orphan disease causing vision loss during childhood or adolescence, before the end of 2017. In parallel, the Company is continuing its on-going programs in age-related retinal diseases, including its ongoing Phase 2/3 clinical trial of Zimura as a monotherapy for the treatment of geographic atrophy, or GA, a form of dry AMD, and its wet AMD program for Zimura. The Company will continue to reassess the Zimura GA development program in light of the results of a competitor's Phase 3 clinical trials of a complement inhibitor being studied for the treatment of GA, which are expected to be available during the second half of 2017. The Company is also continuing its business development efforts to identify and potentially obtain rights to additional products, product candidates and technologies that would complement its strategic goals as well as other compelling ophthalmology opportunities.

The Company's third, pivotal Phase 3 clinical trial of Fovista in combination with the anti-VEGF drugs Eylea® (aflibercept) or Avastin® (bevacizumab) for the treatment of wet AMD, referred to as OPH1004, also remains ongoing with initial, top-line data expected by the end of the third quarter of 2017. The Company's strategy for its Fovista wet AMD development program will be primarily determined based on these data and in the context of its other two failed Phase 3 clinical trials. The Company believes that the failure of its two prior Phase 3 trials and the failure of a competitor's Phase 2 trial investigating the combination of a PDGF inhibitor and a VEGF inhibitor are indicative of a low likelihood of success for OPH1004.

As a result of the Company's ongoing reassessment of its development programs and potential business development opportunities, the Company may modify, expand or terminate some or all of its development programs or clinical trials at any time.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited financial information as of June 30, 2017 and for the three and six months ended June 30, 2017 and 2016 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, 2016 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 2016, as filed with the SEC on February 28, 2017.

In the opinion of management, the unaudited financial information as of June 30, 2017 and for the three and six months ended June 30, 2017 and 2016, reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of the financial position, results of operations and cash flows of the Company. The results of operations for the three and six months ended June 30, 2017 and 2016 are not necessarily indicative of the operating results for the full fiscal year or any future period.

Segment and geographic information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision making group, in deciding how to allocate resources and in assessing performance. The Company views its operations and manages its business in one operating and reporting segment.

Use of Estimates

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

Cash and Cash Equivalents

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

As of June 30, 2017, the Company had cash, cash equivalents and available for sale securities of approximately $196.4 million. The Company believes that its existing cash, cash equivalents and available for sale securities as of June 30, 2017 will be sufficient to fund its operations and capital expenditure requirements as currently planned for at least the next 12 months.

Available for Sale Securities

The Company considers securities with original maturities of greater than 90 days when purchased to be available for sale securities. Available for sale securities with original maturities of greater than one year are recorded as non-current assets. Available for sale securities are recorded at fair value and unrealized gains and losses are recorded within accumulated other comprehensive income (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 the Company's right to terminate the agreement and associated obligation to repay the upfront payment under certain circumstances. In each of September 2014 and March 2015, the Company achieved a $50.0 million enrollment-based milestone, and in June 2016, the Company achieved a $30.0 million enrollment-based milestone, for an aggregate total of $130.0 million, under the Novartis Agreement. The Company uses the relative selling price method to allocate arrangement consideration to the Company's performance obligations under the Novartis Agreement. Below is a summary of the components of the Company's collaboration revenue for the three and six months ended June 30, 2017 and 2016:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
License revenue	$ —	$ 22,937	$ —	$ 22,937
Research and development activity revenue	1,658	5,150	3,316	6,425
API transfer revenue	—	102	—	14,545
Joint operating committee revenue	3	9	7	12
Total collaboration revenue	$ 1,661	$ 28,198	$ 3,323	$ 43,919

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

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

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

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

At the inception of arrangements that include milestone payments, the Company evaluates whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether (a) the consideration is commensurate with either (1) the entity's performance to achieve the milestone, or (2) the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the entity's performance to achieve the milestone, (b) the consideration relates solely to past performance, and (c) the consideration is

reasonable relative to all of the deliverables and payment terms within the arrangement. The Company evaluates factors such as the scientific, regulatory, commercial and other risks that must be overcome to achieve the respective milestone, the level of effort and investment required to achieve the respective milestone and whether the milestone consideration is reasonable relative to all deliverables and payment terms in the arrangement in making this assessment.

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

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

Concentration of Credit Risk

The Company's financial instruments that are exposed to concentration of credit risk consist primarily of cash and cash equivalents and available for sale securities. The Company maintains its cash in bank accounts, which generally exceed federally insured limits. The Company maintains its cash equivalents in investments in money market funds and, at times, in U.S. Treasury securities and investment-grade corporate debt securities with original maturities of 90 days or less.

The Company's available for sale securities are also invested in U.S. Treasury securities and investment-grade corporate debt securities. The Company believes it is not exposed to significant credit risk on its cash, cash equivalents and available for sale securities.

Concentration of Suppliers

The Company currently relies exclusively upon a single third-party manufacturer to provide supplies of the active pharmaceutical ingredient, or API, for both Zimura and Fovista. The Company also engages a single third-party manufacturer to provide fill/finish services for clinical supplies of both Zimura and Fovista. In addition, the Company currently relies exclusively upon Nektar Therapeutics, or Nektar, to supply it with a proprietary polyethylene glycol, or PEG, reagent for Fovista under a manufacturing and supply agreement. PEG reagent is a chemical the Company uses to modify the chemically synthesized aptamer in Fovista. The PEG reagent made by Nektar is proprietary to Nektar. The Company obtains a different proprietary PEG reagent used to modify the chemically synthesized aptamer in Zimura from a different supplier on a purchase order basis. Furthermore, the Company and its contract manufacturers 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 Zimura and Fovista. If the Company's third-party manufacturers or fill/finish service providers should become unavailable to the Company for any reason, including as a result of capacity constraints, financial difficulties or insolvency, the Company believes that there are a limited number of potential replacement manufacturers, and the Company likely would incur added costs and delays in identifying or qualifying such replacements.

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.

Financial Instruments

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

Property and Equipment

Property and equipment, which consists mainly of manufacturing and clinical equipment, furniture and fixtures, computers, software, 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. Amortization of leasehold improvements is recorded over the shorter of the lease term or estimated useful life of the related asset.

Research and Development

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

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

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

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

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

Income Taxes

The Company utilizes the liability method of accounting for deferred income taxes, as set forth in ASC 740, *Income Taxes*. Under this method, deferred tax assets and liabilities are recognized for the expected future tax consequences of temporary differences between the carrying amounts and the tax basis of assets and liabilities. The Company incurred U.S. federal net operating losses (“NOLs”) in each year from its inception in 2007 through 2013 and utilized these NOLs in 2014. Additionally, the Company incurred a U.S. federal net operating loss in 2015 that has been carried back to 2014. Accordingly, all tax years since 2007 are subject to potential tax examination. In the second quarter of 2016, the Internal Revenue Service began an examination of the Company’s 2014 corporate income tax return. This audit was subsequently expanded to include the 2014 and 2015 tax years. Field work on this audit was completed during the second quarter of 2017 resulting in a de minimis preliminary assessment of additional tax. The audit results are currently under review by the IRS Joint Committee. Additionally, the Company received notification from the New York State Department of Taxation and Finance of its intention to perform an audit of the Company's New York State income tax returns for the tax years 2013, 2014 and 2015. Further, the New York City Department of Finance has notified the Company of its intention to audit the Company's New York City General Corporation Tax return for the 2014 tax year. These audits commenced during the second quarter of 2017.

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, restricted stock units (“RSUs”) and the option granted to employees to purchase shares under the 2016 Employee Stock Purchase Plan (the “ESPP”). 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 estimated forfeitures. For grants containing performance-based vesting provisions, expense is recognized over the estimated achievement period.

Stock Options

The Company estimates the fair value of stock options granted to employees and non-employee directors on the date of grant using the Black-Scholes option-pricing model. Due to the lack of trading history, the Company's computation of stock-price volatility is based on the volatility rates of comparable publicly held companies over a period equal to the expected term of the options granted by the Company. The Company's computation of expected term is determined using the "simplified" method, which is the midpoint between the vesting date and the end of the contractual term. The Company believes that it does not have sufficient reliable exercise data in order to justify the use of a method other than the "simplified" method of estimating the expected exercise term of employee stock option grants. The Company utilizes a dividend yield of zero based on the fact that the Company has never paid cash dividends to stockholders and has no current intentions to pay cash dividends. The risk-free interest rate is based on the zero-coupon U.S. Treasury yield at the date of grant for a term equivalent to the expected term of the option.

For stock options granted as consideration for services rendered by consultants, the Company recognizes expense in accordance with the requirements of ASC 505-50, *Equity Based Payments to Non-Employees*. Consultant stock option grants are recorded as an expense over the vesting period of the underlying stock options. At the end of each financial reporting period prior to vesting, the value of these options, as calculated using the Black-Scholes option-pricing model, will be re-measured using the fair value of the Company's common stock and the non-cash expense recognized during the period will be adjusted accordingly. Since the fair value of options granted to consultants is subject to change in the future, the amount of the future expense will include fair value re-measurements until the stock options are fully vested.

RSUs

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

ESPP

In April 2016, the board of directors adopted the ESPP pursuant to which the Company may sell up to an aggregate of 1,000,000 shares of common stock. The ESPP was approved by the Company's stockholders in June 2016. The ESPP is considered compensatory and the fair value of the discount and look back provision are estimated using the Black-Scholes option-pricing model and recognized over the six month withholding period prior to purchase.

Share-based compensation expense includes expenses related to stock options and RSUs granted to employees, non-employee directors and consultants, as well as the option granted to employees to purchase shares under the ESPP, all of which have been reported in the Company's Statements of Operations as follows:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
Research and development	$ 2,897	$ 6,383	$ 7,047	$ 11,068
General and administrative	2,091	1,926	4,005	5,573
Total	$ 4,988	$ 8,309	$ 11,052	$ 16,641

Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board, or FASB, issued Accounting Standards Update, or ASU, No. 2014-9, *Revenue from Contracts with Customers (Topic 606)* ("ASU 2014-9"). ASU 2014-9 outlines a new, single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. This new revenue recognition model provides a five-step analysis in determining when and how revenue is recognized. The new model will require revenue recognition to depict the transfer of promised goods or services to customers in an amount that reflects the consideration a company expects to receive in exchange for those goods or services. The FASB subsequently issued additional clarifying standards to address issues arising from implementation of the new revenue standard, including a one-year deferral of the effective date for the new

revenue standard. Public companies should now apply the guidance in ASU 2014-9 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 2014-9. The Company has not yet completed its final review of the impact of this guidance. The Company anticipates, however, applying the modified retrospective method and initially applying a cumulative effect of the standard as an adjustment to its opening retained earnings balance upon the adoption of ASU 2014-9, effective January 1, 2018.

In February 2016, the FASB issued ASU No. 2016-2, *Leases (Topic 842)*. Under the new guidance, lessees will be required to recognize the following for all leases (with the exception of short-term leases) at the commencement date: (1) a lease liability, which is a lessee's obligation to make lease payments arising from a lease, measured on a discounted basis; and (2) a right-of-use asset, which is an asset that represents the lessee's right to use, or control the use of, a specified asset for the lease term. Under the new guidance, lessor accounting is largely unchanged. Certain targeted improvements were made to align, where necessary, lessor accounting with the lessee accounting model and Topic 606, *Revenue from Contracts with Customers*. The new lease guidance simplified the accounting for sale and leaseback transactions primarily because lessees must recognize lease assets and lease liabilities. Lessees will no longer be provided with a source of off-balance sheet financing. Publicly-traded business entities should apply the amendments in ASU 2016-2 for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years (i.e., January 1, 2019, for a calendar year entity). Early application is permitted for all publicly-traded business entities and all nonpublicly-traded business entities upon issuance. Lessees (for capital and operating leases) and lessors (for sales-type, direct financing, and operating leases) must apply a modified retrospective transition approach for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements. The modified retrospective approach would not require any transition accounting for leases that expired before the earliest comparative period presented. Lessees and lessors may not apply a full retrospective transition approach. The Company is currently assessing the impact that adopting this new accounting guidance will have on its financial statements and footnote disclosures.

In August 2016, the FASB issued ASU No. 2016-15, *Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments*, which clarifies the presentation of certain specific cash flow issues in the Statement of Cash Flows. For public companies, the amendments are effective for annual reporting periods beginning after December 15, 2017, and interim periods within those annual periods and early adoption is permitted. This new guidance is not expected to have a material impact on the Company's Consolidated Financial Statements.

In January 2017, the FASB issued ASU No. 2017-01, *Business Combinations (Topic 805): Clarifying the Definition of a Business*, in an effort to clarify the definition of a business with the objective of adding guidance to assist entities with evaluating whether transactions should be accounted for as acquisitions (or disposals) of assets or businesses. The amendments of this ASU are effective for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years. Early adoption is permitted. This new guidance will be applicable for the Company's acquisitions on or after January 1, 2018.

3. Net 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 and RSUs have been excluded from the calculation of diluted net loss per common share because their effect would be anti-dilutive. Therefore, the weighted average common shares used to calculate both basic and diluted net loss per common share would be the same. The following table sets forth the computation of basic and diluted net income (loss) per common share for the periods indicated:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
Basic and diluted net loss per common share calculation:				
Net loss	$ (22,204)	$ (29,945)	$ (65,326)	$ (66,246)
Weighted average common shares outstanding - basic and diluted	35,858	35,392	35,831	35,324
Net loss per share of common stock - basic and diluted	$ (0.62)	$ (0.85)	$ (1.82)	$ (1.88)

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 June 30,		Six months ended June 30,	
	2017	2016	2017	2016
Stock options outstanding	3,805	3,584	3,805	3,584
Restricted stock units	535	661	535	661
Total	4,340	4,245	4,340	4,245

4. Cash, Cash Equivalents and Available for Sale Securities

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at the date of purchase to be cash equivalents. Cash and cash equivalents included cash of $4.3 million and $25.8 million at June 30, 2017 and December 31, 2016, respectively. Cash and cash equivalents at June 30, 2017 and December 31, 2016 also included $136.6 million and $108.1 million, respectively, of investments in money market funds, U.S. Treasury securities and certain short-term investment-grade corporate debt securities with original maturities of 90 days or less.

The Company considers securities with original maturities of greater than 90 days at the date of purchase to be available for sale securities. The Company held available for sale securities with a fair value totaling $55.5 million and $155.3 million at June 30, 2017 and December 31, 2016, respectively. These available for sale securities consisted of U.S. Treasury securities and investment-grade corporate debt securities. At June 30, 2017, the Company held available for sale securities of $55.5 million with maturities of less than one year. The Company did not hold any securities with maturities of greater than one year at June 30, 2017. 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 June 30, 2017. The Company classifies these securities as available for sale, however, the Company does not currently intend to sell its investments and the Company believes it is more likely than not that the Company will recover the carrying value of these investments.

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

	As of June 30, 2017			
	Cost	Unrealized Gains	Unrealized Losses	Fair Value
U.S. Treasury securities	$ 48,147	$ —	$ (13)	$ 48,134
Corporate debt securities	7,392	—	(1)	7,391
Total	$ 55,539	$ —	$ (14)	$ 55,525

	As of December 31, 2016			
	Cost	Unrealized Gains	Unrealized Losses	Fair Value
U.S. Treasury securities	$ 120,288	$ 6	$ (33)	$ 120,261
Corporate debt securities	35,114	—	(27)	35,087
Total	$ 155,402	$ 6	$ (60)	$ 155,348

The Company's available for sale securities are reported at fair value on the Company's Balance Sheets. Unrealized gains (losses) are reported within accumulated other comprehensive income (loss) in the statements of comprehensive income (loss). The cost of securities sold and any realized gains/losses from the sale of available for sale securities are based on the specific identification method. The changes in accumulated other comprehensive income (loss) associated with the unrealized gain (loss) on available for sale securities during the three and six months ended June 30, 2017 and June 30, 2016 were as follows:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
Beginning balance	$ (222)	$ 4	$ (212)	$ (473)
Current period changes in fair value before reclassifications, net of tax	36	(122)	26	355
Amounts reclassified from accumulated other comprehensive income (loss), net of tax	—	—	—	—
Total other comprehensive income (loss)	36	(122)	26	355
Ending balance	$ (186)	$ (118)	$ (186)	$ (118)

5. Licensing and Commercialization Agreement with Novartis Pharma AG

In May 2014, the Company entered into a licensing and commercialization agreement with Novartis Pharma AG ("Novartis", and such agreement, the "Novartis Agreement"). Under the Novartis Agreement, the Company granted Novartis exclusive rights under specified patent rights, know-how and trademarks controlled by the Company to manufacture, from bulk API supplied by the Company, standalone Fovista products and products combining Fovista with an anti-VEGF drug to which Novartis has rights in a co-formulated product, for the treatment, prevention, cure or control of any human disease, disorder or condition of the eye, and to develop and commercialize those licensed products in all countries outside of the United States (the "Novartis Territory"). The Company agreed to use commercially reasonable efforts to complete its ongoing pivotal Phase 3 clinical program for Fovista and Novartis agreed to use commercially reasonable efforts to develop a standalone Fovista product and a co-formulated product containing Fovista and an anti-VEGF drug to which Novartis has rights, as well as a pre-filled syringe presentation of such products and to use commercially reasonable efforts, subject to obtaining marketing approval, to commercialize licensed products in the Novartis Territory in accordance with agreed development and marketing plans. Novartis 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 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 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 agreed not to commercialize any product comprising Fovista or any other anti-PDGF product in the ophthalmic field in the Novartis Territory.

The original Novartis Agreement does not specifically address the current status of the Fovista Phase 3 program, wherein the parties are dependent on the OPH1004 data outcome to assess and determine future plans for Fovista. In July 2017, the Company and Novartis entered into a letter agreement to streamline the process and timeline for evaluating data, once it becomes available, from the OPH1004 trial, and, depending on the results from the OPH1004 trial, determining a regulatory strategy in the European Union for Fovista. In the letter agreement, the parties agreed to suspend their affirmative obligations under the Novartis Agreement regarding development, manufacture and commercialization of Fovista products pending receipt of the OPH1004 data and the determination of a regulatory strategy in the European Union. See "Note 13-Subsequent Event" below for a further description of this letter agreement.

Novartis paid the Company a $200.0 million upfront fee upon execution of the Novartis Agreement. Novartis also paid the Company $50.0 million upon the achievement of each of two patient enrollment-based milestones, and $30.0 million upon the achievement of a third, and final, enrollment-based milestone, for an aggregate of $130.0 million. Under the terms of the Novartis Agreement, Novartis is also obligated to pay up to an aggregate of an additional $300.0 million upon achievement of specified regulatory milestones, including marketing approval and reimbursement approval in certain countries in the Novartis Territory. In addition, Novartis has agreed to pay the Company up to an aggregate of an additional $400.0 million if Novartis achieves specified sales milestones in the Novartis Territory. Novartis is also obligated to pay the Company royalties with respect to standalone Fovista products and combination Fovista products that Novartis successfully commercializes. The Company will receive royalties at a mid-thirties percentage of net sales of standalone Fovista products and a royalty of approximately equal value for sales of combination Fovista products. Such royalties are subject to customary deductions, credits, and reductions for lack of patent coverage or market exclusivity. Novartis's obligation to pay such royalties will continue on a licensed product-by-licensed product and country-by-country basis until Novartis's last actual commercial sale of such licensed product in such country. The Company will continue to be responsible for royalties it owes to third parties on sales of Fovista products.

Novartis agreed to pay the Company's manufacturing costs for clinical supplies and the Company's manufacturing costs plus a specified percentage margin for commercial supplies of the bulk API for Fovista that the Company supplies to Novartis. If the Company or Novartis exercises each of their respective rights to obtain access to study data from clinical trials conducted by the other party, the party exercising the option will be obligated to pay the other party's associated past development costs and share with such other party any future associated development costs. If the Company exercises its option to obtain Novartis-controlled rights to develop, manufacture and commercialize any co-formulated Fovista product in the United States, the Company will be obligated to pay a specified percentage of Novartis's associated past development costs and share with Novartis any future associated development costs. The Company and Novartis will also need to negotiate and agree on financial and other terms that would apply to such rights. If the Company exercises its option to obtain Novartis-controlled rights to develop and commercialize a pre-filled syringe product in the United States, the Company will be obligated to either enter into a supply agreement with Novartis under which the Company will pay Novartis its manufacturing cost plus a specified percentage margin for supplies of Fovista products in pre-filled syringes that Novartis supplies to the Company, or 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 retained control over the design and execution of its pivotal Phase 3 clinical program for Fovista and is responsible for funding the costs of that program, subject to Novartis's responsibility to provide Lucentis, an anti-VEGF drug to which Novartis has rights in the Novartis Territory, for use in the Phase 3 trials already initiated and in other Phase 2 and Phase 3 clinical trials in the Novartis Territory initiated following the effective date of the Novartis Agreement. Novartis has 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. 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. As part of the July 2017 letter agreement, the Company permanently waived its right to terminate the Novartis Agreement in the event that the parties are prevented from materially progressing the development or commercialization of Fovista products for a specified period as a result of specified governmental actions. The Company would have been liable to pay Novartis a substantial termination fee in the event that it had exercised this right to terminate the agreement. See "Note 13-Subsequent Event" below for a further description of the letter agreement.

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

achievement, with respect to such product, of the milestones that would have otherwise applied to licensed products under the Novartis Agreement.

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

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

The Novartis Agreement provides that, if the Company elects to terminate the Novartis Agreement because specified governmental actions prevent the parties from materially progressing the development or commercialization of licensed products as described above, the Company will be required to pay a substantial termination fee. The Company has concluded that this termination provision constitutes a contingent event that was unknown at the inception of the agreement. As such, the Company has recorded the $200.0 million upfront payment in deferred revenue, long-term until such time that the contingency related to this termination provision is resolved. In July 2017, the Company permanently waived this termination right. See "Note 13-Subsequent Event" below. The Company believes the enrollment-based milestones and certain 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 marketing approval milestones that may be achieved under the Novartis Agreement are consistent with the definition of a milestone included in ASU 2010-17, *Revenue Recognition—Milestone Method*, and, accordingly, the Company will recognize payments related to the achievement of such milestones, if any, when the applicable milestone is achieved. Factors considered in this determination included scientific and regulatory risks that must be overcome to achieve each milestone, the level of effort and investment required to achieve each milestone, and the monetary value attributed to each milestone.

In May 2014, the Company received an upfront payment of $200.0 million in connection with its entry into the Novartis Agreement, which has not been recorded as revenue due to the Company's right to terminate the agreement and associated obligation to repay the upfront payment under certain circumstances. In each of September 2014 and March 2015, the Company achieved a $50.0 million enrollment-based milestone, and in June 2016, the Company achieved a $30.0 million enrollment-based milestone, for an aggregate total of $130.0 million, under the Novartis Agreement. The Company uses the relative selling price method to allocate arrangement consideration to the Company's performance obligations under the Novartis Agreement. Below is a summary of the components of the Company's collaboration revenue for the three and six months ended June 30, 2017 and June 30, 2016:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
License revenue	$ —	$ 22,937	$ —	$ 22,937
Research and development activity revenue	1,658	5,150	3,316	6,425
API transfer revenue	—	102	—	14,545
Joint operating committee revenue	3	9	7	12
Total collaboration revenue	$ 1,661	$ 28,198	$ 3,323	$ 43,919

As of June 30, 2017, the Company had recorded total deferred revenue of approximately $206.7 million, $200.0 million of which relates to the upfront payment, with the remaining $6.7 million primarily attributable to the Company's on-going performance obligations under the R&D Activity Deliverable.

6. Financing Agreement with Novo A/S

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

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

The royalty payment period covered by the Novo Agreement begins on commercial launch and ends, on a product-by-product and country-by-country basis, on the latest to occur of (i) the 12th anniversary of the commercial launch, (ii) the expiration of certain patent rights and (iii) the expiration of the regulatory exclusivity for each product in each country. The Company's obligations under the Novo agreement are secured by a lien on certain of the Company's intellectual property and other rights related to Fovista and other anti-PDGF products the Company may develop.

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 June 30, 2017, 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.

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

7. Income Taxes

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

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 carry back losses to 2014, the only year in which the Company had taxable income. The Company is currently projecting tax losses in 2017. The Company does not expect to realize its net deferred tax assets recorded as of December 31, 2016 in 2017. The Company expects to carry forward its 2015 and 2016 state tax losses due to various state restrictions on the use of carryback claims. The state NOLs are expected to begin to expire in 2027. Due to the Company's history of losses and lack of other positive evidence to support taxable income after the 2014 tax year, the Company has recorded a valuation allowance against those remaining deferred tax assets that are not expected to be realized. As of December 31, 2016, the Company has federal NOL carryforwards of approximately $187.4 million. These losses are due to expire in 2036.

For the three months and six months ended June 30, 2017, the Company recognized a de minimis amount of income tax provision (benefit) from income taxes. For the three and six months ended June 30, 2016, the Company recorded a benefit from income taxes of $0.3 million and $0.2 million, respectively. The provision and benefit from income taxes recorded in each period of 2017 and 2016 was based upon the Company's estimated federal and state income tax liability for those respective years.

Pursuant to ASC 740, *Income Taxes*, the Company routinely evaluates the likelihood of success if challenged on income tax positions claimed on its income tax returns. During the six months ended June 30, 2017, the Company amended certain state income tax returns to claim a refund for taxes previously paid. These claims may result in refunds to the Company of up to approximately $8.4 million. As the Company believes that the likelihood of its position in claiming the refunds does not rise to the level of more likely than not at this time, the Company has not recorded a current tax receivable for the refund claims as of June 30, 2017.

