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

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2018

Or

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

For the transition period from to

Commission file number: 001-36080

OPHTHOTECH CORPORATION

(Exact name of registrant as specified in its charter)

Delaware	**20-8185347**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
One Penn Plaza, 35th 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 o No

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

As of May 8, 2018 there were 36,188,161 shares of Common Stock, $0.001 par value per share, outstanding.

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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 business plan and strategy to develop Zimura® (avacincaptad pegol) in orphan ophthalmic indications and age-related retinal diseases and potentially expand our product pipeline, including through collaborative gene therapy research programs;
- 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, and ability to obtain, additional financing;
- the timing, costs, conduct and outcome of our ongoing and planned clinical trials, 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 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;
- 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)

	March 31, 2018	December 31, 2017
Assets		
Current assets		
Cash and cash equivalents	$ 154,911	$ 166,972
Prepaid expenses and other current assets	2,836	3,146
Income tax receivable	—	1,387
Total current assets	157,747	171,505
Property and equipment, net	468	518
Deferred tax assets	3,296	3,529
Other assets	40	24
Total assets	$ 161,551	$ 175,576
Liabilities and Stockholders' Equity		
Current liabilities		
Accrued research and development expenses	$ 5,196	$ 4,984
Accounts payable and accrued expenses	3,278	7,551
Total current liabilities	8,474	12,535
Royalty purchase liability	125,000	125,000
Total liabilities	133,474	137,535
Stockholders' equity		
Preferred stock—$0.001 par value, 5,000,000 shares authorized, no shares issued or outstanding	$ —	$ —
Common stock—$0.001 par value, 200,000,000 shares authorized, 36,163,811 and 36,110,298 shares issued and outstanding at March 31, 2018 and December 31, 2017, respectively	36	36
Additional paid-in capital	525,868	522,759
Accumulated deficit	(497,827)	(484,754)
Total stockholders' equity	28,077	38,041
Total liabilities and stockholders' equity	$ 161,551	$ 175,576

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 March 31,	
	2018	2017
Collaboration revenue	$ —	$ 1,662
Operating expenses:		
Research and development	7,686	31,979
General and administrative	5,645	13,159
Total operating expenses	13,331	45,138
Loss from operations	(13,331)	(43,476)
Interest income	473	378
Other expense	(16)	(21)
Loss before income tax provision	(12,874)	(43,119)
Income tax provision	199	3
Net loss	$ (13,073)	$ (43,122)
Net loss per common share:		
Basic and diluted	$ (0.36)	$ (1.20)
Weighted average common shares outstanding:		
Basic and diluted	36,153	35,804

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

Ophthotech Corporation

Unaudited Statements of Comprehensive Loss

(in thousands)

	Three Months Ended March 31,	
	2018	2017
Net loss	$ (13,073)	$ (43,122)
Other comprehensive loss:		
Unrealized loss on available for sale securities, net of tax	—	(10)
Other comprehensive loss	—	(10)
Comprehensive loss	$ (13,073)	$ (43,132)

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

Ophthotech Corporation

Unaudited Statements of Cash Flows

(in thousands)

	Three Months Ended March 31,	
	2018	2017
Operating Activities		
Net loss	$ (13,073)	$ (43,122)
Adjustments to reconcile net loss to net cash (used in) provided by operating activities		
Depreciation	50	808
Amortization of premium and discounts on investment securities	—	98
Deferred income taxes	233	—
Share-based compensation	3,082	6,064
Changes in operating assets and liabilities:		
Income tax receivable	1,387	(52)
Prepaid expense and other current assets	310	(1,520)
Accrued interest receivable	—	74
Other assets	(16)	459
Accrued research and development expenses	212	(19,285)
Accounts payable and accrued expenses	(4,273)	(3,375)
Deferred revenue	—	(1,661)
Net cash used in operating activities	(12,088)	(61,512)
Investing Activities		
Purchase of marketable securities	—	(12,014)
Maturities of marketable securities	—	32,119
Net cash provided by investing activities	—	20,105
Financing Activities		
Proceeds from employee stock plan purchases and stock option exercises	27	31
Net cash provided by financing activities	27	31
Net change in cash and cash equivalents	(12,061)	(41,376)
Cash and cash equivalents		
Beginning of period	166,972	133,930
End of period	$ 154,911	$ 92,554
Supplemental disclosure of cash paid		
Income taxes paid (received), net	$ (1,425)	$ —
Supplemental disclosures of non-cash information related to investing activities		
Change in unrealized loss on available for sale securities, net of tax	$ —	$ (10)