The Company will continue to evaluate its ability to realize its deferred tax assets on a periodic basis and will adjust such amounts in light of changing facts and circumstances including, but not limited to, future projections of taxable income, tax legislation, rulings by relevant tax authorities, the progress of ongoing tax audits and the regulatory approval of products currently under development. Any additional changes to the valuation allowance recorded on deferred tax assets in the future would impact the Company's income taxes.

8. Fair Value Measurements

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

The Company reviews investments on a periodic basis for other than temporary impairments. This review is subjective as it requires management to evaluate whether an event or change in circumstances has occurred in the period that may have a

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

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

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

- Level 2—inputs to the valuation methodology include quoted prices for a similar asset or liability in an active market or model-derived valuations in which all significant inputs are observable for substantially the full term of the asset or liability. The Company's Level 2 assets consist of investments in investment-grade corporate debt securities.

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

The following table presents, for each of the fair value hierarchy levels required under ASC 820, the Company's assets and liabilities that are measured at fair value on a recurring basis as of June 30, 2017:

	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*	$ 136,634	$ —	$ —
Investments in U.S. Treasury securities	$ 48,134	$ —	$ —
Investments in Corporate debt securities	$ —	$ 7,392	$ —

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, 2016:

	Fair Value Measurement Using		
	Quoted prices in active markets for identical assets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets			
Investments in money market funds*	$ 108,096	$ —	$ —
Investments in U.S. Treasury securities	$ 120,261	$ —	$ —
Investments in Corporate debt securities	$ —	$ 35,087	$ —

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

No transfer of assets between Level 1 and Level 2 of the fair value hierarchy occurred during the three and six months ended June 30, 2017.

9. Stock Option and Compensation Plans

The Company adopted its 2007 Stock Incentive Plan (the "2007 Plan") for employees, non-employee directors and consultants for the purpose of advancing the interests of the Company's stockholders by enhancing its ability to attract, retain and motivate persons who are expected to make important contributions to the Company. The 2007 Plan provided for the granting of stock option awards, RSUs, and other stock-based and cash-based awards. Following the effectiveness of the 2013 Stock Incentive Plan described below in connection with the closing of the Company's initial public offering, the Company is no longer granting additional awards under the 2007 Plan.

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

Annual increases under the evergreen provisions of the 2013 Plan have resulted in the addition of an aggregate of approximately 5,454,000 additional shares to the 2013 Plan, including for 2017, an increase of approximately 1,429,000 shares, or 4% of the total number of shares of the Company's common stock outstanding as of January 1, 2017. As of June 30, 2017, the Company had approximately 1,927,000 shares available for grant under the 2013 Plan.

In April 2016, the board of directors adopted the ESPP pursuant to which the Company may sell up to an aggregate of 1,000,000 shares of common stock. The ESPP was approved by the Company's stockholders in June 2016. The ESPP allows eligible employees to purchase common stock at a price per share equal to 85% of the lower of the fair market value of the common stock at the beginning or end of each six month offering period during the term of the ESPP. The first offering period began in September 2016.

A summary of the stock option activity, weighted average exercise prices, options outstanding and exercisable as of June 30, 2017 is as follows:

	Common Stock Options		Weighted Average Exercise Price
Outstanding, December 31, 2016	3,359	$	39.92
Granted	1,231	$	4.33
Exercised	(20)	$	1.64
Expired or forfeited	(765)	$	41.96
Outstanding, June 30, 2017	3,805	$	28.18
Options exercisable at June 30, 2017			1,831
Weighted average grant date fair value (per share) of options granted during the period		$	3.00

As of June 30, 2017, there were approximately 3,537,000 options outstanding, net of estimated forfeitures, that had vested or are expected to vest. The weighted average exercise price of these options was $28.73 per option; the weighted average remaining contractual life of these options was 7.7 years; and the aggregate intrinsic value of these options was approximately $0.1 million. A summary of the stock options outstanding and exercisable as of June 30, 2017 is as follows:

		As of June 30, 2017			
		Options Outstanding		Options Exercisable	
Range of Exercise Prices	Total Options Outstanding	Weighted Average Remaining Life (Years)	Weighted Average Exercise Price	Number Exercisable	Weighted Average Exercise Price
$0.12-$10.03	1,346	9.0	$ 4.72	181	$ 7.25
$10.04-$20.00	255	6.0	$ 13.46	196	$ 13.51
$20.01-$30.00	127	6.4	$ 25.13	112	$ 25.12
$30.01-$40.00	910	6.1	$ 32.70	779	$ 32.84
$40.01-$55.00	754	8.0	$ 46.43	390	$ 45.83
$55.01-$73.22	413	8.5	$ 71.41	173	$ 70.78
	3,805	7.8	$ 28.18	1,831	$ 34.12
Aggregate Intrinsic Value	$ 83			$ 78	

Cash proceeds from, and the aggregate intrinsic value of, stock options exercised during the three and six months ended June 30, 2017 and 2016, respectively, were as follows:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
Cash proceeds from options exercised	$ 15	$ 3,039	$ 46	$ 3,808
Aggregate intrinsic value of options exercised	$ 6	$ 5,206	$ 43	$ 7,421

In connection with stock option awards granted to employees, the Company recognized approximately $3.6 million and $5.5 million in share-based compensation expense during the three months ended June 30, 2017 and 2016, respectively, net of expected forfeitures. In connection with stock option awards granted to employees, the Company recognized approximately $8.0 million and $11.6 million in share-based compensation expense during the six months ended June 30, 2017 and 2016, respectively, net of expected forfeitures. As of June 30, 2017, there were approximately $19.6 million of unrecognized compensation costs, net of estimated forfeitures, related to stock option awards granted to employees, which are expected to be recognized over a remaining weighted average period of 2.3 years.

In connection with stock option awards granted to consultants, the Company recognized approximately $0.1 million and $0.5 million in share-based compensation expense during the three months ended June 30, 2017 and 2016, respectively, net of expected forfeitures. In connection with stock option awards granted to consultants, the Company recognized approximately $0.2 million and $0.9 million in share-based compensation expense during the six months ended June 30, 2017 and 2016, respectively, net of expected forfeitures. As of June 30, 2017, there were approximately $0.3 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 2.1 years.

The following table presents a summary of the Company's outstanding RSU awards granted as of June 30, 2017:

	Restricted Stock Units	Weighted Average Grant-Date Fair Value
Outstanding, December 31, 2016	721	$ 55.33
Awarded	248	$ 4.42
Vested	(174)	$ 31.75
Forfeited	(260)	$ 50.32
Outstanding, June 30, 2017	535	$ 38.03

As of June 30, 2017, there were approximately 365,000 RSUs outstanding, net of estimated forfeitures, that are expected to vest. The weighted average fair value of these RSUs was $31.83 per share; and the aggregate intrinsic value of these RSUs was approximately $0.9 million.

In connection with RSUs granted to employees, the Company recognized approximately $1.3 million and $2.3 million in share-based compensation expense during the three months ended June 30, 2017 and 2016, respectively, net of expected forfeitures. In connection with RSUs granted to employees, the Company recognized approximately $2.6 million and $4.1 million in share-based compensation expense during the six months ended June 30, 2017 and 2016, respectively, net of expected forfeitures. As of June 30, 2017, there were approximately $8.9 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 2.4 years.

In connection with RSUs granted to consultants, the Company recognized a de minimis amount of share-based compensation expense during the three months ended June 30, 2017, net of expected forfeitures. In connection with RSUs granted to consultants, the Company recognized $0.1 million of share-based compensation expense during the six months ended June 30, 2017, net of expected forfeitures. There were no RSUs granted to consultants during the three and six months ended June 30, 2016. As of June 30, 2017, there were approximately $0.1 million of unrecognized compensation costs, net of estimated forfeitures, related to RSUs granted to consultants, which are expected to be recognized over a remaining weighted average period of 2.2 years.

In connection with the ESPP made available to employees, the Company recognized a de minimis amount of share-based compensation expense during the three months ended June 30, 2017. In connection with the ESPP made available to employees, the Company recognized $0.1 million of share-based compensation expense during the six months ended June 30, 2017. The Company did not recognize any share-based compensation expense during the three and six months ended June 30, 2016. As of June 30, 2017, there was a de minimis amount of unrecognized compensation costs, net of estimated forfeitures, related to the ESPP, which are expected to be recognized over 0.3 years. Additionally, there were 4,746 shares of common stock issued under the ESPP during the six months ended June 30, 2017. There were no shares of common stock issued under the ESPP plan during the six months ended June 30, 2016. As of June 30, 2017, 995,254 shares were available for future purchases under the ESPP.

10. Property and Equipment

Property and equipment as of June 30, 2017 and December 31, 2016 were as follows:

	Useful Life (Years)	June 30, 2017	December 31, 2016
Manufacturing and clinical equipment	7 - 10	$ 412	$ 617
Computer, software and other office equipment	5	1,711	1,711
Furniture and fixtures	1 - 7	774	774
Leasehold improvements	1 - 5	1,835	1,835
		4,732	4,937
Accumulated depreciation		(2,844)	(1,656)
Property and equipment, net		$ 1,888	$ 3,281

For the three and six months ended June 30, 2017, depreciation expense was $0.6 million and $1.4 million, respectively. For the three and six months ended June 30, 2016, depreciation expense was $0.2 million and $0.4 million, respectively.

11. Commitments and Contingencies

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

- Under the Company's divestiture agreement with OSI (Eyetech), Inc., which agreement is now held by OSI Pharmaceuticals, LLC., or OSI Pharmaceuticals, a subsidiary of Astellas US, LLC, for rights to particular anti-PDGF aptamers, including Fovista, the Company is obligated to pay to OSI Pharmaceuticals future one-time

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

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

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

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

- Under the Novo Agreement, with respect to Fovista, the Company will be obligated to pay Novo A/S a mid-single-digit percentage royalty based on worldwide sales of Fovista. See "Note 6—Financing Agreement with Novo A/S" above for further information about Novo Agreement.

The Company also has 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 the Company without cause, occur.

In addition, in the course of normal business operations, the Company has agreements with contract service providers to assist in the performance of the Company's research and development and manufacturing activities. Expenditures to CROs and CMOs represent significant costs in clinical development. Subject to required notice periods and the Company's obligations under binding purchase orders, the Company can elect to discontinue the work under these agreements at any time.

Legal Proceedings

On January 11, 2017, a putative class action lawsuit was filed against the Company and certain of its current and former executive officers in the United States District Court for the Southern District of New York, captioned Frank Micholle v. Ophthotech Corporation, et al., No. 1:17-cv-00210. The complaint purports to be brought on behalf of shareholders who purchased the Company's common stock between May 11, 2015 and December 12, 2016. The complaint generally alleges that the Company and certain of its officers violated Sections 10(b) and/or 20(a) of the Securities Exchange Act of 1934 and Rule 10b-5 promulgated thereunder by making allegedly false and/or misleading statements concerning the prospects of the Company's Phase 3 trials for Fovista in combination with anti-VEGF drugs for the treatment of wet AMD. The complaint seeks equitable and/or injunctive relief, unspecified damages, attorneys' fees, and other costs.

On March 9, 2017, a second putative class action lawsuit was filed against the Company and the same group of its current and former executive officers in the United States District Court for the Southern District of New York, captioned Wasson v. Ophthotech Corporation, et al., No. 1:17-cv-01758. The complaint purports to be brought on behalf of shareholders

who purchased the Company's common stock between May 11, 2015 and December 9, 2016. The allegations made in the complaint are similar to those made in the Micholle complaint. Putative lead plaintiffs in the Micholle action have moved to consolidate the Micholle and Wasson actions.

On May 30, 2017, a shareholder derivative action was filed against the members of the Company's Board of Directors in the United States District Court for the Southern District of New York, captioned Etelmendorf v. Bolte, et al., No. 1:17-cv-04042. The complaint alleges that defendants breached their fiduciary duties to the Company by causing or permitting the Company to make allegedly false and/or misleading statements concerning the prospects of the Company's Phase 3 trials for Fovista in combination with anti-VEGF drugs for the treatment of wet AMD, and by approving certain executive compensation. The complaint also alleges that defendants were unjustly enriched as a result of the alleged conduct. The complaint purports to seek unspecified damages on behalf of the Company, as well as an order directing the Company to reform and comply with its governance obligations, attorneys' fees, and other costs.

The Company denies any allegations of wrongdoing and intends to vigorously defend against these lawsuits. The Company is unable, however, to predict the outcome of these matters at this time. Moreover, any conclusion of this matter in a manner adverse to the Company and for which it incurs substantial costs or damages not covered by the Company's directors' and officers' liability insurance would have a material adverse effect on its financial condition and business. In addition, the litigation could adversely impact the Company's reputation and divert management's attention and resources from other priorities, including the execution of business plans and strategies that are important to the Company's ability to grow its business, any of which could have a material adverse effect on the Company's business.

12. Restructuring Activities

In December 2016, the Company announced its intention to implement a reduction in personnel to focus on an updated business plan. In January 2017, the Board of Directors approved a plan to implement a reduction in personnel involving approximately 80% of the Company's workforce based on the number of employees at the time the plan was approved. During the first six months of 2017, the Company's workforce has been reduced by 98 employees in connection with the reduction in personnel and through natural attrition, and the Company expects the reduction of the additional 20 affected employees will be substantially completed during the third quarter of 2017. In connection with such reduction in personnel, the Company estimates that it will incur approximately $13.4 million of pre-tax charges through the third quarter of 2017, of which approximately $12.5 million in the aggregate is expected to result in cash expenditures. These pre-tax charges relate to (a) expected severance, stock compensation and other employee costs of approximately $11.3 million and (b) expected lease termination costs of approximately $2.1 million. As of June 30, 2017, the Company's cash expenditures related to such reduction in personnel totaled $8.0 million.

In connection with the reduction in personnel, the Company recognized approximately $1.8 million and $10.5 million of severance, stock compensation and other employee costs for the three and six months ended June 30, 2017, of which $1.1 million and $5.9 million were recorded in "Research and development" expense and $0.7 million and $4.6 million were recorded in "General and administrative" expense in the Company's Statements of Operations.

As of June 30, 2017, the Company's accrual balance for severance and benefit costs was $2.0 million which was recorded in "Accounts payable and accrued expenses" in the Company's Balance Sheet. The severance and other employee cost accruals as of June 30, 2017 are expected to be paid through to the first half of 2018.

The following is a reconciliation of the severance-related accrual activity for the six months ended June 30, 2017:

	Accrued Severance and Other Employee Costs
Beginning Balance	$ —
Accrued restructuring expenses	10,006
Payments	(8,039)
Ending Balance	$ 1,967

On January 26, 2017, the Company issued a notice of termination under the Lease Agreement, dated as of September 30, 2007, between the Company and One Penn Plaza LLC, as previously supplemented and amended (as so supplemented and amended, the "Lease") for office space at One Penn Plaza in New York, New York. The termination of the Lease triggered an early termination payment by the Company of approximately $0.9 million and will be effective in

January 2018, through which time the Company will be responsible for paying continuing rental fees, as well as taxes, operating expenses and utility and other charges related to the leased premises.

On January 26, 2017, the Company issued a notice of termination under the Sublease Agreement between the Company and Otsuka America Pharmaceutical, Inc. (the "Sublease") for office space at One University Square, Princeton, New Jersey. The termination of the Sublease triggered an early termination payment by the Company of approximately $1.2 million and will be effective in February 2018, through which time the Company will be responsible for paying continuing rental fees, as well as taxes, operating expenses and utility and other charges related to the subleased premises.

On January 26, 2017, the Company issued a notice of termination under its Office Lease Agreement between the Company and PSN Partners, L.P. (the "Office Lease") for office space in Palmer Square in Princeton, New Jersey. The termination of the Office Lease did not trigger any early termination payment and will be effective in October 2017, through which time the Company will be responsible for paying continuing rental fees.

During January 2017, the Company made the early termination payments as described above and recognized $2.1 million of additional facilities costs which were recorded in "General and administrative" expense in the Company's Statements of Operations.

13. Subsequent Event

On July 3, 2017, the Company and Novartis entered into a letter agreement with respect to the Novartis Agreement. See "Note 5 - Licensing and Commercialization Agreement" for further information about the Novartis Agreement. Pursuant to the letter agreement, the Company and Novartis have agreed to a process and timeline for evaluating data, once it becomes available, from the Company's Phase 3 OPH1004 trial, and, depending on the results from the OPH1004 trial, determining a regulatory strategy in the European Union and continuing efforts under the Novartis Agreement to develop and commercialize Fovista. Pursuant to the letter agreement, the Company and Novartis have agreed to suspend their affirmative obligations under the Novartis Agreement regarding development, manufacture and commercialization of Fovista products pending receipt of the OPH1004 data and the determination of a regulatory strategy in the European Union. The letter agreement also provides Novartis with a shorter notice period in the event Novartis determines to terminate the Novartis Agreement in certain circumstances and provides for a process for the parties to determine the scope and funding for additional clinical trials, if any, required for regulatory approval of Fovista. If the Company and Novartis do not otherwise agree as to the funding for any additional clinical trials, each party will be required to fund fifty percent (50%) of the cost and expense of such clinical trials. Under the letter agreement, the Company permanently waived its right to terminate the Novartis Agreement under Section 11.06 thereof in the event that the parties are prevented from materially progressing the development or commercialization of Fovista products for a specified period as a result of specified governmental actions. The Company would have been liable to pay Novartis a substantial termination fee in the event that it had exercised its rights under Section 11.06. In addition, the letter agreement provides Novartis with a fully paid-up, royalty-free license to use data from the Lucentis monotherapy arms of the Company's Phase 2b OPH1001 trial and Phase 3 OPH1002 and OPH1003 trials in the Novartis Territory in connection with the development, manufacturing and commercialization of Novartis-controlled anti-VEGF products. The Lucentis study data license will continue until the fifth anniversary of the letter agreement or the date the Novartis Agreement expires or terminates, whichever is later.

The Company is currently estimating the deferred revenue to be recognized as a result of this letter agreement and expects to recognize a majority of the revenue during the third quarter of 2017.

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our financial statements and the notes to those financial statements appearing elsewhere in this Quarterly Report on Form 10-Q and the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the year ended December 31, 2016 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 28, 2017. 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

Updated Business Plan

We are a biopharmaceutical company specializing in the development of novel therapeutics to treat ophthalmic diseases, with a focus on orphan and age-related retinal diseases. We currently have two product candidates, Zimura® (avacincaptad pegol), an anti-complement factor C5 aptamer, and Fovista® (pegpleranib), an anti-platelet derived growth factor, or PDGF, aptamer. Prior to 2017, the Company's primary focus was on developing Fovista and Zimura for various types of age-related macular degeneration, or AMD, which is a disorder of the central portion of the retina, known as the macula, that may result in blindness. In December 2016, we announced that two of our three pivotal, Phase 3 clinical trials for Fovista in combination with the anti-vascular endothelial growth factor, or anti-VEGF, drug Lucentis® (ranibizumab) for the treatment of wet AMD, failed to meet their primary endpoint. We announced in early 2017 that we had engaged a financial advisor to assist us in reviewing our strategic alternatives, including identifying potential business development opportunities. Also beginning in early 2017, we undertook a reassessment of our development plans for Zimura and Fovista, which included an evaluation of the scientific rationale for potentially developing these product candidates in one or more other ophthalmic indications for which there is a high unmet need.

On July 26, 2017, we announced that we are pursuing a strategy to leverage our clinical experience and retina expertise to identify and develop therapies to treat multiple ophthalmic orphan diseases for which there are limited or no treatment options available. We believe that there are advantages of orphan drug development, including regulatory exclusivity, the potential for smaller clinical trials and the potential for an accelerated development timeline of the orphan retinal indications. In parallel, we are continuing our on-going programs in age-related retinal diseases. We believe that by leveraging our ophthalmic expertise and by focusing on orphan eye diseases together with our on-going programs in age-related retinal diseases, we will have multiple potential opportunities to bring ophthalmic therapeutics to market. We also are continuing our business development efforts to identify and potentially obtain rights to additional products, product candidates and technologies that would complement our strategic goals as well as other compelling ophthalmology opportunities.

Zimura

As part of our strategic review process, we reassessed our development plans for our product candidate Zimura. We applied the same rigor in reassessing our development plans for Zimura as we used in performing diligence on third party opportunities considered as part of our strategic review process. Our Zimura reassessment included an extensive review of scientific literature regarding the role of complement in ophthalmic diseases. Upon the conclusion of this reassessment, we determined to accelerate our development of Zimura in additional ophthalmic indications. Our ongoing and planned clinical studies for Zimura are:

- a planned randomized, controlled clinical trial evaluating the safety and efficacy of Zimura monotherapy for the treatment of Stargardt disease, an inherited retinal orphan disease causing vision loss during childhood or adolescence for which patients have no approved treatment. This trial is scheduled to start by the end of 2017 and we plan to work with the U.S. Food and Drug Administration, or FDA, to address the regulatory pathway for Zimura for the treatment of Stargardt disease;

- a planned, open-label Phase 2a clinical trial of Zimura in combination with an anti-VEGF drug for the treatment of wet AMD in patients who have not previously been treated with anti-VEGF drugs, or treatment-naïve patients. This trial is scheduled to start by the end of 2017. We are ceasing to enroll additional patients in our ongoing open label Phase 2a clinical trial of Zimura in wet AMD patients who have been previously treated with anti-VEGF drugs, or treatment-experienced patients;

- a planned open-label Phase 2a clinical trial of Zimura in combination with an anti-VEGF drug for the treatment of idiopathic polypoidal choroidal vasculopathy, or IPCV, an age-related retinal disease involving the choroidal vasculature characterized by the presence of polypoidal lesions, which leads to vision loss. This trial is scheduled to start by the end of 2017;

- an ongoing randomized, double-masked, controlled Phase 2/3 clinical trial to evaluate the safety and efficacy of Zimura monotherapy in patients with geographic atrophy, or GA, a form of dry AMD; and

- a planned Phase 2a clinical trial of Zimura monotherapy in non-infections intermediate and posterior uveitis, a rare inflammatory disease of the back of the eye, which is scheduled to be initiated during 2018.

We plan to further reassess our development strategy for Zimura in treating GA in light of the results of a competitor's Phase 3 clinical trials of a complement inhibitor being studied for the treatment of GA. These results are expected during the second half of 2017.

Fovista

In December 2016, we announced initial, top-line data from two pivotal clinical trials, which we refer to as OPH1002 and OPH1003, evaluating the safety and efficacy of 1.5mg of Fovista administered in combination with monthly 0.5mg Lucentis® (ranibizumab) anti-VEGF therapy compared to monthly 0.5mg Lucentis monotherapy for the treatment of wet AMD. The pre-specified primary endpoint of mean change in visual acuity at 12 months was not achieved for either OPH1002 or OPH1003. Moreover, we did not observe any clinically meaningful visual benefit when 1.5mg of Fovista was added to a monthly regimen of 0.5mg Lucentis therapy for any subgroup of patients that we have analyzed from the OPH1002 and OPH1003 trials, including subgroups based on baseline visual acuity, baseline lesion size or the baseline amount of the classic component of choroidal neovascularization, or CNV. Following the December 2016 data announcement, we subsequently stopped treating patients in, and terminated, both the OPH1002 and OPH1003 trials. In light of the data from the OPH1002 and OPH1003 trials, we also stopped treating patients in our additional Phase 2 clinical trials that were evaluating the potential additional benefits of Fovista administered in combination with anti-VEGF drugs in wet AMD patients, which we previously referred to collectively as the Fovista Expansion Studies.

As part of our strategic review process, we also reassessed our Fovista program and explored the scientific rationale for Fovista as a potential treatment in orphan indications. During 2016, the National Eye Institute commenced a Phase 1/2 clinical trial studying the potential role of Fovista in combination with Lucentis for the treatment of retinal capillary hemangiomas associated with the orphan disease Von-Hippel-Lindau Syndrome. This trial remains ongoing with initial data expected to be available during 2018. We are also planning a pre-clinical program for Fovista in retinoblastoma, a rare cancer of the eye in children. Pre-clinical research has shown that the PDGF signaling pathway may play a role in retinoblastoma. Our strategy for our Fovista wet AMD development program will be primarily determined by the initial, top-line data from OPH1004 and in the context of our other two failed Phase 3 clinical trials. OPH1004, a Phase 3 clinical trial, is evaluating the safety and efficacy of 1.5 mg of Fovista administered in combination with 2.0mg Eylea® (aflibercept) or 1.25mg Avastin® (bevacizumab) anti-VEGF therapy compared to 2.0mg Eylea or 1.25mg Avastin monotherapy for the treatment of wet AMD. Data from our OPH1004 trial are expected by the end of the third quarter of 2017. We believe that the failure of OPH1002 and OPH1003 to show any clinically meaningful visual benefit in adding 1.5mg of Fovista to a monthly regimen of 0.5mg of Lucentis, and the recent failure of a competitor's Phase 2 trial investigating the combination of a PDGF inhibitor and a VEGF inhibitor, are indicative of a low likelihood of success for OPH1004. As a result of our ongoing reassessment of our development programs and potential business development opportunities, we may modify, expand or terminate some or all of our development programs or clinical trials at any time. We generally expect that we will not engage in internal early stage research and drug discovery and will thus avoid the related costs and risks of these activities.

Age-related retinal diseases

There are two forms of AMD, dry AMD and wet AMD. Dry AMD can progress to wet AMD. Although dry AMD is the most common form of AMD, there are no therapies approved by the FDA or European Medicines Agency, or EMA, to treat this condition. We are currently developing our product candidate Zimura as a monotherapy for the treatment of GA, a form of dry AMD, as well as in combination with an anti-VEGF drug for the treatment of wet AMD. Zimura is an inhibitor of complement factor C5, a protein that is associated with complement mediated inflammation and cell damage, which we believe may be involved in the development and progression of dry AMD and wet AMD. We have completed a multicenter, uncontrolled, open-label Phase 1/2a clinical trial of Zimura monotherapy for the treatment of GA, a multicenter, uncontrolled, open-label, ascending dose and parallel group Phase 1/2a clinical trial of Zimura administered in combination with Lucentis for the treatment of wet AMD and a very small, uncontrolled, open-label Phase 2a clinical trial investigating Zimura's potential role when administered in combination with an anti-VEGF drug for the treatment of IPCV, in patients who do not respond adequately to treatment with anti-VEGF monotherapy or for whom anti-VEGF monotherapy fails. A recent peer-reviewed publication cited the role of anti-VEGF therapy in complement activation. We believe that supplementing anti-VEGF therapy with an anti-complement such as Zimura in wet AMD may have the potential to further enhance the efficacy of anti-VEGF monotherapy and decrease its potentially unwanted side effects.

Stargardt Disease

Stargardt disease is a rare, inherited genetic disease that causes progressive damage to the macula and retina and loss of central vision in children and adolescents. The autosomal recessive form of the disease is referred to as STGD1. Multiple sources, including the National Eye Institute and Genetics Home Reference, both of which are affiliated with the U.S. National Institutes of Health, estimate the prevalence of Stargardt disease to be between 1 in 8,000 and 1 in 10,000, implying an overall prevalence of 32,000 to 41,000 affected persons in the United States, with, we believe, at least as many affected persons in Europe. There are currently no therapies approved by the FDA or EMA to treat Stargardt disease. The FDA has recognized Stargardt as an orphan disease, with several treatments in development having received orphan product designation from the FDA.

Visual Cycle Waste Accumulation and MAC Accumulation - The Potential for Zimura

STGD1 is caused by mutations to the ABCA4 gene, which is responsible for making a protein that helps to clear byproducts resulting from the visual cycle from inside photoreceptors in the eye. With a defective copy of the ABCA4 protein, these waste byproducts accumulate in the retinal pigment epithelium, or RPE, which is a layer of cells within the retina on which photoreceptors are dependent. Waste byproducts that accumulate in the RPE are referred to as bisretinoids. We believe that the accumulation of bisretinoids in RPE cells leads to activation of the complement system. The final product of all three complement pathways is the membrane-attack complex, or MAC. In RPE cells, MAC is normally cleared by lysosomes, which are organelles within cells responsible for waste degradation and disposal. Bisretinoid accumulation leads to lysosomal dysfunction, potentially preventing the clearance of MAC. MAC accumulation also negatively impacts energy production mitochondria. We believe that bisretinoid and MAC accumulation in RPE cells leads to RPE cell deterioration, resulting in the loss of photoreceptor cells and decreases in vision over time.

In April 2017, *Proceedings of the National Academy of Sciences*, or PNAS, published a study reporting on the effects of complement modulation in the RPE of a mouse model of Stargardt disease. Injection of a recombinant adeno-associated virus containing the coding sequence for a protein that inhibits complement activation, Crry, into an albino ABCA4 mutant mouse model led to a two-fold reduction in the accumulation of bisretinoids and a 30% increase in the number of photoreceptor nuclei. The study findings indicate that the inhibition of complement activation may lead to healthier RPE cells, which in turn are better capable of processing bisretinoids in the albino ABCA4 mutant mice when compared to untreated mice. Research performed at Duke University and published in a paper appearing in 2013 in *Investigative Ophthalmology & Visual Science* demonstrated that cell damage resulting from the combination of complement activation and visual cycle waste accumulation was far more damaging than either component individually in RPE cells *in vitro*. When complement factor C5 was blocked, there was a significant improvement in RPE cell viability *in vitro*. Based on the data from these *in vitro* and *in vivo* experiments, we believe molecules involved in inhibition or regulation of complement activation and MAC accumulation become prime targets for therapeutic intervention in STGD1.

Zimura is an inhibitor of complement factor C5. Uninhibited, the cleavage of C5 results in the formation of downstream complement molecules, including MAC. We believe that MAC may be responsible for the harmful effects of complement activation and that therefore complement factor C5 is a promising target for inhibition. We believe that the pre-clinical studies described above provide a strong rationale to explore the potential effect of Zimura in STGD1 patients.

We plan to initiate a randomized, controlled clinical trial evaluating the safety and efficacy of Zimura monotherapy for the treatment of Stargardt disease by the end of 2017. We also plan to work with the FDA to address the regulatory pathway for our Stargardt program. In July 2017, we entered into an agreement with Foundation Fighting Blindness, or FFB, a highly-distinguished organization recognized for its scientific commitment to orphan inherited retinal degenerative diseases with an established network of scientists and a robust patient registry. FFB has recently completed the largest natural history in Stargardt disease to date which is referred to as ProgStar. We have engaged FFB to provide us with information from its publicly available ProgStar study which we plan to use in the design and implementation of our planned clinical trial of Zimura for Stargardt disease.

On-going business development activities

In early 2017, we engaged Leerink Partners, a financial advisor, and initiated a plan to review our strategic alternatives in order to maximize shareholder value following the failure of two of our three pivotal Fovista trials. Without limiting any option, the principal focus of this plan, based on our deep expertise and experience in ophthalmology, has been to actively explore obtaining rights to additional products, product candidates and technologies to treat ophthalmic diseases, particularly those of the back of the eye. We reviewed a large number of assets and technology platforms during the first half of 2017 and are actively continuing to review assets or technology platforms that would complement our strategic goals in addition to other compelling ophthalmology opportunities.

Leerink Partners continues to assist our management and our board of directors in evaluating our strategic alternatives. We, with Leerink's assistance, have considered multiple opportunities over the last several months, including in-licensing, obtaining rights to products, product candidates or technologies, acquisitions, mergers and reverse mergers. We are committed to being opportunistic and will reconsider new compelling opportunities if and as they emerge.

As of June 30, 2017, we had $196.4 million in cash, cash equivalents, and marketable securities, of which approximately $20 million to $35 million is committed to the wind-down of OPH1002 and OPH1003 and the Fovista Expansion Studies, implementation of a previously announced reduction in personnel and related costs, cancellation fees related to manufacturing commitments, and obtaining initial, top-line data for the OPH1004 trial by the end of the third quarter of 2017.

Novartis Agreement

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

The original Novartis Agreement does not specifically address the current status of the Fovista Phase 3 program, wherein the parties are dependent on the OPH1004 data outcome to assess and determine future plans for Fovista. In July 2017, we and Novartis entered into a letter agreement to streamline the process and timeline for evaluating data, once it becomes available, from the OPH1004 trial, and, depending on the results from the OPH1004 trial, determining a regulatory strategy in the European Union for Fovista. In the letter agreement, the parties agreed to suspend their affirmative obligations under the Novartis Agreement regarding development, manufacture and commercialization of Fovista products pending receipt of the OPH1004 data and the determination of a regulatory strategy in the European Union.

Novartis paid us a $200.0 million upfront fee upon execution of the Novartis Agreement. Novartis also paid us $50.0 million upon the achievement of each of two patient enrollment-based milestones, and $30.0 million upon the achievement of a third, and final, enrollment-based milestone, for an aggregate of $130.0 million. Under the terms of the Novartis Agreement, Novartis is also obligated to pay us up to an aggregate of an additional $300.0 million upon achievement of specified regulatory milestones, including marketing approval and reimbursement approval in certain countries in the Novartis Territory. In addition, Novartis has agreed to pay us up to an aggregate of an additional $400.0 million if Novartis achieves specified sales milestones in the Novartis Territory. Novartis also is obligated to pay us royalties with respect to standalone Fovista products and combination Fovista products that Novartis successfully commercializes. We will receive royalties at a mid-thirties percentage of net sales of standalone Fovista products and a royalty of approximately equal value for sales of combination Fovista products. Such royalties are subject to customary deductions, credits, and reductions for lack of patent coverage or market exclusivity. Novartis's obligation to pay such royalties will continue on a licensed product-by-licensed product and country-by-country basis until Novartis's last actual commercial sale of such licensed product in such country. We will continue to be responsible for royalties we owe to third parties on sales of Fovista products.

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

responsible.

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 June 30, 2017, we had an accumulated deficit of $664.3 million. Our net loss was $65.3 million for the six months ended June 30, 2017, and $193.4 million for the year ended December 31, 2016, and we expect to continue to incur significant operating losses for the foreseeable 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, $50.0 million upon the achievement of each of two patient enrollment-based milestones, and $30.0 million upon the achievement of a third, and final, enrollment-based milestone, for an aggregate of $130.0 million.

We expect to continue to incur research and development expenses as we continue our Zimura and Fovista development programs, including as we initiate new clinical trials and continue the continue the OPH1004 trial, for which we expect initial, top-line data to be available by the end of the third quarter of 2017, and as we wind down the OPH1002 and OPH1003 trials and the Fovista Expansion Studies. Due to the terminations of the OPH1002 and OPH1003 trials and the Fovista Expansion Studies, our overall research and development expenses have decreased significantly compared to prior years. However, as we commence new clinical trials for Zimura, pre-clinical or clinical development programs for Fovista, or any new development efforts in relation to additional product candidates we may in-license or acquire as we pursue our updated business plan, we expect that our overall research and development expenses will begin to increase from the current level of expenditure.

We plan to continue to reassess our existing Zimura and Fovista development programs throughout 2017. We expect that our reassessment of our Fovista development program for the treatment of wet AMD will be primarily determined by the initial, top-line data from OPH1004 and in the context of our other two failed Phase 3 clinical trials. We expect that our reassessment of our Zimura development program for GA may be particularly affected by the results of a competitor's Phase 3 clinical trials of a complement inhibitor being studied for the treatment of GA. Data from our OPH1004 trial are expected by the end of the third quarter of 2017 and data from our competitor's Phase 3 trials for the treatment of GA are expected during the second half of 2017. As a result of our ongoing assessment of our development programs, we may modify, expand or terminate some or all of our development programs or clinical trials at any time. The outcome of these reassessments, as well as the progress of our plans to potentially acquire additional products, product candidates or technologies will determine whether and to what extent we will continue to incur research and development costs for each of our development programs going forward.

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, any of our product candidates, which, if we are successful, may take at least several years. 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 are successful in our pursuit to acquire or in-license and subsequently develop additional product candidates or technologies. We may need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

Financial Operations Overview

Revenue

Prior to 2014, we had not generated any revenue. In May 2014, we received an upfront payment of $200.0 million in connection with our entry into the Novartis Agreement, which has not been recorded as revenue due to our right to terminate the agreement and associated obligation to repay the upfront payment under certain circumstances. In each of September 2014 and March 2015, we achieved a $50.0 million enrollment-based milestone, and in June 2016, we achieved a $30.0 million enrollment-based milestone, for an aggregate total of $130.0 million, under the Novartis Agreement. We use the relative selling price method to allocate arrangement consideration to our performance obligations under the Novartis Agreement. Below is a summary of the components of our collaboration revenue for the three and six months ended June 30, 2017 and 2016:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
	(in thousands)			
License revenue	$ —	$ 22,937	$ —	$ 22,937
Research and development activity revenue	1,658	5,150	3,316	6,425
API transfer revenue	—	102	—	14,545
Joint operating committee revenue	3	9	7	12
Total collaboration revenue	$ 1,661	$ 28,198	$ 3,323	$ 43,919

In the future, we may generate additional revenue from a combination of product sales and license fees, milestone payments, research and development activity-related payments, payments for manufactured material and royalties from our commercialization partners, such as Novartis for Fovista outside the United States. 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. If we fail to complete the development of Zimura, Fovista or other product candidates we may develop in a timely manner or obtain regulatory approval for them, our ability to generate future revenue and our results of operations and financial position, would be materially adversely affected.

Research and Development Expenses

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

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

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

All research and development expenses are charged to operations as incurred in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic, or ASC, 730, *Research and Development*. We account for non-refundable advance payments for goods and services that will be used in future research and development activities as expenses when the service has been performed or when the goods have been received, rather than when the payment is made. To date, the large majority of our research and development activity has been related to Fovista and Zimura. 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 six months ended June 30, 2017 and 2016:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
	(in thousands)			
Fovista	$ 6,382	$ 29,924	$ 16,822	$ 54,535
Zimura	2,644	1,048	8,276	2,314
Personnel-related	3,348	8,019	14,300	13,217
Share-based compensation	2,897	6,383	7,047	11,068
Other	386	2,888	1,191	4,898
	$ 15,657	$ 48,262	$ 47,636	$ 86,032

We expect to continue to incur research and development expenses as we continue our Zimura and Fovista development programs, including as we initiate new clinical trials and continue the OPH1004 trial, for which we expect initial, top-line data to be available by the end of the third quarter of 2017, and as we wind down the OPH1002 and OPH1003 trials and the Fovista Expansion Studies. However, due to the terminations of the OPH1002 and OPH1003 trials and the Fovista Expansion Studies, we expect our overall research and development expenses to decrease significantly compared to prior years until such time as we initiate our planned additional clinical trials for Zimura and, potentially, undertake additional development programs in relation to Fovista or additional product candidates we may in-license or acquire.

Our expenses may exceed our expectations if we experience delays, including with respect to the availability of drug product for our clinical trials, if we experience any unforeseen issue in our ongoing clinical trials or if we further expand the scope of our clinical trials and programs. Our costs may also exceed our expectations for other reasons, for example, if we experience issues with process development, the scale-up of manufacturing activities or activities to enable and qualify second source suppliers or if we decide to increase licensing or preclinical research and development activities.

The future development of Zimura and Fovista is highly uncertain. If the results from the OPH1004 trial are favorable and we determine to pursue further development of Fovista for use in combination with anti-VEGF drugs for the treatment of wet AMD, we would likely need to conduct one or more further pivotal, clinical trials for Fovista to support potential marketing approval, which would be time-consuming and expensive. Furthermore, we expect the clinical development for Zimura will continue for at least the next several years. At this time, we cannot reasonably estimate the remaining costs necessary to complete the clinical development of either Zimura or Fovista, complete process development and manufacturing scale-up and validation activities associated with Zimura and potentially seek marketing approval for Zimura and Fovista, 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 subject to 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 our product candidates 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 a product candidate or if we experience significant delays in enrollment in any clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development.

See the "Liquidity and Capital Resources" section on page 41 of this Quarterly Report on Form 10-Q for more information regarding our current and future financial resources and our expectations regarding our research and development expenses and funding requirements.

General and Administrative Expenses

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

We anticipate that our general and administrative expenses will decrease in future periods as a result of a reduction in personnel to focus on an updated business plan involving a total expected workforce of approximately 38 employees. We substantially completed the reduction in personnel during the first and second quarters of 2017 as part of implementing our updated business plan.

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 research and development expenses, revenue recognition, share-based compensation and income taxes described in greater detail below. We base our estimates on our limited historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

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

Accrued Research and Development Expenses

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

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

Revenue Recognition—Collaboration Revenue

Prior to 2014, we had not generated any revenue. In May 2014, we received an upfront payment of $200.0 million in connection with our entry into the Novartis Agreement, which has not been recorded as revenue due to our right to terminate the agreement and associated obligation to repay the upfront payment under certain circumstances. In each of September 2014 and March 2015, we achieved a $50.0 million enrollment-based milestone, and in June 2016, we achieved a $30.0 million enrollment-based milestone, for an aggregate total of $130.0 million, under the Novartis Agreement. We use the relative selling price method to allocate arrangement consideration to our performance obligations under the Novartis Agreement. Below is a summary of the components of our collaboration revenue for the three and six months ended June 30, 2017 and 2016:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
	(in thousands)			
License revenue	$ —	$ 22,937	$ —	$ 22,937
Research and development activity revenue	1,658	5,150	3,316	6,425
API transfer revenue	—	102	—	14,545
Joint operating committee revenue	3	9	7	12
Total collaboration revenue	$ 1,661	$ 28,198	$ 3,323	$ 43,919

In July 2017, we entered into a letter agreement with Novartis. In this letter agreement, we permanently waived our right to terminate the Novartis Agreement in the event that the parties are prevented from materially progressing the development or commercialization of Fovista products for a specified period as a result of specified governmental actions. We would have been liable to pay Novartis a substantial termination fee in the event that we had exercised this right to terminate the agreement. As a result of this waiver, during the third quarter of 2017, we expect to recognize a majority of the collaboration revenue we previously deferred upon receipt of the upfront payment.

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 certain of our 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 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 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 that is subject 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.

At the inception of arrangements that include milestone payments, we evaluate whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of

whether (a) the consideration is commensurate with either (1) the entity's performance to achieve the milestone, or (2) the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the entity's performance to achieve the milestone, (b) the consideration relates solely to past performance, and (c) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. We evaluate factors such as the scientific, regulatory, commercial and other risks that must be overcome to achieve the respective milestone, the level of effort and investment required to achieve the respective milestone and whether the milestone consideration is reasonable relative to all deliverables and payment terms in the arrangement in making this assessment.

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

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

Royalty Purchase Liability

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

Share-Based Compensation

We account for all share-based compensation payments issued to employees, non-employee directors, and consultants by estimating the fair value of each equity award. Accordingly, share-based compensation expense is measured based on the estimated fair value of the awards on the date of grant, net of forfeitures. We recognize compensation expense for the portion of the award that is ultimately expected to vest over the period during which the recipient renders the required services to us on a straight-line basis. In accordance with authoritative guidance, we re-measure the fair value of consultant share-based awards as the awards vest, and recognize the resulting value, if any, as expense during the period the related services are rendered.

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

We use the Black-Scholes option pricing model to value our stock option awards and the options to purchase shares under our ESPP. 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 six months ended June 30, 2017 and 2016:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
Expected common stock price volatility	82%	71%	81%	71%
Risk-free interest rate	1.82%-1.95%	1.39% -1.53%	1.82%-2.38%	1.39%-1.92%
Expected term of options (years)	5.7	6.0	6.1	6.1
Expected dividend yield	—	—	—	—

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

We are also required to estimate forfeitures at the time of grant, and revise those estimates in subsequent periods if actual forfeitures differ from our estimates. We use historical data to estimate pre-vesting forfeitures and record share-based compensation expense only for those awards that are expected to vest. To the extent that actual forfeitures differ from our estimates, the difference is recorded as a cumulative adjustment in the period the estimates were revised.

Share-based compensation expense for equity grants to employees, non-employee directors and consultants was $5.0 million and $8.3 million for the three months ended June 30, 2017 and 2016, respectively, net of expected forfeitures. Share-based compensation expense for equity grants to employees, non-employee directors and consultants was $11.1 million and $16.6 million for the six months ended June 30, 2017 and 2016, respectively, net of expected forfeitures. As of June 30, 2017, we had $28.8 million of total unrecognized share-based compensation expense, which we expect to recognize over a weighted-average remaining vesting period of approximately 2.4 years. We expect our share-based compensation expense for our equity awards to employees, non-employee directors and consultants to decrease as a result of our reduction in force.

For the three and six months ended June 30, 2017 and 2016, we allocated share-based compensation as follows:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
	(in thousands)			
Research and development	$ 2,897	$ 6,383	$ 7,047	$ 11,068
General and administrative	2,091	1,926	4,005	5,573
Total	$ 4,988	$ 8,309	$ 11,052	$ 16,641

Income Taxes

In 2014, we received $83.3 million from Novo A/S under the Novo Agreement, which was reported as revenue for income tax purposes. Also in 2014, we received $200.0 million from Novartis upon execution of the Novartis Agreement, a portion of which was reported as revenue for income tax purposes. In addition, we received a milestone payment of $50.0 million in 2014 from Novartis which was reported as revenue for income tax purposes. As a result of these payments, and after taking into account the utilization of our federal and state net operating loss carry-forwards and utilization of our research and development tax credits, we reported taxable income for tax purposes in 2014. We made income tax payments of $40.2 million during the year ended December 31, 2014.

In 2015, we incurred losses for tax purposes. As of December 31, 2015, we had recorded net deferred tax assets of $23.1 million. We realized these net deferred tax assets in 2016 due to our ability to carry back our 2015 federal tax loss to 2014. We expect to carry forward our 2015 and 2016 state tax losses due to various state restrictions on the use of carryback claims. We are projecting tax losses for 2017. The deferred tax assets associated with these losses incurred to date in 2017 have a full valuation allowance recorded against them, however, due to our history of losses and the lack of other positive evidence to support future taxable income against which these losses could be applied. See Note 7 to our financial statements in Part I-Item 1 of this Quarterly Report on form 10-Q for further information regarding our expectations with respect to our income tax provision.

Results of Operations

Comparison of Three Month Periods Ended June 30, 2017 and 2016

	Three months ended June 30,		
	2017	2016	Increase (Decrease)
	(in thousands)		
Statement of Operations Data:			
Collaboration revenue	$ 1,661	$ 28,198	$ (26,537)
Operating expenses:			
Research and development	15,657	48,262	(32,605)
General and administrative	8,552	10,489	(1,937)
Total operating expenses	24,209	58,751	(34,542)
Loss from operations	(22,548)	(30,553)	(8,005)
Interest income	344	446	(102)
Other loss	(1)	(98)	(97)
Loss before income tax benefit	(22,205)	(30,205)	(8,000)
Income tax benefit	(1)	(260)	(259)
Net loss	$ (22,204)	$ (29,945)	$ (7,741)

Collaboration Revenue

Collaboration revenue for the three months ended June 30, 2017 was $1.7 million. Using the relative selling price method, we recognized $1.7 million related to the research and development activities we performed under the Novartis Agreement and a de minimis amount of revenue related to our joint operating committee participation obligations.

Collaboration revenue for the three months ended June 30, 2016 was $28.2 million. Using the relative selling price method, we recognized $22.9 million related to the license delivered to Novartis under the Novartis Agreement, $5.1 million related to research and development activities performed under the Novartis Agreement, $0.1 million related to Fovista API we transferred to Novartis, and a de minimis amount of revenue related to our joint operating committee participation obligation during the same period.

Research and Development Expenses

Our research and development expenses were $15.7 million for the three months ended June 30, 2017, a decrease of $32.6 million compared to $48.3 million for the three months ended June 30, 2016. Research and development expenses for the three months ended June 30, 2017 include approximately $1.1 million in costs related to our previously announced reduction in personnel. The decrease in research and development expenses for the three months ended June 30, 2017 was primarily due to a $23.5 million decrease in costs associated with our Fovista program, including our Fovista Phase 3 clinical program and our Fovista Expansion Studies, a $4.7 million decrease in personnel expenses, a $3.5 million decrease in share-based compensation costs and a $2.0 million decrease in professional services and consulting fees. The decreased costs for our Fovista program included lower costs related to Fovista manufacturing activities and lower clinical trial costs as a result of the wind-down of OPH1002 and OPH1003 and the Fovista Expansion Studies. This overall decrease was offset by a $1.6 million increase associated with our Zimura program, primarily related to manufacturing expenses.

General and Administrative Expenses

Our general and administrative expenses were $8.6 million for the three months ended June 30, 2017, a decrease of $1.9 million compared to $10.5 million for the three months ended June 30, 2016. General and administrative expenses for the three months ended June 30, 2017 include approximately $0.7 million in costs related to our previously announced reduction in personnel and the termination of facilities leases. The decrease in general and administrative expenses was primarily due to a decrease in costs to support our operations and infrastructure offset by the additional severance costs.

Interest Income

Interest income for the three months ended June 30, 2017 was $0.3 million compared to $0.4 million for the three months ended June 30, 2016. The interest income earned during the three months ended June 30, 2017 was the result of an increase of our investment portfolio yields offset by a decrease in our cash and cash equivalent balances available for investment.

Income Tax Provision

For the three months ended June 30, 2017, we recorded a de minimis benefit for income taxes. The benefit for income taxes of $0.3 million for the three month ended June 30, 2016 related to the partial release of our valuation allowance to reflect the income tax associated with unrealized gains on available for sale marketable securities recorded in other comprehensive income. A corresponding income tax provision was also recorded in other comprehensive income.

Comparison of Six Month Periods Ended June 30, 2017 and 2016

	Six months ended June 30,		
	2017	2016	Increase (Decrease)
	(in thousands)		
Statement of Operations Data:			
Collaboration revenue	$ 3,323	$ 43,919	$ (40,596)
Operating expenses:			
Research and development	47,636	86,032	(38,396)
General and administrative	21,711	25,185	(3,474)
Total operating expenses	69,347	111,217	(41,870)
Loss from operations	(66,024)	(67,298)	(1,274)
Interest income	722	892	(170)
Other loss	(22)	(68)	(46)
Loss before income tax provision (benefit)	(65,324)	(66,474)	(1,150)
Income tax provision (benefit)	2	(228)	230
Net loss	$ (65,326)	$ (66,246)	$ (920)

Collaboration Revenue

Collaboration revenue for the six months ended June 30, 2017 was $3.3 million. Using the relative selling price method, we recognized $3.3 million related to the research and development activities we performed under the Novartis Agreement and a de minimis amount of revenue related to our joint operating committee participation obligations.