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

Ophthotech Corporation

NOTES TO UNAUDITED FINANCIAL STATEMENTS

(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 science-driven biopharmaceutical company specializing in the development of novel therapies to treat ophthalmic diseases, with a focus on age-related and orphan retinal diseases. The Company's multi-track strategy is to leverage its clinical experience and retina expertise to develop therapies for large market, age-related retinal diseases, where unmet medical needs remain for these patients, and for orphan eye diseases with a focus on underserved patients, and to utilize a disciplined business development approach to obtain additional products, product candidates and technologies in these disease areas. The Company is developing Zimura® (avacincaptad pegol), its complement C5 inhibitor, for dry and wet forms 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 loss of central vision, and autosomal recessive Stargardt disease, or STGD1, which is an orphan inherited retinal disease that also may result in loss of central and peripheral vision. The Company is actively engaged in extensive business development efforts to identify, evaluate and potentially obtain rights to and develop additional therapeutic and gene therapy product candidates that would complement its strategic goals and leverage its competitive advantages. The Company believes that its strategy will provide multiple potential opportunities to bring ophthalmic therapies to market.

2. Summary of Significant Accounting Policies

The Company’s significant accounting policies are described in Note 2, “Summary of Significant Accounting Policies,” in the notes to the audited consolidated financial statements included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2017 filed with the Securities and Exchange Commission (SEC) on March 5, 2018.

Basis of Presentation

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

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 March 31, 2018, the Company had cash and cash equivalents of approximately $154.9 million.

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. The Company did not hold any available for sale securities at March 31, 2018 or December 31, 2017.

Revenue Recognition

Collaboration Revenue

Prior to 2018, the Company's revenue resulted from payments received under its May 2014 licensing and commercialization agreement (the “Novartis Agreement”) with Novartis Pharma AG ("Novartis"), as modified by the July 2017 letter agreement entered into by the Company and Novartis in relation to the Novartis Agreement (the "Letter Agreement"). See "Note 5—Licensing and Commercialization Agreement with Novartis Pharma AG" below for a description of these agreements. The Company used the relative selling price method to allocate arrangement consideration to the Company’s performance obligations under the Novartis Agreement. The Company completed the deliverables under the Novartis Agreement and the Letter Agreement during the third quarter of 2017. On October 23, 2017, following the failure of the Phase 3 Fovista program and pursuant to the terms of the Letter Agreement, Novartis elected to terminate the Novartis Agreement with immediate effect.

As the Company has no products approved for sale, the Company will not receive any revenue from any product candidates that it develops until it obtains regulatory approval and commercializes such products, or until the Company potentially enters into agreements with third parties for the development and commercialization of product candidates. If the Company's development efforts for any of its product candidates result in regulatory approval or the Company enters into collaboration agreements with third parties, the Company may generate revenue from product sales or from such third parties.

In the future, the Company will evaluate revenue contracts and arrangements, if any, following the provisions of the Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, *Revenue from Contracts with Customers (Topic 606).*

Concentration of Credit Risk

The Company's financial instruments that are exposed to concentration of credit risk consist primarily of cash and cash equivalents. The Company maintains its cash in bank accounts, the balances of 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 Zimura on a purchase order basis. The Company also engages a single third-party manufacturer to provide fill/finish services for clinical supplies of Zimura. In addition, the Company currently relies upon a single third-party supplier to supply it with the proprietary polyethylene glycol, or PEG, reagent used to manufacture Zimura 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 Zimura. 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, historically, 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 academic research collaborators, 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 for employees dedicated to research and development activities, 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. The Company expects that research and development expenses in the future will also include the costs of the Company's collaborative gene therapy research programs, including the costs of its research collaboration with the University of Massachusetts Medical School ("UMMS"), entered into in February 2018.

All research and development expenses are charged to operations as incurred in accordance with 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. 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.

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 options 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 and RSUs 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.