Collaboration revenue for the six months ended June 30, 2016 was $43.9 million. Using the relative selling price method, we recognized $22.9 million related to the license we delivered to Novartis under the Novartis Agreement, $6.4 million related to research and development activities performed under the Novartis Agreement, $14.5 million related to Fovista API we transferred to Novartis, and a de minimis amount of revenue related to our joint operating committee participation obligation during the same period.

Research and Development Expenses

Our research and development expenses were $47.6 million for the six months ended June 30, 2017, a decrease of $38.4 million compared to $86.0 million for the six months ended June 30, 2016. Research and development expenses for the

six months ended June 30, 2017 include approximately $5.9 million in costs related to our previously announced reduction in personnel. The decrease in research and development expenses for the six months ended June 30, 2017 was primarily due to a $37.7 million decrease in costs associated with our Fovista program, including our Fovista Phase 3 clinical program and our Fovista Expansion Studies, a $3.1 million decrease in professional services and consulting fees, and a $4.0 million decrease in share-based compensation costs. The decreased costs for our Fovista program included lower costs related to Fovista manufacturing activities and lower clinical trial costs as a result of the wind-down of OPH1002 and OPH1003 and the Fovista Expansion Studies. This overall decrease was offset by a $6.0 million increase associated with our Zimura program, primarily related to manufacturing expenses, and a $1.1 million increase to personnel expenses associated with additional severance costs.

General and Administrative Expenses

Our general and administrative expenses were $21.7 million for the six months ended June 30, 2017, a decrease of $3.5 million compared to $25.2 million for the six months ended June 30, 2016. General and administrative expenses for the six months ended June 30, 2017 include approximately $4.6 million in costs related to our previously announced reduction in force and the termination of facilities leases. The decrease in general and administrative expenses was primarily due to a decrease in costs to support our operations and infrastructure offset by the additional severance and lease termination costs.

Interest Income

Interest income for the six months ended June 30, 2017 was $0.7 million compared to $0.9 million for the six months ended June 30, 2016. The interest income earned during the six months ended June 30, 2017 was the result of an increase of our investment portfolio yields offset by a decrease in our cash and cash equivalent balances available for investment.

Income Tax Provision

We recorded a provision for income taxes of $2 thousand and a benefit from income taxes of $0.2 million, respectively, for the six months ended June 30, 2017 and 2016. The benefit in the prior year related to the partial release of our valuation allowance to reflect the income tax associated with unrealized gains on available for sale marketable securities recorded in other comprehensive income. A corresponding income tax provision was also recorded in other comprehensive income.

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. In each of November 2014 and April 2015 we received payments of $50.0 million upon the achievement of two patient enrollment-based milestones, and in August 2016, $30.0 million upon the achievement of a third, and final, enrollment-based milestone, for an aggregate total of $130.0 million. In connection with the receipt of the upfront payment from Novartis, we made a milestone payment in June 2014 of approximately $19.8 million to Nektar Therapeutics, or Nektar. We are entitled to certain additional future payments from Novartis based on the continued clinical development, and in the event of regulatory approval and/or commercial success, of Fovista. See “Licensing and Commercialization Agreement with Novartis Pharma AG” below for further information.

Cash Flows

As of June 30, 2017, we had cash, cash equivalents and marketable securities totaling $196.4 million and no debt. We primarily invest our cash, cash equivalents and marketable securities in U.S. Treasury securities, money market funds and certain investment-grade corporate debt securities.

The following table shows a summary of our cash flows for the six months ended June 30, 2017 and 2016:

	Six months ended June 30,	
	2017	2016
	(in thousands)	
Net cash (used in) provided by:		
Operating Activities	$ (92,504)	$ (69,963)
Investing Activities	99,445	38,348
Financing Activities	46	3,808
Net change in cash and cash equivalents	$ 6,987	$ (27,807)

Cash Flows from Operating Activities

Net cash used in operating activities for the six months ended June 30, 2017 was $92.5 million and relates primarily to net cash used for the wind-down of OPH1002 and OPH1003 and the Fovista Expansion Studies, implementation of a previously announced reduction in personnel and related costs, and cancellation fees related to manufacturing commitments, as well as continuation of our OPH1004 trial and general and administrative and corporate infrastructure expense.

Net cash used in operating activities for the six months ended June 30, 2016 was $70.0 million and related primarily to net cash used to fund our Fovista Phase 3 program, our Fovista Expansion Studies, Fovista manufacturing activities, manufacturing and clinical trial costs for our Zimura program and expenditures related to general and administrative expenses, as well as changes in the components of working capital.

See "—Funding Requirements" below for a description of how we expect to use our cash for operating activities in future periods.

Cash Flows from Investing Activities

Net cash provided by investing activities for the six months ended June 30, 2017 was $99.4 million and relates primarily to proceeds from the maturities of marketable securities totaling $111.5 million offset by purchases of marketable securities totaling $12.0 million. Net cash used in investing activities for the six months ended June 30, 2016 was $38.3 million, which related primarily to proceeds from the maturities of marketable securities totaling $50.5 million offset by purchases of marketable securities totaling $12.0 million.

Cash Flows from Financing Activities

Net cash provided by financing activities was $46 thousand for the six months ended June 30, 2017 and $3.8 million for the six months ended June 30, 2016 and related to proceeds from stock option/employee stock purchase plan exercises in each respective period.

Funding Requirements

We currently have two product candidates, Zimura and Fovista, which are in clinical development. We expect to continue to incur significant research and development expenses as we wind-down the OPH1002 and OPH1003 trials and the Fovista Expansion Studies, continue the OPH1004 trial and pursue the development of Zimura as a monotherapy for the treatment of GA, in combination with an anti-VEGF drug for the treatment of wet AMD, in combination with an anti-VEGF drug for the treatment of IPCV and as a monotherapy in orphan indications, including Stargardt disease and non-infectious intermediate and posterior uveitis. We could incur additional research and development expenses if we conclude that there is a scientific rationale for potentially developing, or if we undertake the development of, Zimura or Fovista in additional indications, and as we evaluate and potentially in-license or acquire, and undertake development of, additional product candidates. Furthermore, if we successfully develop and obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. For Fovista, our ex-U.S. commercialization partner Novartis is responsible for these commercialization expenses outside the United States. We are party to agreements, specifically a divestiture agreement with OSI (Eyetech), Inc., or Eyetech, 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 that impose significant milestone payment obligations on us in connection with our achievement of specific clinical, regulatory and commercial milestones with respect to Zimura and Fovista. It is likely that any future in-licensing or acquisition agreements that we enter into with respect to additional products, product candidates or technologies would include similar obligations.

We expect that we will continue to incur significant expenses as we:

- continue the clinical development of Zimura as a monotherapy for the treatment of GA, in combination with an anti-VEGF drug for the treatment of wet AMD, in combination with an anti-VEGF drug for the treatment of IPCV, as a monotherapy in orphan indications, including Stargardt disease and non-infectious intermediate and posterior uveitis, or potentially in other indications if we believe there is a sufficient scientific rationale to pursue such development;
- in-license or acquire the rights to, and pursue the development of, other products, product candidates or technologies;
- complete the activities necessary to receive initial, top-line data from OPH1004 and wind down OPH1002, OPH1003 and the Fovista Expansion Studies;
- potentially undertake additional clinical development of Fovista in wet AMD if the initial, top-line data from OPH1004 is favorable or in other indications if we believe there is a sufficient scientific rationale to pursue such development;
- complete our previously announced reduction in personnel;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, manufacturing, quality control, quality assurance and scientific personnel if we are successful in progressing the clinical development of any of our product candidates;
- seek marketing approval for any product candidates that successfully complete clinical trials; and
- 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 of our product candidates.

As of June 30, 2017, we had cash, cash equivalents, and marketable securities of $196.4 million, of which approximately $20 million to $35 million is committed to the wind-down of OPH1002 and OPH1003 and the Fovista Expansion Studies, implementing a previously announced reduction in personnel and related costs, cancellation fees related to manufacturing commitments, and obtaining initial, top-line data for the OPH1004 trial by the end of the third quarter of 2017. We estimate that our 2017 year-end cash balance will range between $145 million to $160 million, excluding any potential business development activities or any changes to our current or planned clinical development programs. We also had $350.6 million in total liabilities as of June 30, 2017, $331.7 million of which related to the Novo Agreement and deferred revenue associated with the Novartis Agreement, which we are required to show as liabilities on our balance sheets under generally

accepted accounting principles but which, in the case of Novo, do not correspond to any contractual repayment obligation, or in the case of Novartis, are highly unlikely to be triggered.

We believe that our cash, cash equivalents and marketable securities will be sufficient to fund our operations and capital expenditure requirements as currently planned for at least the next 12 months. This estimate does not reflect any additional expenditures resulting from the in-licensing or acquisition of additional product candidates or technologies or associated development that we may pursue following any such transactions. 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 pursuit, by acquisition, in-licensing or otherwise, and subsequent development of additional product candidates or technologies, and the success of our ongoing development programs. We believe that we may need additional funding in the event that we acquire or in-license one or more additional product candidates and undertake development. In addition, our expenses may exceed our expectations if we experience delays in enrollment or with the availability of drug supply for our clinical trials, if we experience any unforeseen issues in our ongoing clinical trials or if we further expand the scope of our clinical trials and programs. Our costs may also exceed our expectations for other reasons, for example, if we experience issues with manufacturing, process development, or if we are required by the FDA, the 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. As a result, we may need or may seek to obtain additional funding in connection with our continuing operations sooner than expected.

The future development of Zimura and Fovista is highly uncertain. If the results from the OPH1004 trial are favorable and we determine to pursue further development of Fovista for use in combination with anti-VEGF drugs for the treatment of wet AMD, we will likely need to conduct one or more further pivotal, clinical trials for Fovista to support potential marketing approval, which will be time-consuming and expensive. Furthermore, we expect the clinical development for Zimura will continue for at least the next several years. At this time, we cannot reasonably estimate the remaining costs necessary to complete the clinical development of either Zimura or Fovista, complete process development and manufacturing scale-up and validation activities associated with Zimura and potentially seek marketing approval for Zimura and Fovista, 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, costs and results of our Zimura clinical programs, including our Phase 2/3 GA clinical trial, our planned Phase 2a wet AMD clinical trial, our planned Phase 2a IPCV clinical trial, our planned randomized, controlled clinical trial in Stargardt disease and our planned Phase 2a clinical trial in non-infectious intermediate and posterior uveitis, as well as any additional clinical trials we undertake to obtain data sufficient to seek marketing approval for Zimura in any indication (including a potential second pivotal trial for GA and for Stargardt disease);

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

- the amount of any upfront, milestone payments and other financial obligations associated with the in-license or acquisition of other product candidates;

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

- the scope, progress, costs and results of the OPH1004 trial and any additional clinical trials we undertake to obtain data sufficient to seek marketing approval for Fovista in wet AMD or any other indication;

- if OPH1004 is positive and we decide to pursue further development of Fovista, the costs and timing of restarting the manufacturing of commercial supply for Fovista;

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

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

- the costs, timing and outcome of regulatory reviews of our product candidates;

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

- the timing, scope and cost of commercialization activities for any of our product candidates if we receive, or expect to receive, marketing approval for a product candidate; and

- subject to receipt of marketing approval, net revenue received from commercial sales of any of our product candidates, after milestone payments and royalty payments that we would be obligated to make.

Furthermore, following our receipt and announcement of initial, top-line results from our pivotal OPH1002 and OPH1003 Fovista trials for the treatment of wet AMD in December 2016, we implemented a restructuring plan that included a reduction in personnel. This reduction in personnel involves approximately 80% of our pre-announcement workforce and includes employees from nearly every department. We expect to realize estimated annualized cost savings from the reduction in personnel in the range of $20 million to $25 million starting in the third quarter of 2017. We may not be able to successfully implement the restructuring and we may not realize the planned or expected cost savings benefits, which could adversely affect our estimate of the period for which our cash, cash equivalents and marketable securities will be sufficient to fund our operations and capital expenditure requirements as currently planned. During the first six months of 2017, our workforce has been reduced by 98 employees in connection with the reduction in personnel and natural attrition. We expect the reduction of the additional 20 affected employees to be substantially completed during the third quarter of 2017. In connection with such reduction in personnel, we estimate that we will incur approximately $13.4 million of aggregate pre-tax charges through the third quarter of 2017, of which approximately $12.5 million in the aggregate is expected to result in cash expenditures. These pre-tax charges relate to (a) expected severance, stock compensation and other employee costs of approximately $11.3 million and (b) expected lease termination costs of approximately $2.1 million. As of June 30, 2017, our cash expenditures related to such reduction in personnel totaled $8.0 million.

We do not have any committed external source of funds other than the Novartis Agreement, and the remaining potential milestone payments under the Novartis Agreement are subject to our achievement of specified regulatory and commercial events related to Fovista, none of which are likely to be achieved given the data outcome of the OPH1002 and OPH1003 trials. Our future commercial revenues, if any, will be derived from sales of any of our product candidates that we are able to successfully develop, which, depending on the product, may not be available for at least several years, if at all. In addition, if approved, Zimura or Fovista or any product that we acquire or in-license may not achieve commercial success. If that is the case, we will need to obtain substantial additional financing to achieve our business objectives. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

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.

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.

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 would be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect their rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Our pledge of assets, including intellectual property rights, as collateral to secure our obligations under the Novo Agreement may limit our ability to obtain debt financing. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, products or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves.

Licensing and Commercialization Agreement with Novartis Pharma AG

In May 2014, we entered into a licensing and commercialization agreement with Novartis Pharma AG, which we refer to as the Novartis Agreement. Under the Novartis Agreement, we granted Novartis exclusive rights under specified patent rights, know-how and trademarks controlled by us to manufacture, from bulk API supplied by us, standalone Fovista products and products combining Fovista with an anti-VEGF drug to which Novartis has rights in a co-formulated product, for the treatment, prevention, cure or control of any human disease, disorder or condition of the eye, and to develop and commercialize those licensed products in all countries outside of the United States, which we refer to as the Novartis Territory. We agreed to use commercially reasonable efforts to complete our ongoing pivotal Phase 3 clinical program for Fovista and Novartis agreed to use commercially reasonable efforts to develop a standalone Fovista product and a co-formulated product containing Fovista and an anti-VEGF drug to which Novartis has rights, as well as a pre-filled syringe presentation of such products and to use commercially reasonable efforts, subject to obtaining marketing approval, to commercialize licensed products in the Novartis Territory in accordance with agreed development and marketing plans. Novartis 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 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 agreed to exclusively supply Novartis, and Novartis 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 agreed not to commercialize any product comprising Fovista or any other anti-PDGF product in the ophthalmic field in the Novartis Territory.

The original Novartis Agreement does not specifically address the current status of the Fovista Phase 3 program, wherein the parties are dependent on the OPH1004 data outcome to assess and determine future plans for Fovista. In July 2017, we and Novartis entered into a letter agreement to streamline the process and timeline for evaluating data, once it becomes available, from the OPH1004 trial, and, depending on the results from the OPH1004 trial, determining a regulatory strategy in the European Union for Fovista. In the letter agreement, the parties agreed to suspend their affirmative obligations under the Novartis Agreement regarding development, manufacture and commercialization of Fovista products pending receipt of the OPH1004 data and the determination of a regulatory strategy in the European Union. The letter agreement also provides Novartis with a shorter notice period in the event Novartis determines to terminate the Novartis Agreement in certain circumstances and provides for a process for the parties to determine the scope and funding for additional clinical trials, if any, required for regulatory approval of Fovista. If we and Novartis do not otherwise agree as to the funding for any additional clinical trials, each party will be required to fund fifty percent (50%) of the cost and expense of such clinical trials. In addition, the letter agreement provides Novartis with a fully paid-up, royalty-free license to use data from the Lucentis monotherapy arms of our Phase 2b OPH1001 trial and Phase 3 OPH1002 and OPH1003 trials in the Novartis Territory in connection with the development, manufacturing and commercialization of Novartis-controlled anti-VEGF products. The Lucentis study data license shall continue until the fifth anniversary of the letter agreement or the date the Novartis Agreement expires or terminates, whichever is later.

Novartis paid us a $200.0 million upfront fee upon execution of the Novartis Agreement. Novartis also paid us $50.0 million upon the achievement of each of two patient enrollment-based milestones, and $30.0 million upon the achievement of a third, and final, enrollment-based milestone, for an aggregate of $130.0 million. Under the terms of the Novartis Agreement, Novartis is also obligated to pay us up to an aggregate of an additional $300.0 million upon achievement of specified regulatory milestones, including marketing approval and reimbursement approval in certain countries in the Novartis Territory, and up to an aggregate of an additional $400.0 million if Novartis achieves specified sales milestones in the Novartis Territory. Novartis is also obligated to pay us royalties with respect to standalone Fovista products and combination Fovista products that Novartis successfully commercializes. We will receive royalties at a mid-thirties percentage of net sales of standalone Fovista products and a royalty of approximately equal value for sales of combination Fovista products. Such royalties are subject to customary deductions, credits, and reductions for lack of patent coverage or market exclusivity. Novartis's obligation to pay such royalties will continue on a licensed product-by-licensed product and country-by-country basis until Novartis's last actual commercial sale of such licensed product in such country. We will continue to be responsible for royalties we owe to third parties on sales of Fovista products.

Novartis agreed to pay our manufacturing costs for clinical supplies and our manufacturing costs plus a specified percentage margin for commercial supplies of the bulk API for Fovista that we supply to Novartis. If we or Novartis exercise our or its respective rights to obtain access to study data from clinical trials conducted by the other party, the party exercising the option will be obligated to pay the other party's associated past development costs and share with such other party any future associated development costs. If we exercise our option to obtain Novartis-controlled rights to develop, manufacture and commercialize any co-formulated Fovista product in the United States, we will be obligated to pay a specified percentage of Novartis's associated past development costs and share with Novartis any future associated development costs. Novartis and we

will also need to negotiate and agree on financial and other terms that would apply to such rights. If we exercise our option to obtain Novartis-controlled rights to develop and commercialize a pre-filled syringe product in the United States, we will be obligated to either enter into a supply agreement with Novartis under which we will pay Novartis its manufacturing cost plus a specified percentage margin for supplies of Fovista products in pre-filled syringes that Novartis supplies to us, or obtain supplies of products in pre-filled syringes from a third party manufacturer and pay Novartis a low single-digit percentage of our net sales of such products.

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

The Novartis Agreement, unless earlier terminated by Novartis or us, will expire upon the expiration of Novartis's obligation to pay us royalties on net sales of licensed products. Novartis and we each may terminate the Novartis Agreement if the other party materially breaches the agreement and does not cure such breach within a specified cure period, if the other party experiences any specified insolvency event, if the other party challenges or assists a third party in challenging the validity or enforceability of certain patent rights controlled by the terminating party, or if the parties are prevented in any manner that materially adversely affects the progression of the development or commercialization of licensed products for a specified period as a result of specified governmental actions. Novartis may terminate the Novartis Agreement at any time without cause, or within a specified period after a change in control of us, as defined in the Novartis Agreement, or for specified safety reasons, effective at the end of a specified period following Novartis's written notice to us of Novartis's election to terminate the agreement. We may also terminate the agreement if Novartis determines to seek marketing approval of an alternative anti-PDGF product in the Novartis Territory as more fully described below. 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. As part of the July 2017 letter agreement, we permanently waived our right to terminate the Novartis Agreement in the event that the parties are prevented from materially progressing the development or commercialization of Fovista products for a specified period of time as a result of specified governmental actions.

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

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

Manufacturing and Supply Agreements for API and Fill/Finish Services

Clinical API Supply Agreement with Agilent Technologies, Inc.

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

Commercial API Supply Agreement with Agilent Technologies, Inc.

In September 2015, we entered into a Commercial Manufacturing and Supply Agreement with Agilent, pursuant to which Agilent has agreed to manufacture and supply to us, and we have agreed to purchase from Agilent, a specified percentage of our commercial requirements in all jurisdictions of Fovista API. The commercial supply agreement has an initial term that runs for seven years from the date of our first commercial sale of Fovista, and which is subject to one two-year automatic renewal period, absent termination by either party in accordance with the terms of the commercial supply agreement. The commercial supply agreement provides for pricing for Fovista API structured on a tiered basis, with the price reduced as the volume of Fovista API ordered increases. We may cancel any purchase order under the commercial supply agreement at any time, subject to the payment of specified cancellation fees. We may terminate the commercial supply agreement with no financial penalty in the event that we cannot commercialize Fovista due to regulatory or other medical, scientific or legal reasons. Agilent may terminate the commercial supply agreement in the event that we do not, over a specified period, purchase and take delivery from Agilent of a specified minimum quantity of Fovista API. Each party also has the right to terminate the commercial supply agreement for other customary reasons such as material breach and bankruptcy. The commercial supply agreement contains provisions relating to compliance by Agilent with current Good Manufacturing Practices, cooperation by Agilent in connection with marketing applications for Fovista, indemnification, confidentiality, dispute resolution and other customary matters for an agreement of this kind.

In December 2016, following receipt of initial, top-line data from our pivotal OPH1002 and OPH1003 Fovista trials for the treatment of wet AMD, we canceled all of our outstanding purchase orders with Agilent for the manufacture of Fovista API for commercial drug product. In April 2017, we agreed to pay Agilent $12.7 million to satisfy our outstanding obligations for cancellation fees with respect to such canceled purchase orders, payment of which was made in April 2017.

Clinical and Commercial Services Agreement with Ajinomoto Althea, Inc.

In October 2016, we and Ajinomoto Althea, Inc., or Althea, entered into a Clinical and Commercial Services Agreement, which we refer to as the Fill/Finish Services Agreement. Pursuant to the Fill/Finish Services Agreement, Althea has agreed to provide clinical and commercial fill/finish services for Zimura and Fovista, as well as any future product candidates that we and Althea may mutually agree. The Fill/Finish Services Agreement has an initial term that will expire at the end of 2027, absent termination by either party in accordance with the terms of the Fill/Finish Services Agreement. The initial term of the Fill/Finish Services Agreement may be extended by mutual agreement of the parties. The amount payable by us to Althea under the Fill/Finish Services Agreement is based on the volume of finished drug product that we order, subject to periodic adjustments over the term of the Fill/Finish Services Agreement. In addition, in the event that we order a specified volume of product, Althea has agreed to supply biological or pharmaceutical drug products meeting certain parameters exclusively to us. We may cancel any purchase order under the Fill/Finish Services Agreement at any time, subject to the payment of specified cancellation fees. We may terminate the Fill/Finish Services Agreement, without cause, as of any date following the third anniversary of the effective date upon six months’ prior notice to Althea. Each party also has the right to terminate the Services Agreement for other customary reasons such as material breach and bankruptcy. The Fill/Finish Services Agreement contains provisions relating to compliance by Althea with current Good Manufacturing Practices, cooperation by Althea in connection with marketing applications for our product candidates, indemnification, confidentiality, dispute resolution and other customary matters for an agreement of this kind.

In December 2016, following receipt of initial, top-line data from our pivotal OPH1002 and OPH1003 Fovista trials for the treatment of wet AMD, we canceled all of our outstanding purchase orders with Althea for the fill and finish of Fovista commercial drug product. We incurred approximately $0.6 million in connection with such cancellations in relation to non-returnable materials procured by Althea in anticipation of fulfilling such purchase orders, payment of which was made in the second quarter of 2017.

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-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 used the remaining proceeds 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 June 30, 2017:

	Payments Due by Period				
	Total	Less than 1 year	1-3 years	3-5 years	More than 5 years
			(in thousands)		
Operating Leases (1)	$ 1,124	$ 1,124	$ —	$ —	$ —
Severance and Other Employee Benefits (2)	4,441	4,441	—	—	—
Total (3)	$ 5,565	$ 5,565	$ —	$ —	$ —

(1) The table above includes our continuing rent obligations through February 2018.

(2) Severance and Other Employee Benefits represents our commitments under the Board of Directors' approved plan to implement a reduction in personnel that involves approximately 80% of our workforce based on the number of employees at the time the plan was approved.

(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, (e) 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 and (f) our royalty purchase liability of $125.0 million as of June 30, 2017, due to the fact that the royalty payment period, if any, is not known.

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

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

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

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

- Under a license, manufacturing and supply agreement with Nektar 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 2017 annual meeting of stockholders, as filed with the SEC on April 24, 2017.

In December 2016, we announced that we had determined to implement a reduction in personnel to focus on an updated business plan. In January 2017, our Board of Directors approved a plan to implement a reduction in personnel that involves approximately 80% of our workforce based on the number of employees at the time the plan was approved. We expect to realize estimated annualized cost savings from the reduction in personnel in the range of $20 million to $25 million starting in the third quarter of 2017. During the first six months of 2017, our workforce has been reduced by 98 employees in connection with the reduction in personnel and natural attrition. We expect the reduction of the additional 20 affected employees will be substantially completed during the third quarter of 2017. In connection with such reduction in personnel, we estimate that we will make cash expenditures totaling approximately $12.5 million in the aggregate, which relate to expected severance and other employee costs. As of June 30, 2017, our cash expenditures related to such reduction in

personnel totaled $8.0 million.

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.

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 $196.4 million as of June 30, 2017, consisting of cash, investments in money market funds and certain investment-grade corporate debt securities, and direct investment in U.S. Treasury securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because a significant portion of our investments are in short-term securities. Our available for sale securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

We contract with CROs and CMOs globally. We may be subject to fluctuations in foreign currency rates in connection with certain of these agreements. Transactions denominated in currencies other than the U.S. dollar are recorded based on exchange rates at the time such transactions arise. As of June 30, 2017, 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 June 30, 2017. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Based on the evaluation of our disclosure controls and procedures as of June 30, 2017, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

No changes 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 quarter ended June 30, 2017 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

On January 11, 2017, a putative class action lawsuit was filed against us and certain of our current and former executive officers in the United States District Court for the Southern District of New York, captioned Frank Micholle v. Ophthotech Corporation, et al., No. 1:17-cv-00210. The complaint purports to be brought on behalf of shareholders who purchased our common stock between May 11, 2015 and December 12, 2016. The complaint generally alleges that we and certain of our officers violated Sections 10(b) and/or 20(a) of the Securities Exchange Act of 1934 and Rule 10b-5 promulgated thereunder by making allegedly false and/or misleading statements concerning the prospects of our Phase 3 trials for Fovista in combination with anti-VEGF drugs for the treatment of wet AMD. The complaint seeks equitable and/or injunctive relief, unspecified damages, attorneys' fees, and other costs.

On March 9, 2017, a second putative class action lawsuit was filed against us and the same group of our current and former executive officers in the United States District Court for the Southern District of New York, captioned Wasson v. Ophthotech Corporation, et al., No. 1:17-cv-01758. The complaint purports to be brought on behalf of shareholders who purchased our common stock between May 11, 2015 and December 9, 2016. The allegations made in the complaint are similar to those made in the Micholle complaint. Putative lead plaintiffs in the Micholle action have moved to consolidate the Micholle and Wasson actions.

On May 30, 2017, a shareholder derivative action was filed against the members of our Board of Directors in the United States District Court for the Southern District of New York, captioned Etelmendorf v. Bolte, et al., No. 1:17-cv-04042. The complaint alleges that defendants breached their fiduciary duties to our company by causing or permitting the company to make allegedly false and/or misleading statements concerning the prospects of our Phase 3 trials for Fovista in combination with anti-VEGF drugs for the treatment of wet AMD, and by approving certain executive compensation. The complaint also alleges that defendants were unjustly enriched as a result of the alleged conduct. The complaint purports to seek unspecified damages on our behalf, as well as an order directing us to reform and comply with its governance obligations, attorneys' fees, and other costs.

We deny any allegations of wrongdoing and intend to vigorously defend against these lawsuits. We are unable, however, to predict the outcome of these matters at this time. Moreover, any conclusion of these matters in a manner adverse to us and for which we incur substantial costs or damages not covered by our directors' and officers' liability insurance would have a material adverse effect on our financial condition and business. In addition, the litigation could adversely impact our reputation and divert management's attention and resources from other priorities, including the execution of business plans and strategies that are important to our ability to grow our business, any of which could have a material adverse effect on our business.

Item 1A. Risk Factors.

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

Risks Related to Our Updated Business Plan, Financial Position and Need for Additional Capital

We are in the process of implementing a new, updated business plan that will continue to evolve as we await relevant clinical data and evaluate new opportunities. Our updated business plan may lead to the initiation of one or more development programs or the execution of one or more transactions that you do not agree with or that you do not perceive as favorable to your investment.

We have invested a significant portion of our efforts and financial resources in the development of Fovista to be administered in combination with anti-VEGF drugs for the treatment of wet AMD, as well as the potential commercial launch of Fovista. In December 2016, we announced the failure of our two pivotal, Phase 3 clinical trials, which we refer to as OPH1002 and OPH1003, evaluating the safety and efficacy of 1.5mg of Fovista administered in combination with monthly 0.5mg Lucentis® (ranibizumab) anti-VEGF therapy compared to monthly 0.5mg Lucentis monotherapy for the treatment of wet AMD, to meet their pre-specified primary endpoints. OPH1004, our remaining Phase 3 clinical trial, which is evaluating the safety and efficacy of 1.5 mg of Fovista administered in combination with 2.0mg Eylea® (aflibercept) or 1.25mg Avastin®

(bevacizumab) anti-VEGF therapy compared to 2.0mg Eylea or 1.25mg Avastin monotherapy for the treatment of wet AMD, remains ongoing, with initial, top-line data expected to be available by the end of the third quarter of 2017.

We announced in early 2017 that we had engaged a financial advisor to assist us in reviewing our strategic alternatives, including identifying potential business development opportunities. Also beginning in early 2017, we undertook a reassessment of our development plans for Zimura and Fovista, which included an evaluation of the scientific rationale for potentially developing these product candidates in one or more other ophthalmic indications for which there is a high unmet need.

On July 26, 2017, we announced that we are pursuing a strategy to leverage our clinical experience and retina expertise to identify and develop therapies to treat multiple ophthalmic orphan diseases for which there are limited or no treatment options available. In parallel, we are continuing our on-going programs in age-related retinal diseases. We also are continuing our business development efforts to identify and potentially obtain rights to additional products, product candidates and technologies that would complement our strategic goals as well as other compelling ophthalmology opportunities.

This updated business plan requires us to be successful in a number of challenging, uncertain and risky activities, including pursuing development of Zimura in indications for which we have no human clinical data, identifying promising new assets for development that are available for acquisition or in-license and that fit our strategic focus and, if so identified, negotiating and executing an acquisition or in-license agreement for one or more of those programs on favorable terms and designing and executing a clinical program for any newly acquired product candidates. We may not be successful at one or more of the activities required for us to execute this new updated business plan. We are also continuing to consider other alternatives, including the acquisition of products, product candidates or technologies or other assets outside of ophthalmology, mergers or other transactions involving our company as a whole, collaboration transactions, or the license, sale or divestiture of some of our assets or technologies. We cannot be sure when or if this review process will result in any type of transaction. Even if we pursue a transaction, such transaction may not be consistent with our stockholders' expectations or may not ultimately be favorable for our stockholders, either in the shorter or longer term.

Our growth prospects and the future value of our company are dependent on the progress of our ongoing and planned clinical development programs for our current product candidates, Zimura and Fovista, together with the amount of our remaining available cash. The development of each of Zimura and Fovista is highly uncertain. We have only very limited data from small, uncontrolled clinical trials regarding the safety and efficacy of Zimura as a monotherapy for the treatment of GA or in combination with anti-VEGF drugs for the treatment of wet AMD or IPCV, and we have no human clinical data regarding the safety and efficacy of Zimura as a treatment for Stargardt disease or non-infectious intermediate and posterior uveitis. Our prior Zimura trials were not powered to demonstrate the efficacy of Zimura therapy with any statistical significance. We remain at the very early phase of enrollment in our ongoing Zimura clinical trials, we will not be commencing our planned clinical trials for several months, and we cannot currently estimate when additional clinical data for Zimura from these or other trials will become available. In addition, 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 and are unable to predict whether the 12-month results from the OPH1004 trial will be favorable. We currently expect initial, top-line data from the OPH1004 trial to be available by the end of the third quarter of 2017. The failure of our pivotal OPH1002 and OPH1003 trials to show any clinically meaningful visual benefit in adding 1.5mg of Fovista to a monthly regimen of 0.5mg of Lucentis and the recent failure of a competitor's Phase 2 trial investigating the combination of a PDGF inhibitor and a VEGF inhibitor may be indicative of a low likelihood of success for OPH1004.

We plan to continue to reassess our existing Zimura and Fovista development programs throughout 2017. We expect that our reassessment of our Fovista development program for the treatment of wet AMD will be primarily determined by the initial, top-line data from OPH1004 and in the context of our other two failed Phase 3 clinical trials. We expect that our reassessment of our Zimura development program for GA may be particularly affected by the results of a competitor's Phase 3 clinical trials of a complement inhibitor being studied for the treatment of GA. Data from our OPH1004 trial are expected by the end of the third quarter of 2017 and data from our competitor's Phase 3 trials for the treatment of GA are expected during the second half of 2017. As a result of our ongoing assessment of our development programs, we may modify, expand or terminate some or all of our development programs or clinical trials at any time.

We expect that our remaining cash balances will continue to decline as we pursue these development programs, pursue our updated business plan and until such time, if any, as we receive additional funding, and the value of our stockholders' investment may decline as a result.

Even if we receive positive data from OPH1004 during the third quarter of 2017, the prospects for Fovista combination therapy for the treatment of wet AMD may not be readily ascertainable and we would need to assess the financial, operational and regulatory implications of such an outcome. We may make further changes to our business plan once such data is available.

While we believe that there is a low likelihood of a positive data outcome for OPH1004, a positive data outcome may pose significant financial, operational and regulatory challenges. Even if such data are favorable, the regulatory path for the potential approval of Fovista combination therapy for the treatment of wet AMD would be highly uncertain. 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. Even with a positive data outcome from OPH1004, the FDA may not consider OPH1004 to be one of the two adequate and well controlled clinical trials required for marketing approval and, unless we are able to agree with the FDA and similar regulatory authorities outside the United States on an alternative regulatory pathway for approval, we may be required to conduct one or more additional clinical trials to demonstrate the safety and efficacy of Fovista in combination with one or more anti-VEGF drugs for the treatment of wet AMD. Conducting one or more large Phase 3 clinical trials of Fovista combination therapy for the treatment of wet AMD would be expensive and time consuming and would require us to seek additional funding. Because we have already substantially reduced, and expect to continue to reduce, our personnel as part of an overall planned reduction in personnel involving approximately 80% of our workforce, a positive outcome would require us to either hire additional employees or engage a large number of consultants or other vendors in order to successfully execute one or more large Phase 3 clinical trials. We would also need to make arrangements with our suppliers to restart activities for clinical and commercial manufacturing, supply, packaging and distribution of Fovista which could result in delays in commencing a Phase 3 clinical trial. Even if we do obtain favorable data from two adequate and well controlled clinical trials, the FDA or similar regulatory authorities outside the United States may not grant marketing approval for a product candidate if such regulatory authorities do not believe that the benefits offered by such product candidate are clinically meaningful or that such benefits outweigh the observed or potential risks, which they may conclude based on potentially inconsistent and conflicting data regarding the efficacy of Fovista combination therapy.

Even if we are able to obtain marketing approval for Fovista for use in combination with one or more anti-VEGF drugs, because Avastin is not approved for use in treating wet AMD, either in the United States or outside of the United States, regulatory authorities may not permit the product label for Fovista to include the use of Fovista in combination with Avastin. For example, we have had interactions regarding our planned application for marketing approval with the European regulatory authorities, including 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; Avastin is not licensed for intravitreal use in the European Union.

Even with a positive result from OPH1004 or any other clinical trial we may conduct for Fovista in wet AMD and receipt of marketing approval, it likely would be difficult to estimate the size of the potential market for Fovista for the treatment of wet AMD and anticipate the level of market acceptance of Fovista in such market, as the failure of our pivotal, Phase 3 OPH1002 and OPH1003 trials of Fovista in combination with Lucentis and the failure of a competitor's Phase 2 trial of an anti-PDGF inhibitor administered in combination with an anti-VEGF agent have called into question PDGF as a target for the treatment of wet AMD. Furthermore, even with a positive data outcome, the inconsistent results from our Phase 3 Fovista program may adversely affect our ability to establish a price for Fovista to enable us to sell Fovista profitably, even if we obtain marketing approval. Moreover, it is possible that Novartis, our ex-U.S. commercialization partner for Fovista, may terminate the Fovista Licensing and Commercialization Agreement with us following our receipt of initial, top-line data from OPH1004, even if the data from the trial are positive. In this event, we would need to change our commercialization plans for Fovista outside the United States. For these and other reasons, the return on any further investment in developing Fovista in wet AMD following a favorable data outcome from OPH1004 may be highly uncertain.

Undertaking and pursuing the further development of Fovista in wet AMD may require that we divert resources away from, and may distract our management and other personnel from continuing to execute on, our plan to realign corporate resources toward a more broadly-diversified product portfolio. In such event, we may therefore abandon some or all of the initiatives that we undertake in pursuit of our updated business plan.

Our strategy of obtaining rights to products, product candidates or technologies for the treatment of ophthalmic diseases through in-licenses and acquisitions may not be successful. Our failure to successfully acquire or in-license and develop additional product candidates would likely impair our ability to grow.

An element of our strategy has been and continues to be to expand our product pipeline through potentially in-licensing or acquiring the rights to products, product candidates or technologies that would complement our strategic goals as well as other compelling ophthalmology opportunities. Because we expect generally that we will not engage directly in early stage research and drug discovery, the future growth of our business beyond our current product portfolio will depend significantly on our ability to in-license or acquire the rights to approved products, additional product candidates or technologies. We may be unable, however, to in-license or acquire the rights to any such products, product candidates or technologies from third parties for several reasons. The success of this strategy depends partly upon our ability to identify, select and acquire or in-license promising product candidates and technologies. We may be unable to identify suitable products, product candidates or technologies within our area of focus. The process of proposing, negotiating and implementing a license or acquisition of a product candidate is lengthy and complex. The in-licensing and acquisition of pharmaceutical products is an area characterized by intense competition, and a number of companies (both more established and early stage biotechnology companies) are also pursuing strategies to in-license or acquire products, product candidates or technologies that we may consider attractive. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities, while earlier stage companies may be more aggressive or have a higher risk tolerance. 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 product, product candidate or technology on terms that would allow us to make an appropriate return on our investment. Moreover, we may devote resources to potential acquisitions or in-licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts or we may incorrectly judge the value or worth of an acquired or in-licensed product candidate or technology.

Further, any product candidate that we acquire would most likely require additional development efforts prior to commercial sale, including extensive clinical testing and approval by the FDA and applicable foreign regulatory authorities. All product candidates are prone to risks of failure typical of pharmaceutical product development, including the possibility that a product candidate would not be shown to be sufficiently safe and effective for approval by regulatory authorities.

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. In addition, acquisitions and in-licensing arrangements for product candidates and technologies are inherently risky, and ultimately, if we do not complete an announced acquisition or license transaction or integrate an acquired or licensed product or technology successfully and in a timely manner, we may not realize the benefits of the acquisition or license to the extent anticipated and the perception of the effectiveness of our management team and our company may suffer in the marketplace. In addition, even if we are able to successfully identify, negotiate and execute one or more transactions to acquire or in-license new products, product candidates or technologies, our expenses and short-term costs may increase materially and adversely affect our liquidity.

In addition, future acquisitions and in-licenses may entail numerous operational, financial and legal risks, including:

- exposure to known and unknown liabilities, including possible intellectual property infringement claims, violations of laws, tax liabilities and commercial disputes;
- incurrence of substantial debt, dilutive issuances of securities or depletion of cash to pay for acquisitions;
- higher than expected acquisition and integration costs;
- difficulty in combining the operations and personnel of any acquired businesses with our operations and personnel;
- inability to maintain uniform standards, controls, procedures and policies;
- restructuring charges related to eliminating redundancies or disposing of assets as part of any such combination;
- large write-offs and difficulties in assessing the relative percentages of in-process research and development expense that can be immediately written off as compare to the amount that must be amortized over the appropriate life of the asset;

- increased amortization expenses or, in the event that we write-down the value of acquired assets, impairment losses;
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership;
- inability to retain personnel, key customers, distributors, vendors and other business partners integral to an in-licensed or acquired product candidate or technology;
- potential failure of the due diligence processes to identify significant problems, liabilities or other shortcomings or challenges of an acquired or licensed product candidate or technology, including, without limitation, problems, liabilities or other shortcomings or challenges with respect to intellectual property, product quality, revenue recognition or other accounting practices, partner disputes or issues and other legal and financial contingencies and known and unknown liabilities; and
- entry into indications or markets in which we have no or limited direct prior development or commercial experience and where competitors in such markets have stronger market positions.

If we are unable to successfully manage our acquisitions or other in-license transactions, our ability to develop new products and continue to expand and diversify our product pipeline may be limited.

We may not use our available cash and other sources of funding effectively as we pursue our updated business plan.

Our revised business plan may not be successful, or we may be unsuccessful in effectively executing our revised business plan, which, in either case, could result in the expenditure of our available cash and other sources of funding 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. For example, as we implement our revised business plan, we could allocate our available capital resources to pursue the development or acquisition of a particular product candidate or technology that proves to be ineffective, or we could fail to allocate sufficient resources to strategic opportunities or product candidates or technologies that may be more profitable or for which there is a greater likelihood of success. If we fail to effectively allocate our available capital resources, we may not be able to achieve our goals, and our financial condition and prospects for growth could suffer.

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

We were incorporated and commenced active operations in 2007. Our operations to date have been focused on organizing and staffing our company, acquiring rights to product candidates, business planning, raising capital and developing Fovista, Zimura and other product candidates. We have not yet demonstrated our ability to successfully complete a large-scale, pivotal clinical trial with data sufficient to obtain marketing approval, apply for and obtain marketing approval, qualify a commercial manufacturer through a pre-approval inspection with respect to any of our products, 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. The failure of our pivotal OPH1002 and OPH1003 Fovista trials for the treatment of wet AMD has required us to reevaluate our future development plans for our product candidates, as well as our business plan more broadly, and has significantly decreased the likelihood that we will commercialize Fovista or any other product in the near term. We may never be successful in developing or commercializing any of our product candidates. If successful in developing and obtaining marketing approval of one of our product candidates, we would need to transition from a company with a product development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

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

Since inception, we have experienced significant cash outflows in funding our operations. As of June 30, 2017, we had an accumulated deficit of $664.3 million. Our net loss was $65.3 million for the six months ended June 30, 2017, and $193.4 million for the year ended December 31, 2016, and we expect to continue to incur significant operating losses for the foreseeable 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, which we entered into in May 2013, our initial public offering, which we closed in September 2013, our follow-on public offering, which we closed in February 2014, and funds we received under the Novartis Agreement, which we entered into in May 2014.

We have devoted substantially all of our financial resources and efforts to the research and development of Fovista and Zimura and preparations for the potential commercial launch of Fovista, including manufacturing scale-up activities. We expect to continue to incur significant expenses and operating losses over the next few years as we implement our updated business plan and continue to implement and reassess our development plans for our existing product candidates. Our net losses may fluctuate significantly from quarter to quarter and year to year.

We currently have two product candidates, Zimura and Fovista, which are in clinical development. We expect to continue to incur significant research and development expenses as we wind-down the OPH1002 and OPH1003 trials and the Fovista Expansion Studies, continue the OPH1004 trial and pursue the development of Zimura as a monotherapy for the treatment of GA, in combination with an anti-VEGF drug for the treatment of wet AMD, in combination with an anti-VEGF drug for the treatment of IPCV and as a monotherapy in orphan indications, including Stargardt disease and non-infectious intermediate and posterior uveitis. We could incur additional research and development expenses if we conclude that there is a scientific rationale for potentially developing, or if we undertake development of, Zimura or Fovista in additional indications, and as we evaluate and potentially in-license or acquire, and undertake development of, additional product candidates. Furthermore, if we successfully develop and obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. For Fovista, our ex-U.S. commercialization partner Novartis is responsible for these commercialization expenses outside the United States. We are party to agreements, specifically a divestiture agreement with OSI (Eyetech), Inc., or Eyetech, 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 Zimura and Fovista. It is likely that any future in-licensing or acquisition agreements that we enter into with respect to additional products, product candidates or technologies would include similar obligations.

We expect that we will continue to incur significant expenses as we:

- continue the clinical development of Zimura as a monotherapy for the treatment of GA, in combination with an anti-VEGF drug for the treatment of wet AMD, in combination with an anti-VEGF drug for the treatment of IPCV, as a monotherapy in orphan indications, including Stargardt disease and non-infectious intermediate and posterior uveitis, or potentially in other indications if we believe there is a sufficient scientific rationale to pursue such development;
- in-license or acquire the rights to, and pursue the development of, other products, product candidates or technologies;
- complete the activities necessary to receive initial, top-line data from OPH1004 and wind down OPH1002, OPH1003 and the Fovista Expansion Studies;
- potentially undertake additional clinical development of Fovista in wet AMD if the initial, top-line data from OPH1004 is favorable or in other indications if we believe there is a sufficient scientific rationale to pursue such development;
- complete our previously announced reduction in personnel;
- maintain, expand and protect our intellectual property portfolio;

- hire additional clinical, manufacturing, quality control, quality assurance and scientific personnel if we are successful in progressing the clinical development of any of our product candidates;

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

- 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 of our product candidates.

Our ability to become and remain profitable depends on our ability to generate revenue in excess of our expenses. Our ability to generate revenues from product sales is dependent on our obtaining marketing approval for and commercializing our product candidates or any product candidates we may in-license or acquire. We may be unsuccessful in our efforts to develop and commercialize product candidates or in our efforts to in-license or acquire additional product candidates. Even if we succeed in developing and commercializing one or more of our product candidates, we may never achieve sufficient sales revenue to achieve or maintain profitability. See "-Risks Related to Product Development and Commercialization" for a further discussion of the risks we face in successfully developing and commercializing our product candidates and achieving profitability.

We may require substantial, additional funding in order to complete the activities necessary to commercialize one or more of our product candidates. 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 June 30, 2017, we had cash, cash equivalents, and marketable securities of $196.4 million, of which approximately $20 million to $35 million is committed to the wind-down of OPH1002 and OPH1003 and the Fovista Expansion Studies, implementing a previously announced reduction in personnel and related costs, cancellation fees related to manufacturing commitments, and obtaining initial, top-line data for the OPH1004 trial by the end of the third quarter of 2017. We estimate that our 2017 year-end cash balance will range between $145 million to $160 million, excluding any potential business development activities or any changes to our current clinical development programs. We also had $350.6 million in total liabilities as of June 30, 2017, of which $331.7 million related to the Novo Agreement and deferred revenue associated with the Novartis Agreement, which we are required to show as liabilities on our balance sheets under generally accepted accounting principles but which, in the case of Novo, do not correspond to any contractual repayment obligation, or in the case of Novartis, are highly unlikely to be triggered.

We believe that our cash, cash equivalents and marketable securities will be sufficient to fund our operations and capital expenditure requirements as currently planned for at least the next 12 months. This estimate does not reflect any additional expenditures resulting from the in-licensing or acquisition of additional product candidates or technologies or associated development that we may pursue following any such transactions. 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 pursuit, by acquisition, in-licensing or otherwise, and subsequent development of additional product candidates or technologies, and the success of our ongoing development programs. We believe that we may need additional funding in the event that we acquire or in-license one or more additional product candidates and undertake development. In addition, our expenses may exceed our expectations if we experience delays in enrollment or with the availability of drug supply for our clinical trials, if we experience any unforeseen issues in our ongoing clinical trials or if we further expand the scope of our clinical trials and programs. Our costs may also exceed our expectations for other reasons, for example, if we experience issues with manufacturing, process development, or if we are required by the FDA, the 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. As a result, we may need or may seek to obtain additional funding in connection with our continuing operations sooner than expected.

The future development of Zimura and Fovista is highly uncertain. If the results from the OPH1004 trial are favorable and we determine to pursue further development of Fovista for use in combination with anti-VEGF drugs for the treatment of wet AMD, we would likely need to conduct one or more further pivotal, clinical trials for Fovista to support potential marketing approval, which would be time-consuming and expensive. Furthermore, we expect the clinical development for Zimura will continue for at least the next several years. At this time, we cannot reasonably estimate the remaining costs necessary to complete the clinical development of either Zimura or Fovista, complete process development and manufacturing scale-up and validation activities associated with Zimura and potentially seek marketing approval for Zimura and Fovista, 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, costs and results of our Zimura clinical programs, including our Phase 2/3 GA clinical trial, our planned Phase 2a wet AMD clinical trial, our planned Phase 2a IPCV clinical trial, our planned randomized, controlled clinical trial in Stargardt disease and our planned Phase 2a clinical trial in non-infectious intermediate and posterior uveitis, as well as any additional clinical trials we undertake to obtain data sufficient to seek marketing approval for Zimura in any indication (including a potential second pivotal trial for GA and for Stargardt disease);
- the extent to which we in-license or acquire rights to, and undertake development of products, product candidates or technologies;
- the amount of any upfront, milestone payments and other financial obligations associated with the in-license or acquisition of other product candidates;
- the scope, progress, results and costs of preclinical development and/or clinical trials for any other product candidates that we may acquire or in-license and develop;
- the scope, progress, costs and results of the OPH1004 trial and any additional clinical trials we undertake to obtain data sufficient to seek marketing approval for Fovista in wet AMD or any other indication;
- if OPH1004 is positive and we decide to pursue further development of Fovista, the costs and timing of restarting the manufacturing of commercial supply for Fovista;
- the costs and timing of process development and manufacturing scale-up and validation activities associated with Zimura;
- our ability to establish collaborations on favorable terms, if at all;
- the costs, timing and outcome of regulatory reviews of our product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending intellectual property-related claims;
- the timing, scope and cost of commercialization activities for any of our product candidates if we receive, or expect to receive, marketing approval for a product candidate; and
- subject to receipt of marketing approval, net revenue received from commercial sales of any of our product candidates, after milestone payments and royalty payments that we would be obligated to make.

Furthermore, following our receipt and announcement of initial, top-line results from our pivotal OPH1002 and OPH1003 Fovista trials for the treatment of wet AMD in December 2016, we implemented a restructuring plan that included a reduction in personnel. This reduction in personnel involves approximately 80% of our pre-announcement workforce and includes employees from nearly every department. We expect to realize estimated annualized cost savings from the reduction in personnel in the range of $20 million to $25 million starting in the third quarter of 2017. During the first six months of 2017, our workforce has been reduced by 98 employees in connection with the reduction in personnel and natural attrition. We expect the reduction of the additional 20 affected employees will be substantially completed during the third quarter of 2017. In connection with such reduction in personnel, we estimate that we will incur approximately $13.4 million of aggregate pre-tax charges through the third quarter of 2017, of which approximately $12.5 million in the aggregate is expected to result in cash expenditures. As of June 30, 2017, our cash expenditures related to such reduction in personnel totaled $8.0 million.