The weighted-average assumptions used to estimate grant date fair value of stock options using the Black-Scholes option pricing model were as follows for the three months ended March 31, 2018 and 2017:

	Three Months Ended March 31,	
	2018	2017
Expected common stock price volatility	81%	81%
Risk-free interest rate	2.39%-2.65%	2.10%-2.38%
Expected term of options (years)	5.9	6.2
Expected dividend yield	—	—

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 options 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 March 31,	
	2018	2017
Research and development	$ 1,440	$ 4,150
General and administrative	1,642	1,914
Total	$ 3,082	$ 6,064

Recently Adopted Accounting Pronouncements

In May 2014, the FASB issued Accounting Standards Codification 606, *Revenue from Contracts with Customers* (“ASC 606”). ASC 606 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 requires 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. On January 1, 2018, the Company adopted this guidance using the modified retrospective approach. Due to the termination of the Novartis Agreement and the Company's current lack of other revenue sources, the Company's financial statements were not impacted by adoption of this standard. The future impact of ASC 606 will be dependent on the nature of the Company's future revenue contracts and arrangements, if any.

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. During the three months ended March 31, 2018, the Company adopted this guidance. The adoption did not have a material impact on the Company's 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.

Recent Accounting Pronouncements

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.

3. Net Loss Per Common Share

Basic and diluted net loss per common share is determined by dividing net loss by the weighted average common shares outstanding during the period. For the periods where there is a net loss, shares underlying stock options and RSUs have been excluded from the calculation of diluted net loss per common share because the effect of including such shares 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 loss per common share for the periods indicated:

	Three Months Ended March 31,	
	2018	2017
Basic and diluted net income (loss) per common share calculation:		
Net loss	$ (13,073)	$ (43,122)
Weighted average common shares outstanding - basic and dilutive	36,153	35,804
Net loss per common share - basic and diluted	$ (0.36)	$ (1.20)

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

	Three Months Ended March 31,	
	2018	2017
Stock options outstanding	5,021	3,999
Restricted stock units	228	678
Total	5,249	4,677

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. As of March 31, 2018 and December 31, 2017, the Company had cash and cash equivalents of approximately 154.9 million and 167.0 million, respectively. Cash and cash equivalents included cash of $5.0 million at March 31, 2018 and $9.5 million at December 31, 2017. Cash and cash equivalents at March 31, 2018 and December 31, 2017 also included $149.9 million and $157.4 million, respectively, of investments in money market funds.

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 no available for sale securities at March 31, 2018 or at December 31, 2017, respectively. During the year ended December 31, 2017, the Company's investments matured and were reinvested in money market funds.

The Company believes that its existing cash and cash equivalents as of March 31, 2018 will be sufficient to fund its operations and capital expenditure requirements as currently planned for at least the next 12 months.

5. Prior Licensing and Commercialization Agreement with Novartis Pharma AG

Prior to 2018, the Company's revenue resulted from payments received under the Novartis Agreement, as modified by the July 2017 letter agreement entered into by the Company and Novartis. These two agreements are described below. The Company used the relative selling price method to allocate arrangement consideration to the Company’s performance obligations under the Novartis Agreement. The Company completed the deliverables under the Novartis Agreement and the Letter Agreement during the third quarter of 2017.

Below is a summary of the components of the Company's collaboration revenue for the three months ended March 31, 2018 and 2017:

	Three months ended March 31,	
	2018	2017
License revenue	$ —	$ —
Research and development activity revenue	—	1,658
API transfer revenue	—	—
Joint operating committee revenue	—	4
Total collaboration revenue	$ —	$ 1,662

In May 2014, the Company entered into 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 agent 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 agent 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.

In July 2017, the Company and Novartis entered into the Letter Agreement to streamline the process and timeline for evaluating data from the OPH1004 trial once it became available. On October 23, 2017, following the failure of the Phase 3 Fovista program and pursuant to the terms of the Letter Agreement, Novartis elected to terminate the Novartis Agreement with immediate effect.

In May 2014, Novartis paid the Company a $200.0 million upfront payment. 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 in enrollment-based milestones under the Novartis Agreement. The Company used the relative selling price method to allocate these payments to contract deliverables based on its performance obligations 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”)), the relevant codification applicable at the time, to determine if they represented a multiple element revenue arrangement. The Novartis Agreement included 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”). The Company’s obligation to provide access to clinical and regulatory information as part of the License Deliverable included the obligation to provide access to all clinical data, regulatory filings, safety data and manufacturing data to Novartis which was necessary for the commercialization of Fovista in the Novartis Territory. The R&D Activity Deliverable included the right and responsibility for the Company to conduct the Phase 3 Fovista clinical program and other Phase 2 studies of Fovista which were necessary or desirable for regulatory approval or commercialization of Fovista. The Manufacturing Deliverable included the obligation for the Company to supply API to Novartis for clinical purposes, for which Novartis agreed to pay the Company’s manufacturing costs. The Joint Operating Committee Deliverable included 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 was accounted for separately.