We may not be able to successfully implement the restructuring and we may not realize the planned or expected cost savings benefits, which could adversely affect our estimate of the period for which our cash, cash equivalents and marketable securities will be sufficient to fund our operations and capital expenditure requirements as currently planned.

We do not have any committed external source of funds other than the Novartis Agreement, and the remaining potential milestone payments under the Novartis Agreement are subject to our achievement of specified regulatory and commercial events related to Fovista, none of which are likely to be achieved given the data outcome of the OPH1002 and

OPH1003 trials. Our future commercial revenues, if any, will be derived from sales of any of our product candidates that we are able to successfully develop, which, depending on the product, may not be available for at least several years, if at all. In addition, if approved, Zimura or Fovista or any product that we acquire or in-license may not achieve commercial success. If that is the case, we will need to obtain substantial additional financing to achieve our business objectives. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

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

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

Until such time, if ever, as we can generate substantial product revenues, we may need or may seek to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our existing stockholders' ownership interests would be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our existing stockholders' rights as holders of our common stock. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

We are subject to diligence and other obligations under the Novo Agreement, including an obligation to use commercially reasonable efforts to develop, seek marketing approval for and commercialize Fovista. Our obligations under the Novo Agreement are secured by collateral, which includes certain intellectual property rights, including all of our intellectual property rights relating to Fovista and regulatory approvals, if any, of Fovista. If we fail to satisfy our diligence obligations or breach any other of our obligations under the Novo Agreement and fail to cure the breach within any applicable grace period, Novo A/S could declare an event of default. In such event, Novo A/S could seek to foreclose on the collateral securing our obligations. If Novo A/S successfully does so, we would lose our rights to develop and commercialize Fovista. Our obligations under the Novo Agreement and the pledge of our intellectual property rights in and regulatory approvals, if any, of Fovista as collateral under such agreement may limit our ability to obtain debt financing.

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

We and certain of our current and former executive officers have been named as defendants in lawsuits that could result in substantial costs and divert management's attention.

We and certain of our current and former executive officers have been named as defendants in two purported class action lawsuits initiated earlier this year that generally allege that we and certain of our officers violated Sections 10(b) and/or 20(a) of the Securities Exchange Act of 1934 and Rule 10b-5 promulgated thereunder by making allegedly false and/or misleading statements concerning the prospects of our Phase 3 trials for Fovista in combination with anti-VEGF drugs for the treatment of wet AMD. We and our Board of Directors have also been named as defendants in a shareholder derivative lawsuit initiated during the quarter ended June 30, 2017 that generally alleges similar claims as in the purported class actions. These complaints seek equitable and/or injunctive relief, unspecified damages, attorneys' fees, and other costs. We deny any allegations of wrongdoing and intend to vigorously defend against these lawsuits. We are unable, however, to predict the outcome of these matters at this time. Moreover, any conclusion of these matters in a manner adverse to us and for which we incur substantial costs or damages not covered by our directors' and officers' liability insurance would have a material adverse effect on our financial condition and business. In addition, the litigation could adversely impact our reputation and divert management's attention and resources from other priorities, including the execution of business plans and strategies that are important to our ability to grow our business, any of which could have a material adverse effect on our business. Additional similar lawsuits might be filed.

Risks Related to Product Development and Commercialization

Companies in our industry face a wide range of challenging activities, each of which entails separate, and in many cases substantial, risk.

The long-term success of our company, and our ability to become profitable as a biopharmaceutical company will require us to be successful in a range of challenging activities, including:

- designing, conducting and completing clinical trials for our product candidates;
- obtaining favorable results from required clinical trials, including for each ophthalmic product candidate, favorable results from two adequate and well controlled pivotal clinical trials in the relevant indication;
- applying for and receiving marketing approvals from applicable regulatory authorities for the use of our product candidates;
- making arrangements with third-party manufacturers, receiving regulatory approval of our manufacturing processes and our third-party manufacturers' facilities from applicable regulatory authorities and ensuring adequate supply of drug product;
- establishing sales, marketing and distribution capabilities, either internally or through collaborations or other arrangements, to effectively market and sell our product candidates;
- achieving acceptance of the product candidate, if and when approved, by patients, the medical community and third-party payors;
- if our product candidates are approved, obtaining from governmental and third-party payors adequate coverage and reimbursement for our product candidates and, to the extent applicable, associated injection procedures conducted by treating physicians;
- 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 during development and following approval;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity, including under the Orphan Drug Act of 1983, or the Orphan Drug Act, and the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, if we choose to seek such protections for any of our product candidates;
- protecting and enforcing our rights in our intellectual property portfolio; and
- complying with all applicable regulatory requirements, including FDA Good Clinical Practices, or GCP, Good Manufacturing Practices, or GMP, and standards, rules and regulations governing promotional and other marketing activities.

Each of these activities has associated risks, many of which are detailed below and throughout this "Risk Factors" section. We may never succeed in these activities and, even if we do, may never generate revenues from product sales that are significant enough to achieve commercial success and profitability. Our failure to be commercially successful and profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or continue our operations. A decrease in the value of our company would also cause our stockholders to lose all or part of their investment.

Drug development is a highly uncertain undertaking. Our development efforts may be delayed for any number of reasons, in which case potential marketing approval or commercialization of our product candidates could be delayed or prevented.

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.

We may experience numerous unforeseen events during drug development that could delay or prevent our ability to receive marketing approval or commercialize our product candidates. In particular, clinical trials of our product candidates may produce inconclusive or negative results, such as the results we observed in our pivotal OPH1002 and OPH1003 Fovista trials for the treatment of wet AMD. 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 recent failure of a Phase 2 trial of a competitor evaluating the co-formulation of an approved anti-VEGF drug with an investigational anti-PDGF agent as compared to anti-VEGF monotherapy may call into question the hypothesis underlying the use of combination anti-VEGF and anti-PDGF therapy as a method for treating wet AMD, as well as the potential success of the entire class of PDGF inhibitors that are in development in this disease area, including Fovista. In addition, we have limited data regarding the safety, tolerability and efficacy of Zimura administered for the treatment of GA or administered in combination with anti-VEGF drugs for the treatment of wet AMD or IPCV and no data regarding the safety, tolerability and efficacy of Zimura administered for the treatment of Stargardt disease or non-infectious intermediate and posterior uveitis. The failure of a competitor's Phase 2 clinical trial evaluating an investigational anti-C5 antibody administered via intravitreal injections for the treatment of GA, as well as the failure of a different competitor's Phase 2 clinical trial evaluating an anti-C5 antibody administered systemically for the treatment of GA, may call into question the hypothesis underlying the use of a C5 inhibitor as a method for treating GA. In addition, the anti-C5 antibody administered via intravitreal injections that was studied for the treatment of GA did not show any benefit when studied in a cohort of anti-VEGF treatment-resistant wet AMD patients. Our clinical development programs may fail to produce positive safety or efficacy data that support the use of these product candidates in the indications we are pursuing.

Additional development risks include the following:

- we may not be able to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation of clinical studies for any preclinical product candidates that we in-license or acquire;
- regulators or institutional review boards may not agree with our study design, including our selection of endpoints, or 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 clinical research organizations or clinical trial sites;
- our contract research organizations or clinical trial sites may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we, through our clinical trial sites, may not be able to locate and enroll a sufficient number of eligible patients to participate in our clinical trials as required by the FDA or similar regulatory authorities outside the United States, especially in our clinical trials for orphan or other rare diseases;
- 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, including GCPs, or a finding that the participants are being exposed to unacceptable health risks;
- as there are no therapies approved for either GA or Stargardt disease in either the United States or the European Union, the regulatory pathway for product candidates in these indications is highly uncertain;
- there may be changes in regulatory requirements and guidance or we may have changes in trial design that require amending or submitting new clinical protocols;
- there may be changes in the standard of care on which a clinical development plan was based, which may require new or additional trials;

- we may decide, or regulators may require us, to conduct additional clinical trials beyond those we currently contemplate or to 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;

- 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 Zimura and Fovista in our wet AMD trials, may become insufficient or inadequate or we may face delays in the manufacture and supply of such materials as a result our decision to transfer manufacturing between contract manufacturers or on account of interruptions in our supply chain, including in relation to the packaging and distribution or import / export of clinical materials.

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

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

- not obtain marketing approval at all;

- obtain approval for indications or patient populations that are not as broad as intended or desired;

- obtain approval with labeling that includes significant use limitations, distribution restrictions or safety warnings, including boxed warnings;

- be subject to additional post-marketing testing requirements; or

- have the product removed from the market after obtaining marketing approval.

Despite our current development plans and ongoing efforts, we may not complete any of our ongoing or planned clinical trials or other clinical trials for our product candidates. Moreover, the timing of the completion of, and the availability of results from, clinical trials is difficult to predict. Furthermore, our development plans may change based on feedback we may receive from regulatory authorities throughout the development process. If we experience delays in testing or marketing approvals, our product development costs would increase. 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 our product, we may need to abandon or limit our development of such product candidate.

If any of our product candidates 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 trial, as well as after one year of treatment in our pivotal OPH1002 and OPH1003 trials, based upon our preliminary analysis of safety data from these Phase 3 trials. There remains, however, the potential for patients receiving Fovista combination therapy to experience an increase in cumulative side effects resulting from two separate intravitreal injections and increased intraocular pressure, as compared to patients receiving monotherapy anti-VEGF treatment. We may observe an unfavorable safety and tolerability profile in the Fovista combination therapy arms of OPH1004 or any other clinical

trial of Fovista that we may conduct, as compared to our prior Fovista clinical trials 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 or 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 who receive Fovista combination therapy.

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 combination therapy trials, including OPH1004, are dependent, in part, on the safety and tolerability of the anti-VEGF drug(s) administered in combination with our product candidate. Avastin is not approved for the treatment of wet AMD, and according to some third party clinical trials, may be associated with a greater risk of serious adverse events or undesirable side effects as compared to Lucentis.

We have limited data regarding the safety, tolerability and efficacy of Zimura administered for the treatment of GA or administered in combination with anti-VEGF drugs for the treatment of wet AMD or IPCV and no data regarding the safety, tolerability and efficacy of Zimura administered for the treatment of Stargardt disease or non-infectious intermediate and posterior uveitis. Our clinical trials for Zimura will involve multiple intravitreal injections over an extended period of time and, as such, may involve risks regarding multiple and chronic intravitreal injections, similar to those described above for Fovista.

Our experience manufacturing our product candidates is limited. Manufacturing issues may arise that could cause delays in our development programs or increase costs. In addition, we may experience delays in regulatory approval of our product candidates if we do not satisfy applicable manufacturing regulatory requirements.

Our current product candidates, Zimura and Fovista, are each chemically-synthesized aptamers. In pursuing our business development activities, we could acquire or in-license a variety of types of product candidates, including small molecule drugs, protein drugs or biologics. Small molecule drugs are organic compounds of low molecular weight that are generally associated with ready availability of starting materials and ease of synthesis. In contrast, manufacturing for proteins and biologics is more complex, especially in large quantities. For example, biologic products must be made consistently and in substantial compliance with a clearly defined manufacturing process, and often must be manufactured under aseptic conditions.

We do not have any internal manufacturing capabilities and likely would be dependent on outside contract manufacturers to manufacture any of the product candidates that we would acquire or in-license as part of pursuing our updated business plan. Manufacturing for these product candidates could be complicated or present novel technical challenges. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet FDA, EMA or other applicable standards or specifications with consistent and acceptable production yields and costs.

We currently rely upon a single third-party manufacturer, Agilent Technologies, to supply us with the chemically synthesized aptamers comprising the API for both Zimura and Fovista and a different, single third-party manufacturer, Ajinomoto Althea, to provide fill/finish services for both Zimura and Fovista. In order to obtain and maintain regulatory approval for Zimura or Fovista, our third-party manufacturers will be required to consistently produce the API used in Zimura or Fovista in commercial quantities and of specified quality and to execute fill/finish services on a repeated basis and document their ability to do so. If the third-party manufacturers are unable to satisfy this requirement, our business would be materially and adversely affected. During the third quarter of 2016, we completed the manufacture of validation batches of Fovista API produced at commercial scale. However, given the negative results from our pivotal OPH1002 and OPH1003 Fovista trials for the treatment of wet AMD, we are not currently manufacturing clinical or commercial supplies of Fovista. If OPH1004 is positive and we decide to pursue further development of Fovista, we would eventually need to restart the process for commercial supply, including obtaining scheduling commitments from both Agilent and Althea which could prove challenging if these manufacturers are at or near capacity. To date, we have not yet scaled up the manufacturing process for Zimura beyond the scale used for developmental clinical batches, nor have we validated the manufacturing process.

These manufacturing processes and the facilities of our third-party manufacturers, including our third-party API manufacturer and our third-party fill/finish service provider, are subject to inspection and approval by the FDA, referred to as a pre-approval inspection, before we can commence the commercial sale of any approved product candidate, and thereafter on an ongoing basis. Our third-party API manufacturer has undergone only one pre-approval inspection by the FDA, and has not yet gone through a pre-approval inspection for Zimura or Fovista. Our third-party fill/finish service provider 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 delays in the approval of our applications for marketing approval in the event a recommendation to withhold is issued, as well as 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.

Some of 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 are no established generally accepted manufacturing or quality standards for the production of Zimura or Fovista. Even though the FDA has reviewed the quality standards for Fovista 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, Zimura or any future product candidate.

In addition, in order to manufacture and supply any of our product candidates on a commercial scale in the future, we will need to bolster our quality control and quality assurance capabilities, including by augmenting our manufacturing processes and adding personnel. We also may encounter problems hiring and retaining the experienced specialist scientific and manufacturing personnel needed to operate our manufacturing process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements. 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 or stability of the product, and we may be required to refine or alter our manufacturing processes to address these issues. Resolving these issues could result in significant delays and may result in significantly increased costs. If we underestimate the demand for an approved product, given the long lead times required to manufacture or obtain regulatory approvals for our products, we could potentially face commercial drug product supply shortages. If we experience significant delays or other obstacles in producing any approved product at commercial scale, our ability to market and sell any approved products may be adversely affected and our business could suffer.

Any problems in our manufacturing process or our third-party contract manufacturers' facilities could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in our manufacturing process or facilities also could restrict our ability to meet market demand for our products.

Even if any of our product candidates 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 any of our product candidates, if approved, do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of Fovista, Zimura or any other product candidate that we may develop, if approved for commercial sale, will depend on a number of factors, including:

- efficacy and potential advantages compared to alternative treatments, including the existing standard of care;
- any restrictions in the label on the use of our products in combination with other medications;
- any restrictions in the label on the use of our products by a subgroup of patients;

- restrictions in the label on of any for our combination therapy product candidates, such as Zimura or Fovista, limiting their use in combination with particular standard of care drugs, such as a particular anti-VEGF drug;

- our and any commercialization partner's ability to offer our products at competitive prices, particularly in light of the cost of any of our combination therapy product candidates in addition to the cost of the underlying standard of care drug;

- availability of third-party coverage and adequate reimbursement, particularly by Medicare given the target market for AMD indications for persons over age 55;

- increasing reimbursement pressures on treating physicians 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; and

- 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.

In addition, the potential market opportunity for any product candidate is difficult to estimate precisely. Our estimates of the potential market opportunity for our product candidates include several key assumptions based on our expectations of the safety and effectiveness of the relevant product candidate, 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 and any of these assumptions could prove to be inaccurate, and the actual market for such product candidates could be smaller than our estimates of our potential market opportunity.

With respect to our programs for orphan diseases, our understanding of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. These estimates may prove to be incorrect and new studies may reduce the estimated incidence or prevalence of these diseases. The number of patients in the United States, the European Union and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our products or patients may become increasingly difficult to identify and access, all of which would adversely affect our business, financial condition, results of operations and prospects.

We face substantial competition, which may result in others 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 our product candidates 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, GA, Stargardt disease or other disease indications for which we may develop Zimura or Fovista. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. We also will face similar competition with respect to any other products or product candidates that we may seek to develop or commercialize in the future for the treatment of wet AMD, GA, Stargardt disease or any other indication we may pursue.

There are a number of products in preclinical research and clinical development by third parties to treat wet AMD. Based on publicly available information, we are aware that Ohr Pharmaceutical, Inc., Santen Inc., Tyrogenex, Inc., Allergan plc and GrayBug Vision, Inc. each have PDGF inhibitors in clinical or pre-clinical development for wet AMD. Several of these product candidates also inhibit VEGF or are administered directly with an anti-VEGF agent in a manner that could negatively

impact demand for a separate intravitreal injection of an anti-PDGF agent such as Fovista. Ohr Pharmaceutical recently announced plans to amend its ongoing clinical trial to enable efficacy analyses by the end of 2017 or early 2018.

Moreover, based on publicly available information, we are aware that several companies and research organizations are pursuing treatments targeting other molecular targets, potential gene therapy treatments and stem cell transplant treatments for the treatment of wet AMD. In addition, other companies are undertaking efforts to develop technologies to allow for a less frequent dosing schedule than the dosing schedule currently in use for standard of care anti-VEGF drugs.

There are a number of products in preclinical research and clinical development by third parties to treat dry AMD. In general, these product candidates can be categorized based on their proposed mechanisms of action. The mechanisms of action for these product candidates include inflammation suppression, such as complement system inhibitors and corticosteroids, visual cycle modulators, antioxidants and neuroprotectants, cell and gene therapies and vascular enhancers. Based on publicly available information, we are aware that Genentech, Inc., a member of the Roche Group, Novartis AG and MorphoSys AG, Apellis Pharmaceuticals, Inc., Hemera Biosciences, Inc., Achillion Pharmaceuticals, Inc., Ra Pharmaceuticals, Inc. and Catalyst Biosciences, Inc. each have complement inhibitors in development, the most advanced of which is Genentech's humanized Fab fragment targeting complement factor D, which is at a different part of the complement cascade than complement factor C5. Data from Genentech's Phase 3 trials is expected during the second half of 2017. If Genentech's Phase 3 trials for its complement factor D product candidate are successful, it is likely that Genentech would obtain marketing approval for such product candidate several years in advance of when we could reasonably expect marketing approval for Zimura in GA, if at all. Moreover, based on publicly available information, we are aware that several other companies have announced development programs for the treatment of dry AMD targeting different mechanisms of action outside of the complement cascade.

There are a number of products in preclinical research and clinical development by third parties to treat Stargardt disease. Based on publicly available information, we are aware that Sanofi, Acucela Inc., Alkeus Pharmaceuticals, Inc. and Vision Medicines, Inc. each have development programs in Stargardt disease. Three of these programs, Acucela, Alkeus and Vision Medicines, are exploring the use of oral therapeutics, while Sanofi, with technology provided by Oxford BioMedica plc, is using a gene therapy approach. Acucela's, Alkeus's and Sanofi's product candidates are each in Phase 2 development.

In the case of orphan diseases such as Stargardt disease, should we be successful in development, our commercialization efforts may rely on non-patent market exclusivity periods under the Orphan Drug Act and the Hatch-Waxman Act. There are limited circumstances under each of the Orphan Drug Act and the Hatch-Waxman Act that could result in our loss of data and marketing exclusivity, which could allow a competitor to enter the market. Failure to maintain either data or market exclusivity period would have a material adverse effect on our ability to commercialize our product candidates.

In addition, 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 our product candidates. The commercial opportunity for Zimura or 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.

Furthermore, 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 Zimura or Fovista is approved for combination therapy for the treatment of wet AMD, the cost of combination treatment would be higher than the cost of treatment of wet AMD with Lucentis, Eylea or particularly Avastin monotherapy. Insurers and other third-party payors may encourage the use of anti-VEGF drugs as monotherapy and discourage the use of Fovista in combination with these drugs. This could limit sales of Fovista.

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

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

As a company, we have no experience in the sale, marketing or distribution of pharmaceutical products. We currently do not have any sales, marketing or distribution infrastructure. 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. We expect that our commercial strategy for any of our product candidates, including whether to retain commercial rights and market and sell the product candidate ourselves or to utilize collaboration, distribution or other marketing arrangements with third parties, would be determined based on a variety of factors, including the size and nature of the patient population, the disease area, the particular indication, the territory in which the product candidate may be marketed and the commercial potential for such product candidate. 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. In addition, our commercial strategy would vary depending on whether the disease is typically treated by general ophthalmology practitioners, specialists, such as retinal specialists, or sub-specialists.

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

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

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

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

If Fovista is successfully developed, which, again, we believe has a low likelihood following our receipt of initial, top-line data from two of our three pivotal clinical trials, and 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 would be costly.

Even if we are able to commercialize any of the product candidates 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. Health care reform is an issue of intense political focus, particularly in the United States. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals or in ways that could alter the mechanism by which pharmaceutical prices are negotiated or otherwise determined. 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 or negotiation 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 and are widely accepted and prescribed or used by physicians.

Our ability and the ability of any commercialization partner, such as 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 and encouraging the substitution of lower cost or generic products. Pricing pressures recently experienced by the pharmaceutical industry may be further exacerbated by legislative and policy changes under consideration by the Trump administration. For example, the new Administration has expressed an interest in authorizing and/or directing the Center for Medicare & Medicaid Service or other agencies of the U.S. government to negotiate prices for drugs covered by Medicare directly with pharmaceutical companies. If this were to occur, especially for wet AMD drugs where a large portion of the patient population is over the age of 65 and is therefore covered by Medicare, there could be significant downward pressure on prices charged, not only for patients covered by Medicare, but also for patients covered by private insurers who may follow the government's lead on price. Moreover, 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 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, a policy that President Trump has expressed interest in pursuing. Third-party payors often rely upon Medicare coverage policy

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

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

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

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

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

We currently hold $10.0 million in product liability insurance coverage in the aggregate, with a per incident limit of $10.0 million, which may not be adequate to cover all liabilities that we may incur. We will need to increase our insurance coverage when and if we begin commercializing an approved product. 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

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

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

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

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

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

We may utilize a variety of types of collaboration, distribution and other marketing arrangements with third parties to commercialize our product candidates, either in the United States, or in markets outside the United States, such as the Novartis Agreement. 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 would likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements and our arrangement with Novartis for Fovista will depend on our collaborators' and Novartis's abilities and efforts to successfully perform the functions assigned to them in these arrangements. Collaborations involving our product candidates, including our collaboration with Novartis, could pose numerous risks to us, including the following:

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

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

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

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

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

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

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

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

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

For example, the Novartis 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. 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 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. 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 would 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 would 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 a collaborator of ours, such as 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 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 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 our product candidates. 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 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 would not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and would 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 Zimura and Fovista for clinical trials and expect to continue to do so in connection with the potential commercialization of either product candidate 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 Zimura or Fovista and have limited personnel with manufacturing experience. We currently rely upon and expect to continue to rely upon third-party contract manufacturers to manufacture preclinical and clinical supplies of product candidates we are developing or 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 the product candidates that we are developing or may develop may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis. In addition, any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. Under the Novartis Agreement, we are responsible for supplying to Novartis Fovista API for clinical and commercial supply.

We currently rely exclusively upon a single third-party manufacturer to provide supplies of both Zimura API and Fovista API and a different single third-party manufacturer to provide fill/finish services for Zimura and Fovista. Although we have agreements in place with Agilent for the supply of Fovista API and with Althea for clinical and commercial fill/finish services, we do not currently have any contractual commitments for the supply of Zimura API. Additionally, given the negative results from our pivotal OPH1002 and OPH1003 Fovista trials for the treatment of wet AMD, we are not currently manufacturing commercial supplies of Fovista. If OPH1004 is positive and we decide to pursue further development of Fovista, we would eventually need to restart the process for commercial supply, including obtaining scheduling commitments from both Agilent and Althea which could prove challenging if these manufacturers are at or near capacity. We also do not currently have arrangements in place for redundant supply or a second source for API for Zimura or Fovista or for a redundant supply or a second source for fill/finish services. The prices for manufacturing activities that are not yet contractually committed may vary substantially over time and adversely affect our financial results. Furthermore, we and our contract manufacturers 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 Zimura and Fovista.

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 any of our third-party manufacturers fail to fulfill our purchase orders, or if any of these manufacturers should become unavailable to us for any reason, including as a result of capacity constraints, financial difficulties or insolvency, we believe that there are a limited number of potential replacement manufacturers, and we likely would incur added costs and delays in identifying or qualifying such replacements. We could also incur additional costs and delays in identifying or qualifying a replacement manufacturer for fill/finish services 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.

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

- our product candidates 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, quality assurance and quality control;
- 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 product candidates and technologies. Termination of these rights or the failure by us or our licensees, including our commercialization or collaboration partners to comply with obligations under these or other agreements under which we obtain such rights or have obtained funding could materially harm our business and prevent us from developing or commercializing our products and product candidates.

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

We also have diligence and development obligations under our divestiture agreement with OSI Pharmaceuticals and our license agreements with Archemix and Nektar. Generally, these diligence obligations require us to use commercially reasonable efforts to develop, seek regulatory approval for and commercialize our products in the United States, the European

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

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

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

- Under the terms of the amended license, manufacturing and supply agreement with Nektar, pursuant to which we obtained, among other licenses, an exclusive, worldwide license to make, develop, use, import, offer for sale and sell certain products that incorporate a specified PEG reagent linked with the active ingredient in Fovista, if we fail to use commercially reasonable efforts to achieve the first commercial sale of Fovista in the United States 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 would be in material breach of the agreement and Nektar would 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. 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.