The Novartis Agreement included a termination right for the Company in the event that specified governmental actions prevented the parties from materially progressing the development or commercialization of licensed products. If the Company elected to exercise this termination option, it would have been required to pay a substantial termination fee equivalent to the entire upfront payment amount. The Company concluded that this termination provision constituted a contingent event that was unknown at the inception of the agreement. As such, the Company recorded the $200.0 million upfront payment in deferred revenue, long-term until such time that the contingency related to this termination provision was resolved. In July 2017, the contingency was resolved when the Company permanently waived this termination right as part of the Letter Agreement.

The Letter Agreement also provided Novartis with a shorter notice period in the event Novartis determined to terminate the Novartis Agreement in certain circumstances. In addition, the Letter Agreement provided 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 continues until the fifth anniversary of the Letter Agreement.

The Company evaluated the Letter Agreement under ASC 605-25, the relevant codification applicable at the time, and determined that the Letter Agreement does not create any new deliverables. The Company is treating the Fovista license granted at the inception of the Novartis Agreement and the Lucentis study data license granted under the Letter Agreement as one collective technology license (the "Licenses") delivered at the inception of the Novartis Agreement. In addition, as the waiver of its right to terminate the Novartis Agreement as a result of specified governmental actions resolved the Company’s contingency with respect to such termination right and the associated termination fee, the Company allocated the entire previously deferred amount, $200.0 million, to the deliverables that were determined based on the relative selling price at contract inception. Upon entry into the Letter Agreement in July 2017, the Company immediately recognized as revenue $189.8 million of the upfront payment allocated to contract deliverables completed during prior periods. Upon termination of the OPH1004 trial in August 2017, the Company recognized the remaining $16.9 million of collaboration revenue, attributable to the R&D Deliverable, previously deferred under the Novartis Agreement. In total, during the third quarter of 2017, the

Company recognized $206.7 million in previously deferred collaboration revenue in connection with the Novartis Agreement. The recognition of this revenue during the third quarter of 2017 did not impact the Company's cash balance.

The Company's collaboration revenue for the three months ended March 31, 2017 related to the research and development activities performed by the Company during the period under the Novartis Agreement. All activities under the Novartis agreement were completed during the third quarter of 2017 prior to the adoption of ASC 606.

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 March 31, 2018, 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. If the Company were to make royalty payments under the Novo Agreement, it would reduce the liability balance at such time. 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

On December 22, 2017, the U.S. Tax Cuts and Jobs Act (“TCJA”) was enacted reducing the corporate tax rate from 35% to 21% effective for tax years beginning on or after January 1, 2018. ASC 740, *Income Taxes*, requires the effects of changes in tax laws to be recognized in the period in which the legislation is enacted. However, due to the complexity and significance of TCJA's provisions, the SEC staff issued *Staff Accounting Bulletin* 118, which allows companies to record the tax effects of the TCJA on a provisional basis based on a reasonable estimate, and then, if necessary, subsequently adjust such amounts during a limited measurement period as more information becomes available. The measurement period ends when a company has obtained, prepared, and analyzed the information necessary to finalize its accounting, but cannot extend beyond one year from enactment of the TCJA.

Under the TCJA, the Corporate Alternative Minimum Tax ("AMT") was repealed. The Company's previously recorded Alternative Minimum Tax (“AMT”) credits of approximately $3.5 million are now refundable over a four year period beginning in 2018, and the previously recorded valuation allowance for these AMT credits was reversed during the fourth quarter of 2017. During the three months ended March 31, 2018, the Company reduced its estimate of refundable AMT credits to approximately