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 expired in early 2017, which is well in advance of any marketing approval we may ultimately receive for Fovista. 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 Hatch-Waxman Act permits a patent restoration term of up to five years as partial compensation for patent term effectively lost during product development and the FDA regulatory review process occurring after the issuance of a patent. We may be able to obtain a patent term extension for this U.S. patent. The European patent rights covering the composition of matter of Fovista are expected to expire in 2018. Such expiration date is before the date by which we expect Fovista to be commercialized in Europe. 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 would 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 patent applications in the United States covering a method of treating wet AMD in patients with Fovista in combination with Eylea and patent applications in Europe and Japan covering a combination of Fovista and Eylea for use in a method for treating wet AMD. We also have filed patent applications in the United States, Europe, Japan and elsewhere covering our formulation for Fovista. These patent applications are in the early stages of prosecution and may not result in patents being issued that protect the use of Fovista in combination with Eylea for treating wet AMD, that protect our formulation for Fovista or that effectively prevent others from commercializing competitive technologies and products. If a patent is granted following prosecution of any such application for the combination of Fovista and Eylea, the latest projected patent expiry, absent any patent term adjustment or extension or patent restoration, would be in 2030. If a patent is granted following prosecution of any such application for our formulation for Fovista, the latest projected patent expiry, absent any patent term adjustment or extension or patent restoration, would be in 2034.

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 Zimura or Fovista 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 Zimura or Fovista, if approved.

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

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

The issuance of a patent is not conclusive as to its inventorship, ownership, scope, term, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. For example, if we receive marketing approval for our product candidates, other pharmaceutical companies may seek approval of generic versions of our products with the FDA or regulatory authorities in other jurisdictions. We may then be required to initiate proceedings against such companies in order to enforce our intellectual property rights. The risk of being involved in such proceedings is likely to increase if our products are commercially successful. In any such proceedings, the inventorship, ownership, scope, term, validity and enforceability of our patents may be challenged. These and other challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to prevent others from using or commercializing similar or identical technology and products or from launching generic versions of our products, or could limit the duration of the patent protection of our technology and products. The launch of a generic version of one of our products in particular would be likely to result in an immediate and substantial reduction in the demand for our product, which could have a material adverse effect on our business. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

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

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation.

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

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

If we are or one of our collaboration or commercialization partners is found to infringe or otherwise violate a third party's intellectual property rights, we or they could be required to obtain a license from such third party to continue developing and marketing our or their products and technology or to continue using a trademark. However, we or our collaboration and commercialization partners may not be able to obtain any required license on commercially reasonable terms or at all. Even if we or they were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us or our collaboration and commercialization partners and could require us or them to make substantial licensing and royalty payments. We or our collaboration and commercialization partners could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages, including treble 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' and their successors' 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' and their successors' practices, and those of their predecessors, with regard to the protection of Fovista-related trade secrets before we acquired them. Any party with whom we or they have executed a non-disclosure and confidentiality agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Our proprietary information may also be obtained by third parties by other means, such as breaches of our physical or computer security systems.

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

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

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of some or all of our product candidates. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize our product candidates, and our ability to generate revenue would be materially impaired.

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

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. 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, in the case of Fovista, Novartis, to assist us in this process. Securing marketing approval requires obtaining positive safety and efficacy data from required clinical trials, as well as the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authorities. The FDA or other regulatory authorities may determine that Fovista, Zimura or any other product candidate that we may develop is not effective, is only moderately effective or has undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use. The FDA or other regulatory authority may limit the approval of Zimura or Fovista to use with only specified anti-VEGF drugs rather than with all anti-VEGF drugs. Such limitation could limit sales of Zimura or 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 Zimura and Fovista 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.

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

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

In order to market and sell our product candidates 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.

Additionally, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. On March 29, 2017, the country formally notified the European Union of its intention to withdraw pursuant to Article 50 of the Lisbon Treaty. Since a significant proportion of the regulatory framework in the United Kingdom is derived from European Union directives and regulations, the referendum could materially impact the regulatory regime with respect to the approval of our product candidates in the United Kingdom or the European Union. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for our product candidates, which could significantly and materially harm our business.

We currently do not have orphan drug designations or orphan drug exclusivity for any product. If our competitors are able to obtain orphan drug exclusivity for products that constitute the same drug and treat the same indications as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

Regulatory authorities in some jurisdictions, including the United States and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, the EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than 5 in 10,000 persons in the European Union. Additionally, orphan designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biologic product.

If we request orphan drug designation for any of our product candidates in one or more indications, there can be no assurances that the FDA or the European Commission will grant any of our product candidates such designation. Additionally, the designation of any of our product candidates as an orphan product does not guarantee that any regulatory agency will accelerate regulatory review of, or ultimately approve, that product candidate, nor does it limit the ability of any regulatory agency to grant orphan drug designation to product candidates of other companies that treat the same indications as our product candidates prior to our product candidates receiving exclusive marketing approval.

Generally, if a product candidate with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the European Commission from approving another marketing application for a product that constitutes the same drug treating the same indication for that marketing exclusivity period, except in limited circumstances. If another sponsor receives such approval before we do (regardless of our orphan drug designation), we will be precluded from receiving marketing approval for our product for the applicable exclusivity period. The applicable period is seven years in the United States and 10 years in the European Union. The exclusivity period in the United States can be extended by six months if the sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different drugs can be approved for the same condition. In the United States, even after an orphan drug is approved, the FDA may subsequently approve another drug for the same condition if the FDA concludes that the latter drug is not the same drug or is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

- the second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;
- the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- the holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product.

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

In the United States, our lead product candidate, Fovista, received fast track designation for wet AMD 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.

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 would receive marketing approval.

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

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

Any product candidate 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 for which we or our commercialization partners obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include

submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to manufacturing, quality control and quality assurance, complaints and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or may be subject to significant conditions of approval or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the medicine, including the requirement to implement a risk evaluation and mitigation strategy.

The FDA and other agencies, including the Department of Justice, or the DOJ, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA and DOJ impose stringent restrictions on manufacturers' communications regarding off-label use and if we do not market our products for their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug, and Cosmetic Act and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription drugs may lead to investigations and enforcement actions alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws.

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

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on distribution or use of a product;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- damage to relationships with any potential collaborators;
- unfavorable press coverage and damage to our reputation;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure;
- injunctions or the imposition of civil or criminal penalties; and
- litigation involving patients using our products.

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

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

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

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

- the federal False Claims Act imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented, false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties, currently set at $5,500 to $11,000 per false claim;

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

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

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

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

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

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

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

The efforts of the Trump Administration to pursue regulatory reform may limit the FDA's ability to engage in oversight and implementation activities in the normal course, and that could negatively impact our business.

The Trump Administration has taken several executive actions, including the issuance of a number of executive orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. On January 30, 2017, President Trump issued an executive order, applicable to all executive agencies, including the FDA, that requires that for each notice of proposed rulemaking or final regulation to be issued in fiscal year 2017, the agency shall identify at least two existing regulations to be repealed, unless prohibited by law. These requirements are referred to as the "two-for-one" provisions. This executive order includes a budget neutrality provision that requires the total incremental cost of all new regulations in the 2017 fiscal year, including repealed regulations, to be no greater than zero, except in limited circumstances. For fiscal years 2018 and beyond, the executive order requires agencies to identify regulations to offset any incremental cost of a new regulation. In interim guidance issued by the Office of Information and Regulatory Affairs within the Office of Management and Budget on February 2, 2017, the administration indicates that the "two-for-one" provisions may apply not only to agency regulations, but also to significant agency guidance documents. It is difficult to predict how these requirements will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

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

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, 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 for which we or they obtain marketing approval. 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 may receive for any approved products.

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

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription products and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for products that are inhaled, infused, instilled, implanted or injected;
- expansion of healthcare fraud and abuse laws, including the civil False Claims Act and the federal Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand products to eligible beneficiaries during their

coverage gap period, as a condition for the manufacturer's outpatient products to be covered under Medicare Part D;

- extension of manufacturers' Medicaid rebate liability to individuals enrolled in Medicaid managed care organizations;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements to report certain financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report product samples that manufacturers and distributors provide to physicians;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research;
- a new Independent Payment Advisory Board, or IPAB, which has authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription products; and
- establishment of the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2024 unless additional Congressional action is taken. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

With the new Administration and Congress, there will likely be additional administrative or legislative changes, including modification, repeal, or replacement of all, or certain provisions of, the ACA. In January 2017, Congress voted to adopt a budget resolution for fiscal year 2017, or the Budget Resolution, that authorizes the implementation of legislation that would repeal portions of the ACA. The Budget Resolution is not a law, however, it is widely viewed as the first step toward the passage of legislation that would repeal certain aspects of the ACA. Further, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices.

At the same time, Congress has focused on additional legislative changes, including in particular repeal and replacement of certain provisions of the ACA. To those ends, on May 4, 2017, the U.S. House of Representatives passed the American Health Care Act and the Senate is currently considering legislative proposals leading to new healthcare reform legislation. It remains to be seen, however, whether new legislation repealing and replacing the ACA is enacted and, if so, precisely what the new legislation will provide, when it will be enacted and what impact it will have on the availability of healthcare and containing or lowering the cost of healthcare. It is possible that these repeal and replacement initiatives, if enacted into law, could ultimately result in fewer individuals having health insurance coverage or in individuals having insurance coverage with less generous benefits. It is also possible that some ACA provisions that generally are not favorable for the research-based pharmaceutical industry could also be repealed along with ACA coverage expansion provisions. At this point, healthcare reform and its impacts on the Company are highly uncertain in many respects.

The costs of prescription pharmaceuticals in the United States has also been the subject of considerable discussion in the United States, and members of Congress and the Administration have stated that they will address such costs through new

legislative and administrative measures. The pricing of prescription pharmaceuticals is also subject to governmental control outside 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 may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidates 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 ability to generate revenues and become profitable could be impaired.

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

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

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

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

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

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

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

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

Risks Related to Employee Matters and Managing Our Operations

We are in the process of implementing a substantial reduction in personnel, which could disrupt our operations. In addition, we may experience difficulties in retaining key employees that we have identified for retention.

In December 2016, following our receipt and announcement of initial, top-line results from our pivotal OPH1002 and OPH1003 Fovista trials for the treatment of wet AMD, we announced that we had determined to implement a reduction in personnel to focus on an updated business plan. This reduction in personnel involves approximately 80% of our pre-announcement workforce and includes personnel from nearly every department. During the first six months of 2017, our workforce has been reduced by 98 employees in connection with the reduction in personnel and natural attrition. We expect the reduction of the additional 20 affected employees will be substantially completed during the third quarter of 2017. Although we are reducing our personnel substantially, we are continuing to function as a development company and need to continue all or nearly all of our prior business functions to support such development, including clinical operations, regulatory affairs, drug safety, data management, outsourced manufacturing and supply chain, analytical development and quality assurance, as well as all of our general and administrative functions and public company infrastructure. Due to our limited financial resources and the inherent challenges associated with managing such a reduction in personnel, we may not be able to manage effectively the reduction in personnel and transition of operations to remaining employees. In addition, as part of implementing our reduction in personnel, we have issued notices of termination under our existing office leases, each of which now is scheduled to terminate in late 2017 or early 2018. It is possible that we may not be able to find suitable replacement office space for our remaining employees on acceptable terms, or at all, which could harm our operating results and/or materially disrupt our operations.

Notwithstanding the reduction in personnel, we remain highly dependent on David R. Guyer, M.D., our Executive Chairman, and Glenn P. Sblendorio, our Chief Executive Officer and President, as well as the other principal members of our management, scientific and clinical teams. We do not maintain “key person” insurance for any of our executives or other employees. Although we have entered into letter agreements with our executive officers, each of them and our non-executive employees may terminate their employment with us at any time. As a result, key employees that we expect to retain through specific dates to assist with transition activities may choose not to remain employees. In addition, we may experience difficulties in retaining key employees that we have identified for retention, given the change in prospects for our company as well as other challenges. 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 updated business strategy. Furthermore, replacing any such 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 strategically attract or retain high quality personnel as we implement our new business plan, our ability to pursue our development strategy would be limited.

In connection with the reduction in personnel, we expect that we will incur approximately $13.4 million of aggregate pre-tax charges through the third quarter of 2017, of which approximately $12.5 million in the aggregate is expected to result in cash expenditures. As of June 30, 2017, our cash expenditures related to such reduction in personnel totaled $8.0 million. The reduction in personnel may divert our management’s time and attention. Any inability to manage the reduction in personnel could delay the execution of our updated business plan or disrupt our operations.

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

A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the company’s annual or interim financial statements would not be prevented or detected on a timely basis. If any material weakness in our internal control over financial reporting is discovered or occurs in the future, our financial statements may contain material misstatements and we could be required to restate our financial results. As previously disclosed in July 2015, our management concluded that we experienced a material weakness in internal controls related to technical accounting expertise over the accounting for deferred tax assets and income tax accounting in general during certain prior financial reporting periods. The deficiency in the application of our controls

relating to technical accounting expertise over the accounting for deferred tax assets and income tax accounting in general resulted in the audit committee of our board of directors concluding that the relevant financial statements should not be relied upon, and our subsequent restatement of the relevant financial statements.

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

Risks Related to Information Technology

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

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

Risks Related to Our Common Stock

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 and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. 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, including results of clinical trials of product candidates of our competitors;
- results of clinical trials for our product candidates and the timing of the receipt of such results;
- 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;
- political, 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;

- 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.

For example, following our announcement of initial, top-line results from our pivotal OPH1002 and OPH1003 Fovista trials for the treatment of wet AMD, the closing price of our common stock declined from $38.77 on December 9, 2016 to $5.29 on December 12, 2016. In addition, following the announcement by Regeneron on September 30, 2016 that its co-formulated anti-PDGF/Eylea product candidate failed to meet the primary endpoint of a Phase 2 clinical trial at 12 weeks, the closing price of our common stock declined from $54.12 on September 29, 2016 to $30.85 on November 3, 2016. Following periods of volatility in the market price of a company's stock, securities class-action litigation has often been instituted against that company. We and certain of our current and former executive officers have been named as defendants in purported class action lawsuits and a derivative lawsuit following our announcement of the initial, top-line results. See "Part II, Item 1-Legal Proceedings" and "-Risks Related to Our Updated Business Plan, Financial Position and Need for Additional Capital-We and certain of our current and former executive officers have been named as defendants in lawsuits that could result in substantial costs and divert management's attention." This proceeding and other similar 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.

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. Moreover, we have filed registration statements on Form S-8 registering all shares of common stock that we may issue under our equity compensation plans. 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.

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

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

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to furnish with our periodic Exchange Act reports a report by our management on our internal control over financial reporting. We are also required to include with our annual report an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404, we must document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources and engage outside consultants to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. There is a risk that our internal control over financial reporting may, in the future, be found to be ineffective under Section 404. Our identification of one or more material weaknesses could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

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

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

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

Recent Sales of Unregistered Securities

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

Purchase of Equity Securities

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

Item 5. Other Information.

None.

Item 6. Exhibits.

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

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: August 2, 2017

By: /s/ David F. Carroll

David F. Carroll
Chief Financial Officer
(Principal Financial and Accounting Officer)

EXHIBIT INDEX

Exhibit Number	Description of Exhibit
10.1	Letter Agreement between the Registrant and David R. Guyer, dated April 24, 2017
10.2	Letter Agreement between the Registrant and Glenn P. Sblendorio, dated April 24, 2017
10.3	Letter Agreement between the Registrant and David F. Carroll, dated April 25, 2017
31.1	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS*	XBRL Instance Document
101.SCH*	XBRL Taxonomy Extension Schema Document
101.CAL*	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF*	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE*	XBRL Taxonomy Extension Presentation Linkbase Document

* Submitted electronically herewith.

Attached as Exhibit 101 to this report are the following formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheets at June 30, 2017 and December 31, 2016 (unaudited), (ii) Statements of Operations (unaudited) for the three and six month periods ended June 30, 2017 and 2016, (iii) Statements of Cash Flows (unaudited) for the six month period ended June 30, 2017 and 2016 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.

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

April 24, 2017

Dr. David R. Guyer
c/o Ophthotech Corporation
One Penn Plaza
New York, NY 10119

Dear David:

Subject to your execution below, this letter hereby further amends the employment letter, dated April 26, 2013, between you and Ophthotech Corporation (the "Company") and as amended by the letter dated February 26, 2015 between you and the Company (as amended, the "Employment Letter") by making the following changes:

1. Section 1 of the Employment Letter is hereby replaced in its entirety by the following:

1. **Employment.** Effective immediately, you will continue to be employed on a full time basis as the Company's Chief Executive Officer, reporting to the Company's Board of Directors (the "Board"), and you shall have the duties, responsibilities and authority commensurate with your position in companies of similar type and size; provided, however, that it is understood that among such responsibilities shall be your assistance, as reasonably directed by the Board, with the transition of such responsibilities to a new Chief Executive Officer effective July 1, 2017. Effective July 1, 2017, you will be employed to serve on a full time basis as the Executive Chairman of the Board. As the Company's Executive Chairman, you will report to the Board and you shall have the duties, responsibilities and authority commensurate with your position in companies of similar type and size. You will continue while employed as Executive Chairman to be nominated to serve on the Board each time your term(s) as a director would otherwise expire, provided that such nomination(s) shall be subject to the Board's exercise of its fiduciary duties. You agree to devote your full business time, efforts, skill, knowledge, attention and energies to the advancement of the Company's business and interests and to the performance of your duties and responsibilities as an employee of the Company. Notwithstanding the foregoing, you shall be permitted to continue serving on the boards of directors of other companies, provided in each case that such service (a) does not entail an operating role, does not materially interfere with the performance of your duties and responsibilities to the Company, and does not compete with the Company and your role as provided in Section 16, and (b) shall, with respect to public company boards, be limited if, as determined by the Board, such service exceeds generally prevailing

"overboarding" limits for non-chief executive officer directors. For purposes hereof, a business will be deemed to be competitive with the Company if it engages in the research, development or commercialization of pharmaceutical or diagnostic products for ocular diseases with the same primary mechanism of action as any compound or drug that is at any such time of determination under active research or development or being commercialized by the Company or any of its subsidiaries (whether the Company or any such subsidiary currently has or in the future acquires rights to such compound or drug). In addition, you shall be permitted to provide consulting services to companies that are not competitive with the Company, provided that such services do not materially interfere with the performance of your duties and responsibilities as an employee of the Company. You agree to furnish a summary of the time you spend providing service as a consultant to the Board upon request. You further agree to abide by the rules, regulations, instructions, personnel practices and policies of the Company and any changes therein not inconsistent with this letter that may be adopted from time to time by the Company. In the event that, due to future business activities of the Company or of another entity to which you are providing services, a competitive situation arises, you shall promptly, and in any event within ten (10) business days of gaining knowledge thereof, discuss the same with the Board and, if requested by the Board, resign from the other competitive activities as soon as reasonably feasible and, in the interim, recuse yourself from discussion of competitive matters.

2. Section 2 of the Employment Letter is hereby replaced in its entirety by the following:

2. **Base Salary.** Through December 31, 2017, your base salary will be at the rate of $24,038.46 per bi-weekly pay period (which if annualized equals $625,000), less all applicable taxes and withholdings. Effective January 1, 2018, your base salary will be at the rate of $20,192.31 per bi-weekly pay period (which if annualized equals $525,000), less all applicable taxes and withholdings. Base salary will be paid in installments in accordance with the Company's regular payroll practices.

3. Section 3 of the Employment Letter is hereby replaced in its entirety by the following:

3. **Discretionary Bonus.** Following the end of calendar year 2017 and subject to the approval of the Board, you will be eligible for a performance bonus of up to 65% of your annualized base salary, based on your personal performance and the Company's performance during the 2017 calendar year, as determined by the Board in its sole discretion. Following the end of each calendar year beginning with calendar year 2018 and subject to the approval of the Board, you will be eligible for a performance bonus of up to 50% of your annualized base salary, based on your personal performance and the Company's performance during the applicable calendar year, as determined by the Board in its sole discretion. In any event, you must be an active employee of the Company on the date the bonus is distributed in order to be eligible for and to earn any bonus award, as it also serves as an incentive to remain employed by the Company, except as otherwise provided herein.

4. Section 6 of the Employment Letter is hereby replaced in its entirety by the following:

6. **Severance.** If your employment is terminated by the Company, or, if applicable, its successor, without Cause or by you for any reason, then (subject to your executing (and not revoking) a separation agreement as described below) the Company, or its successor, will (i) pay you an amount equal to twelve (12) months of your base salary (at the greater of (x) an annualized base salary rate of $625,000 or (y) your then-current annualized base salary rate), less standard employment-related withholdings and deductions, which amount shall be paid to you in a lump sum on the Payment Date (as defined below), (ii) pay you a pro-rated portion of the bonus to which you would otherwise be entitled pursuant to Section 3 hereof for the year in which your employment terminates (at the greater of a 65% target bonus rate or your then-current target bonus rate, and without regard to whether the performance goals with respect to such target bonus have been established or met), less standard employment-related withholdings and deductions, which amount shall be paid to you on the Payment Date, and (iii) provide for continued coverage, at the Company's expense, under the Company's medical and dental benefit plans to the extent permitted under such plans for a period of twelve (12) months immediately following the date of the termination of your employment. The Company shall not be obligated to pay to you the severance payments provided for herein unless you have timely executed (and not revoked) a separation agreement in substantially the form attached hereto. Such separation agreement must be executed and become binding and enforceable within sixty (60) calendar days after the effective date of your termination of employment (such 60th day, the "Payment Date"); *provided, however,* that if the 60th day following the date of termination occurs in the next calendar year following the date of termination, then the Payment Date shall be no earlier than January 1 of such following calendar year and, if applicable, shall be subject to Section 17. You shall also be entitled to (A) prompt payment in accordance with the Company's regular payroll practices of any unpaid base salary and accrued unused vacation time in accordance with Company policy through the date of your termination, (B) if earned and unpaid, payment of any prior year bonus at such time as it would otherwise be paid to Company employees, (C) vested benefits under Company benefit plans in accordance with the terms of such plans, and (D) vesting and payment, as may be applicable, of equity grants and/or retention bonuses in accordance with the terms of the plans and/or other documents governing such grants and/or bonuses.

If your employment is terminated by the Company or, if applicable, its successor without Cause or by you for Good Reason within twelve months following a Change in Control Event (as defined in the Company's 2013 Stock Incentive Plan), then (subject to your executing (and not revoking) a separation agreement as described in the immediately preceding paragraph) the Company or its successor will, in addition to the severance payments set forth in the immediately preceding paragraph, provide that any then unvested equity awards held by you that vest solely based on the passage of time shall immediately vest in full and become exercisable or free from forfeiture or repurchase, as applicable; provided, however, that this equity award acceleration provision shall not

supersede or replace any other provision in an agreement covering an equity award granted to you by the company that is at least as beneficial to you.

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

For purposes hereof, "Good Reason" shall mean, without your written consent: (i) any change in your position, title or reporting relationship with the Company that diminishes in any material respect your title, authority, duties or responsibilities, including your removal as a member of the Board; (ii) any material reduction in your base compensation; (iii) a material change in the primary geographic location at which services are to be performed by you (unless the new location is closer to your primary residence than the prior location); or (iv) a material breach of any provision hereof by the Company or any successor or assign. Notwithstanding the foregoing, "Good Reason" shall not be deemed to have occurred unless: (A) you provide the Company with written notice that you intend to terminate your employment hereunder for one of the grounds set forth in subsections (i), (ii), (iii) or (iv) of the immediately preceding sentence within sixty (60) days of such reason(s) occurring, (B) if such ground is capable of being cured, the Company has failed to cure such ground within a period of thirty (30) days from the date of such written notice, and (C) you terminate your employment within six (6) months from the date that Good Reason first occurs. For purposes of clarification, the above-listed conditions shall apply separately to each occurrence of Good Reason and failure to adhere to such conditions in the event of Good Reason shall not disqualify you from asserting Good Reason for any subsequent occurrence of Good Reason.

5. The form of separation agreement previously attached to the Employment Letter as the "Separation Agreement and Release of Claims," is hereby replaced in its entirety with the form attached to this amendment as Exhibit A. The form of release required in connection with the provision to you of any retention bonus or grant shall be deemed modified to the extent necessary for you not to release thereunder any rights you may

have to an annual bonus or equity grant that has vested or remains subject to vesting due to your continued employment with the Company.

6. Section 9 of the Employment Letter is hereby replaced in its entirety by the following:

   9. **Invention, Non-Disclosure, Non-Competition and Non-Solicitation Agreement.** Section 4(b) of the Invention, Non-Disclosure, Non-Competition and Non-Solicitation Agreement dated April 26, 2013 that you previously executed for the benefit of the Company is hereby amended as follows: (b) As used herein, a business will be deemed "Competitive" with the Company if it engages in the research, development or commercialization of pharmaceutical or diagnostic products for ocular diseases with the same primary mechanism of action as any compound or drug that is at any such time of determination under active research or development or being commercialized by the Company or any of its subsidiaries (whether the Company or any such subsidiary currently has or in the future acquires rights to such compound or drug).

7. Section 12 of the Employment Letter is hereby replaced in its entirety by the following:

   12. **At-Will Employment.** This letter shall not be construed as an agreement, either express or implied, to employ you for any stated term, and shall in no way alter the Company's policy of employment at-will, under which both the Company and you remain free to end the employment relationship for any reason, at any time, with or without cause or notice. Although your job duties, title, compensation and benefits, as well as the Company's personnel policies and procedures, may change from time to time, the "at-will" nature of your employment may only be changed by a written agreement signed by you and an authorized representative of the Board that expressly states the intention to modify the at-will nature of your employment. Similarly, nothing in this letter shall be construed as an agreement, either express or implied, to pay you any compensation or grant you any benefit beyond the end of your employment with the Company. This letter supersedes all prior understandings, whether written or oral, relating to the terms of your employment.

The Company agrees to reimburse you for your reasonable attorneys' fees and expenses in connection with reviewing and negotiating the terms of this amendment in an amount not to exceed $10,000.

You hereby agree that you and the Company are executing this amendment by mutual agreement, that you hereby consent to the changes described herein, and that nothing herein shall constitute grounds for "Good Reason" as defined in Employment Letter. In the event of any conflict between the terms of this amendment and the terms of the Employment Letter, the terms of this amendment shall control. Except as expressly modified herein, the terms of the Employment Letter remain in full force and effect. This amendment may only be modified in a document signed by both the Company and you. This amendment may be executed in counterparts, each of which will be deemed an original, but all of which will be deemed one and the same instrument.

[*Remainder of page intentionally left blank*]

If this amendment is acceptable to you, please sign and date this amendment below and return the signed and dated amendment to me on or before April 24, 2017 at 12:00 p.m., Eastern time.

Sincerely,

OPHTHOTECH CORPORATION

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

ACCEPTED AND AGREED:

/s/ David R. Guyer
Dr. David R. Guyer

Date: 4/24/2017

Exhibit A

SEPARATION AGREEMENT AND RELEASE OF CLAIMS

Ophthotech Corporation, a Delaware corporation (the "Company"), and David Guyer (the "Employee") (together, the "Parties"), entered into a letter agreement dated April 26, 2013, as amended February 26, 2015 and April 24, 2017 (as amended, the "Employment Letter"). Any capitalized terms not defined herein shall have the meanings ascribed to them in the Employment Letter. This is the release by Employee of all claims against the Releasees (as defined below) arising out of the Employee's employment with or separation from the Company (the "Release"). The consideration for the Employee's agreement to this Release consists of the severance payments set forth in Section 6 of the Employment Letter, which are conditioned on, among other things, termination of the Employee's employment by the Company without Cause or by the Employee for any reason and effectiveness of this Release based on the Employee's timely execution and nonrevocation hereof.

1. Tender of Release. This Release is automatically tendered to the Employee upon the termination of the Employee's employment by the Company without Cause or by the Employee for any reason.

2. Release of Claims. The Employee voluntarily, fully, forever, irrevocably and unconditionally releases and discharges the Company, its affiliates, subsidiaries and parent companies and each of their predecessors, successors, assigns, and their current and former members, partners, directors, managers, officers, employees, representatives, attorneys, agents, and all persons acting by, through, under or in concert with any of the foregoing (any and all of whom or which are hereinafter referred to as the "Releasees"), from any and all charges, complaints, claims, liabilities, obligations, promises, agreements, controversies, damages, actions, causes of action, suits, rights, demands, costs, losses, debts and expenses (including attorney's fees and costs actually incurred), of any nature whatsoever, known or unknown that the Employee now has, owns or holds, or claims to have, own, or hold, or that he at any time had, owned, or held, or claimed to have had, owned, or held against any Releasee arising out of the Employee's employment with or separation from the Company (collectively, "Claims"). This release of Claims includes, without implication of limitation, the release of all Claims:

    - of breach of contract;

    - of retaliation or discrimination under federal, state or local law (including, without limitation, Claims of age discrimination or retaliation under the Age Discrimination in Employment Act, Claims of disability discrimination or retaliation under the Americans with Disabilities Act, Claims of discrimination or retaliation under Title VII of the Civil Rights Act of 1964 and Claims of discrimination or retaliation under state law);

- under any other federal or state statute, to the fullest extent that Claims may be released;
- of defamation or other torts;
- of violation of public policy;
- for wages, salary, bonuses, vacation pay or any other compensation or benefits; and
- for damages or other remedies of any sort, including, without limitation, compensatory damages, punitive damages, injunctive relief and attorney's fees.

Notwithstanding anything to the contrary contained herein, this Release does not apply to or affect (i) the Employee's right to receive the severance payments set forth in Section 6 of the Employment Letter, (ii) the Employee's ownership of, and the Employee's rights by virtue of his ownership of, any capital stock or other securities of the Company, including vested equity grants, (iii) the Employee's rights under the retention letter agreement dated January 17, 2017 and documents referenced therein, or (iv) the Indemnification Agreement between the Company and the Employee dated June 2, 2016, any other rights of indemnification or exculpation of which the Employee is the beneficiary under the corporate charter, bylaws or other charter or organizational instruments or benefit or equity plans of the Company or any other Releasee or at law and rights of coverage to which the Employee may be entitled under any director and officer liability insurance policy of the Company or any other Releasee. Further, nothing in this Release prevents Employee from filing a charge with, cooperating with, or participating in any investigation or proceeding before, the Equal Employment Opportunity Commission or a state fair employment practices agency (except that Employee acknowledges that he may not recover any monetary benefits in connection with any such charge, investigation, or proceeding, and Employee further waives any rights or claims to any payment, benefit, attorneys' fees or other remedial relief in connection with any such charge, investigation or proceeding).

3. Ongoing Obligations of the Employee; Enforcement Rights. The Employee reaffirms his ongoing obligations as well as the Company's enforcement rights provided for in the Invention, Non-Disclosure, Non-Competition and Non-Solicitation Agreement between the Company and the Employee dated April 26, 2013, as amended (the "NDA").

4. Scope of Disclosure Restrictions. Nothing in this Release, or in the NDA or elsewhere, prohibits Employee from communicating with government agencies about possible violations of federal, state, or local laws or otherwise providing information to government agencies, filing a complaint with government agencies, or participating in government agency investigations or proceedings. Employee is not required to notify the Company of any such communications; provided, however, that nothing herein authorizes the disclosure of information Employee obtained through a communication that was subject to the attorney-client privilege. Further, notwithstanding Employee's

confidentiality and nondisclosure obligations, Employee is hereby advised as follows pursuant to the Defend Trade Secrets Act: "An individual shall not be held criminally or civilly liable under any Federal or State trade secret law for the disclosure of a trade secret that (A) is made (i) in confidence to a Federal, State, or local government official, either directly or indirectly, or to an attorney; and (ii) solely for the purpose of reporting or investigating a suspected violation of law; or (B) is made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal. An individual who files a lawsuit for retaliation by an employer for reporting a suspected violation of law may disclose the trade secret to the attorney of the individual and use the trade secret information in the court proceeding, if the individual (A) files any document containing the trade secret under seal; and (B) does not disclose the trade secret, except pursuant to court order."

5. No Assignment. The Employee represents that he has not assigned to any other person or entity any Claims against any Releasee.

6. Right to Consider and Revoke Release. The Employee acknowledges that he has been given the opportunity to consider this Release for a period ending twenty-one (21) days after the tender of the Release. In the event the Employee executed this Release within less than twenty-one (21) days after the tender of the Release, he acknowledges that such decision was entirely voluntary and that he had the opportunity to consider this Release until the end of the twenty-one (21) day period. To accept this Release, the Employee shall deliver a signed Release to the Chairman of the Compensation Committee of the Board (the "Chair") within such twenty-one (21) day period. For a period of seven (7) days from the date when the Employee executes this Release (the "Revocation Period"), he shall retain the right to revoke this Release by written notice that is received by the Chair on or before the last day of the Revocation Period. This Release shall take effect only if it is executed within the twenty-one (21) day period as set forth above and if it is not revoked pursuant to the preceding sentence. If those conditions are satisfied, this Release shall become effective and enforceable on the date immediately following the last day of the Revocation Period.

7. Other Terms.

    a. Legal Representation; Review of Release. The Employee acknowledges that he has been advised to discuss all aspects of this Release with his attorney, that he has carefully read and fully understands all of the provisions of this Release and that he is voluntarily entering into this Release.

    b. Binding Nature of Release. This Release shall be binding upon the Employee and upon his heirs, administrators, representatives and executors.

    c. Modification of Release; Waiver. This Release may be amended, only upon a written agreement executed by the Employee and the Company.

d. Severability. In the event that at any future time it is determined by an arbitrator or court of competent jurisdiction that any covenant, clause, provision or term of this Release is illegal, invalid or unenforceable, the remaining provisions and terms of this Release shall not be affected thereby and the illegal, invalid or unenforceable term or provision shall be severed from the remainder of this Release. In the event of such severance, the remaining covenants shall be binding and enforceable.

e. Governing Law and Interpretation. This Release shall be deemed to be made and entered into in the State of New York and shall in all respects be interpreted, enforced and governed under the laws of the State of New York, without giving effect to the conflict of laws provisions of New York law that would require the application of law of any other jurisdiction. The language of all parts of this Release shall in all cases be construed as a whole, according to its fair meaning, and not strictly for or against either of the Parties.

f. Entire Agreement; Absence of Reliance. The Employee acknowledges that he is not relying on any promises or representations by the Company or its agents, representatives or attorneys of either of them regarding any subject matter addressed in this Release.

So agreed by the Employee:

_____ _____

Dr. David Guyer Date

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

April 24, 2017

Glenn Sblendorio
c/o Ophthotech Corporation
One Penn Plaza
New York, NY 10119

Dear Glenn:

Subject to your execution below, this letter hereby amends the employment letter, dated January 4, 2016, between you and Ophthotech Corporation (the "Company") (the "Employment Letter") by making the following changes:

1. Section 1 of the Employment Letter is hereby replaced in its entirety by the following:

1. **Employment.** Effective immediately, you will continue to be employed on a full time basis as the Company's President. As the Company's President, you will report to the Company's Board of Directors (the "Board"), and you shall have the duties, responsibilities and authority commensurate with your position in companies of similar type and size; provided, however, that it is understood that among such responsibilities shall be your assistance, as directed by the Board, with the transition to other personnel (as designated by the Board) of the responsibilities you had in your former position of Executive Vice President, Chief Operating Officer, Chief Financial Officer and Treasurer, and your assistance with your own transition to the position of Chief Executive Officer of the Company. Effective July 1, 2017, you will be employed to serve on a full time basis as the Company's Chief Executive Officer, while also continuing to serve as the Company's President. As the Company's Chief Executive Officer and President, you will report to the Board and you shall have the duties, responsibilities and authority commensurate with your position in companies of similar type and size. The Board has nominated you to serve as a member of the Board, with your election subject to stockholder approval at the 2017 annual meeting of stockholders. You shall perform and discharge faithfully and diligently your duties and responsibilities hereunder. You agree to devote your full business time, efforts, skill, knowledge, attention and energies to the advancement of the Company's business and interests and to the performance of your duties and responsibilities as an employee of the Company. Notwithstanding the foregoing, you may continue to serve as a member of the boards of directors of Intercept Pharmaceuticals, Inc. and Amicus Therapeutics, Inc., may serve on civic, charitable,

educational, religious, public interest or public service boards, may provide *de minimis* consulting services, and may manage your personal and family investments, in each case, to the extent such activities, whether individually or in the aggregate, do not materially interfere or conflict with the performance of your duties and responsibilities for the Company and do not compete with the Company. You agree to furnish a summary of the time you spend providing service as a consultant to the Board upon request. For purposes hereof, a business or activity will be deemed to be competitive with the Company if it engages in the research, development or commercialization of pharmaceutical or diagnostic products for ocular diseases with the same primary mechanism of action as any compound or drug that is at any such time of determination under active research or development or being commercialized by the Company or any of its subsidiaries (whether the Company or any such subsidiary currently has or in the future acquires rights to such compound or drug). You further agree to abide by the rules, regulations, instructions, personnel practices and policies of the Company and any changes therein that may be adopted from time to time by the Company.

2. Section 2 of the Employment Letter is hereby replaced in its entirety by the following:

2. **Base Salary.** Through June 30, 2017, your base salary will be at the rate of $19, 038.46 per bi-weekly pay period (which if annualized equals $495,000), less all applicable taxes and withholdings. Effective July 1, 2017, your base salary will be at the rate of $24,038.46 per bi-weekly pay period (which if annualized equals $625,000), less all applicable taxes and withholdings. Base salary will be paid in installments in accordance with the Company's regular payroll practices.

3. Section 3 of the Employment Letter is hereby replaced in its entirety by the following:

3. **Discretionary Bonus.** Following the end of each calendar year and subject to the approval of the Board, you will be eligible for a performance bonus of up to 65% of your annualized base salary (the "Target Bonus"), based on your personal performance and the Company's performance during the applicable calendar year, as determined by the Board in its sole discretion. In any event, you must be an active employee of the Company on the date the bonus is distributed in order to be eligible for and to earn any bonus award, as it also serves as an incentive to remain employed by the Company.

4. Section 8 of the Employment Letter is hereby replaced in its entirety by the following:

8. **Invention, Non-Disclosure, Non-Competition and Non-Solicitation Agreement.** As a condition of your continued employment under the terms and conditions set forth herein, you will be required to execute the Invention, Non-Disclosure, Non-Competition and Non-Solicitation Agreement attached hereto as Exhibit A.

5. Section 11 of the Employment Letter is hereby replaced in its entirety by the following:

11. **At-Will Employment.** This letter shall not be construed as an agreement, either

express or implied, to employ you for any stated term, and shall in no way alter the Company's policy of employment at-will, under which both the Company and you remain free to end the employment relationship for any reason, at any time, with or without cause or notice. Although your job duties, title, compensation and benefits, as well as the Company's personnel policies and procedures, may change from time to time, the "at-will" nature of your employment may only be changed by a written agreement signed by you and a Company representative duly authorized by the Board that expressly states the intention to modify the at-will nature of your employment. Similarly, nothing in this letter shall be construed as an agreement, either express or implied, to pay you any compensation or grant you any benefit beyond the end of your employment with the Company. This letter supersedes all prior understandings, whether written or oral, relating to the terms of your employment, except that it does not supersede the January 4, 2016 letter between you and the Company with respect to severance benefits (the "Severance Letter").

You hereby agree that you and the Company are executing this amendment by mutual agreement, that you hereby consent to the changes described herein, and that nothing herein shall constitute grounds for "Good Reason" as defined in the Severance Letter. In the event of any conflict between the terms of this amendment and the terms of the Employment Letter, the terms of this amendment shall control. Except as expressly modified herein, the terms of the Employment Letter remain in full force and effect. This amendment may only be modified in a document signed by both the Company and you. This amendment may be executed in counterparts, each of which will be deemed an original, but all of which will be deemed one and the same instrument.

[*Remainder of page intentionally left blank*]

If this amendment is acceptable to you, please sign and date this amendment below and return the signed and dated amendment to me on or before April 24, 2017 at 12:00 p.m., Eastern time.

Sincerely,

OPHTHOTECH CORPORATION

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

ACCEPTED AND AGREED:

/s/ Glenn Slendorio
Glenn Sblendorio

Date: 4/24/2017

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

April 24, 2017

Mr. David Carroll
c/o Ophthotech Corporation
One Penn Plaza, Suite 19th Floor
New York, NY 10119

Dear Dave:

The board of directors (the "Board") of Ophthotech Corporation (the "Company") has provided for the following severance benefits to be provided to you in the event of your termination of employment with the Company, on the terms and conditions set forth herein. The severance arrangement set forth herein replaces in its entirety Section 6 of your offer letter dated June 8, 2016 (the "Offer Letter").

1. **Severance.**

(a) Subject to Section 1(b), if your employment is terminated (1) at any time by the Company without Cause or by you for Good Reason (as such terms are herein defined) or (2) within one year following a Change in Control Event (as defined in the Company's 2013 Stock Incentive Plan), by the Company, or its successor, without Cause or by you for Good Reason, the Company or its successor will (i) pay you in a lump sum on the Payment Date (as herein defined) (A) an amount equal to nine (9) months of your then-current base salary, less standard employment-related withholdings and deductions and (B) an amount equal to a pro-rated portion of your then-current target bonus for the year in which your employment terminates, provided, however, that if your employment is terminated under the circumstances described in (2) of this Section 1(a), the Company or its successor will instead pay you an amount equal to your then-current target bonus for the year in which your employment terminates, in either case, without regard to whether the performance goals with respect to such target bonus have been established or met and less standard employment-related withholdings and deductions, and (ii) provided you elect to continue your and your eligible dependents' participation in the Company's medical and dental benefit plans pursuant to the Consolidated Omnibus Budget Reconciliation Act of 1986 ("COBRA"), reimburse you for the monthly premium to continue such coverage for the lesser of the nine (9) full calendar months immediately following the month in which the termination of your employment occurs and the end of the calendar month in which you become eligible to receive group health plan coverage under another employee benefit plan. Notwithstanding the foregoing, if the reimbursement of monthly premiums would otherwise violate the nondiscrimination rules or cause the reimbursement of claims to be taxable under the Patient Protection and Affordable Care Act of 2010, together with the Health Care and Education Reconciliation Act of 2010 (collectively, the "Healthcare Reform Act") or Section 105(h) of the Internal Revenue Code of 1986, as amended (the "Code"), these payments shall be treated as taxable payments to you and you shall be subject to imputed income tax treatment to the extent necessary to eliminate any discriminatory treatment or taxation under the Act or Section 105(h).

(b) Notwithstanding the foregoing, (i) the Company shall not be obligated to pay you the severance payments provided for herein unless you have timely executed (and not revoked) a separation agreement in a form to be provided by the Company. Such separation agreement must be executed and become binding and enforceable within sixty (60) calendar days after the effective date of your termination of employment (such 60th day, the "Payment Date"); provided however, that if the 60th day following the date of termination occurs in the next calendar year following the date of termination, then the Payment Date shall be no earlier than January 1 of such following calendar year and (ii) the severance payments provided for herein shall be reduced by any payments to which you may be entitled as a result of any applicable laws regarding plant closings or mass layoffs which require notice prior to termination or pay or benefits following termination, such that the amount of the payments made to you pursuant to such laws (whether such payments are made to you during any notice period prior to termination (regardless of whether the Company requires you to work during such notice period) or any period following termination), will reduce the severance payments otherwise due to you under Section 1(a).

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

(d) For purposes hereof, "Good Reason" shall mean, without your written consent: (i) any change in your position or reporting relationship with the Company that diminishes in any material respect your authority, duties or responsibilities; (ii) any material reduction in your base compensation; (iii) a material change in the primary geographic location at which services are to be performed by you (unless the new location is closer to your primary residence than the prior location); or (iv) a material breach of any provision hereof by the Company or any successor or assign. Notwithstanding the foregoing, "Good Reason" shall not be deemed to have occurred unless: (A) you provide the Company with written notice that you intend to terminate your employment hereunder for one of the grounds set forth in subsections (i), (ii), (iii) or (iv) of the immediately preceding sentence within sixty (60) days of such reason(s) occurring, (B) if such ground is capable of being cured, the Company has failed to cure such ground within a period of thirty (30) days from the date of such written notice, and (C) you terminate your employment within six (6) months from the date that Good Reason first occurs. For purposes of clarification, the above-listed conditions shall apply separately to each occurrence of Good Reason and failure to adhere to such conditions in the event of Good Reason shall not disqualify you from asserting Good Reason for any subsequent occurrence of Good Reason.

2. **Equity Acceleration.** If your employment with the Company, or its successor, is terminated by the Company or such successor without Cause or by you for Good Reason within the one (1) year period following a Change in Control Event, then the then-unvested portion of any equity awards

held by you that vest solely based on the passage of time shall immediately vest in full and become exercisable or free from forfeiture or repurchase, as applicable, as of the date of such termination.

3. **Modified Cutback.**

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

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

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

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

(ii) "Contingent Compensation Payment" shall mean any payment (or benefit) in the nature of compensation that is made or made available (under this Agreement or otherwise) to a "disqualified individual" (as defined in Section 280G(c) of the Code) and that is contingent (within the meaning of Section 280G(b)(2)(A)(i) of the Code) on a Change in Ownership or Control of the Company.

(d) Any payments or other benefits otherwise due to you following a Change in Ownership or Control that could reasonably be characterized (as determined by the Company) as Contingent Compensation Payments (the "Potential Payments") shall not be made until the dates provided for in this Section 3(d). Within 30 days after each date on which you first become entitled to receive (whether or not then due) a Contingent Compensation Payment relating to such Change in Ownership or Control, the Company shall determine and notify you (with reasonable detail regarding the basis for its

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

(e) The Contingent Compensation Payments to be treated as Eliminated Payments shall be determined by the Company by determining the "Contingent Compensation Payment Ratio" (as defined below) for each Contingent Compensation Payment and then reducing the Contingent Compensation Payments in order beginning with the Contingent Compensation Payment with the highest Contingent Compensation Payment Ratio. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio, such Contingent Compensation Payment shall be reduced based on the time of payment of such Contingent Compensation Payments with amounts having later payment dates being reduced first. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio and the same time of payment, such Contingent Compensation Payments shall be reduced on a pro rata basis (but not below zero) prior to reducing Contingent Compensation Payment with a lower Contingent Compensation Payment Ratio. The term "Contingent Compensation Payment Ratio" shall mean a fraction the numerator of which is the value of the applicable Contingent Compensation Payment that must be taken into account by you for purposes of Section 4999(a) of the Code, and the denominator of which is the actual amount to be received by you in respect of the applicable Contingent Compensation Payment. For example, in the case of an equity grant that is treated as contingent on the Change in Ownership or Control because the time at which the payment is made or the payment vests is accelerated, the denominator shall be determined by reference to the fair market value of the equity at the acceleration date, and not in accordance with the methodology for determining the value of accelerated payments set forth in Treasury Regulation Section 1.280G-1Q/A-24(b) or (c)).

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

4. **Miscellaneous.**

(a) Code Section 409A. The intent of the parties is that payments and benefits under this letter comply with, or be exempt from, Internal Revenue Code Section 409A and the regulations and guidance promulgated thereunder (collectively "Code Section 409A"). Accordingly, if any provision of this letter is ambiguous, such that one interpretation would subject a payment or benefit to the excise tax imposed by Code Section 409A and an alternative interpretation would not so subject the payment or benefit, the parties intend the interpretation that would not so subject the payment or benefit to apply. With regard to any provision herein that provides for reimbursement of costs and expenses or in-kind benefits, except as permitted by Code Section 409A, (i) the right to reimbursement or in-kind benefits shall not be subject to liquidation or exchange for another benefit, (ii) the amount of expenses eligible for reimbursement, or in-kind benefits, provided during any taxable year shall not affect the expenses eligible for reimbursement, or in-kind benefits to be provided, in any other taxable year, provided that this clause (ii) shall not be violated with regard to expenses reimbursed under any arrangement covered by Section 105(a) of the Code solely because such expenses are subject to a limit related to the period the arrangement is in effect, and (iii) such payments shall be made on or before the last day of your taxable year following the taxable year in which the expense occurred, provided that any tax gross-ups may be reimbursed by the end of the calendar year following the calendar year in which such taxes are remitted to the taxing authorities. For purposes of Code Section 409A, each payment hereunder shall be treated as a separate payment and your right to receive any installment payments pursuant to this Agreement shall be treated as a right to receive a series of separate and distinct payments. In no event may you, directly or indirectly, designate the calendar year of any payment to be made under this letter that is considered nonqualified deferred compensation. Termination of employment as used herein shall mean separation from service within the meaning of Code Section 409A. In the event at the time of any separation from service you are a "specified employee" within the meaning of Code Section 409A, any deferred compensation subject to Code Section 409A payable as a result of such termination shall not be paid prior to the earlier of six (6) months after such termination and your death and shall be paid immediately thereafter.

(b) Governing Law. This letter shall be governed by and construed in accordance with the laws of the State of New York (without reference to the conflicts of laws provisions thereof). Any action, suit, or other legal proceeding which is commenced to resolve any matter arising under or relating to any provision of this letter shall be commenced only in a court of the State of New York (or, if appropriate, a federal court located within New York), and the Company and you each consents to the jurisdiction of such a court. The Company and you each hereby irrevocably waive any right to a trial by jury in any action, suit or other legal proceeding arising under or relating to any provision hereof.

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

[*Remainder of page intentionally left blank*]

If the provisions of this agreement are acceptable to you, please sign and date this agreement below and return the signed and dated agreement to me on or before April 25, 2017.

Sincerely,

OPHTHOTECH CORPORATION

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

ACCEPTED AND AGREED:

/s/ David Carroll
David Carroll

Date: 4/25/2017

Exhibit 31.1

CERTIFICATIONS

I, Glenn P. Sblendorio, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the quarter ended June 30, 2017 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: August 2, 2017

By: /s/ Glenn P. Sblendorio

Glenn P. Sblendorio
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, David F. Carroll, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the quarter ended June 30, 2017 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: August 2, 2017

By: /s/ David F. Carroll

David F. Carroll
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,

AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of Ophthotech Corporation (the "Company") for the period ended June 30, 2017 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Glenn P. Sblendorio, 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: August 2, 2017

By: /s/ Glenn P. Sblendorio

Glenn P. Sblendorio
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 June 30, 2017 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, David F. Carroll, 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: August 2, 2017

By: /s/ David F. Carroll

David F. Carroll
Chief Financial Officer
(Principal Financial Officer)