$3.3 million to reflect the impact of sequestration as required by the Balanced Budget and Emergency Deficit Control Act of 1985, as amended. As a result of the TCJA's reduction in the corporate tax rate from 35% to 21% the value of the Company's deferred tax assets, and related valuation allowance, were reduced by a provisional amount of approximately $54.6 million during the year ended December 31, 2017. The Company does not have any offshore earnings from which to record the mandatory transition tax enacted under the TCJA. Given the significant complexity of the TCJA, anticipated guidance from the US Treasury and the Internal Revenue Service about implementing the TCJA, and the potential for additional guidance from the SEC or the FASB related to the TCJA, the deferred taxes provisional amounts may be adjusted during the measurement period. These provisional amounts were based on the Company's present interpretations of the TCJA and current available information, including assumptions and expectations about future events, such as its projected financial performance, and are subject to further refinement as additional information becomes available (including potential new interpretative guidance) and further analyses are completed.

For the three months ended March 31, 2018, the Company recorded a $0.2 million provision for income taxes to reflect a reduction in the amount of deferred tax assets expected to be realized in the future. For the three months ended March 31, 2017, the Company recorded a de minimis provision for income taxes.

The Company will continue to evaluate its ability to realize its deferred tax assets on a quarterly 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*, 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 may consist of investments in investment-grade corporate debt securities. The Company does not currently hold any assets that are measured using Level 2 inputs.

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

The following table presents, for each of the fair value hierarchy levels required under ASC 820, the Company's assets and liabilities that are measured at fair value on a recurring basis as of March 31, 2018:

	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*	$ 149,928	$ —	$ —

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, 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*	$ 162,457	$ —	$ —

* Investments in money market funds 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 measurement hierarchy occurred during the three months ended March 31, 2018.

The Company's available for sale securities are reported at fair value on the Company's Balance Sheets. The Company held no available for sale securities for the three month period ended March 31, 2018. Unrealized gains (losses) are reported within accumulated other comprehensive income (loss) in the statements of comprehensive 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 loss associated with the unrealized loss on available for sale securities for the three months ended March 31, 2018 and March 31, 2017 were as follows:

	Three Months Ended March 31,	
	2018	2017
Beginning balance	$ —	$ (212)
Current period changes in fair value before reclassifications, net of tax	—	(10)
Amounts reclassified from accumulated other comprehensive income (loss), net of tax	—	—
Total other comprehensive income (loss)	—	(10)
Ending balance	$ —	$ (222)

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 6,898,000 additional shares to the 2013 Plan, including for 2018, an increase of approximately 1,444,000 shares, or 4% of the total number of shares of the Company's common stock outstanding as of January 1, 2018. As of March 31, 2018, the Company had approximately 2,212,000 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 March 31, 2018 is as follows (in thousands except weighted average exercise price):

	Three Months Ended March 31, 2018	
	Number of Shares Underlying Options	Weighted Average Exercise Price
Outstanding, December 31, 2017	5,284	$ 19.58
Granted	64	$ 2.79
Forfeited	(327)	$ 33.76
Outstanding, March 31, 2018	5,021	$ 18.44
Options exercisable at March 31, 2018		2,225
Weighted average grant date fair value (per share) of options granted during the period		$ 1.95

As of March 31, 2018, there were outstanding, net of estimated forfeitures, options to purchase approximately 4,713,000 shares, which options had vested or are expected to vest. The weighted-average exercise price of these options was $19.10 per share; the weighted-average remaining contractual life of these options was 7.9 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 March 31, 2018 is as follows (in thousands except exercise prices and weighted average exercise price):

	March 31, 2018					
		Options Outstanding			Options Exercisable	
Range of Exercise Prices	Total Shares Underlying Options Outstanding	Weighted Average Remaining Life (Years)	Weighted Average Exercise Price		Number of Shares for which Options are Exercisable	Weighted Average Exercise Price
$0.12-$10.03	3,028	9.1	$	3.66	495	$ 5.43
$10.04-$20.00	147	5.3	$	13.63	147	$ 13.63
$20.01-$30.00	127	5.6	$	25.13	127	$ 25.13
$30.01-$40.00	827	5.3	$	32.81	827	$ 32.81
$40.01-$55.00	598	7.3	$	46.35	424	$ 46.35
$55.01-$73.22	294	7.8	$	72.50	205	$ 72.18
	5,021	8.0	$	18.44	2,225	$ 31.22
Aggregate Intrinsic Value	$ 75				$ 69	

Cash proceeds from, and the aggregate intrinsic value of, stock options exercised during the three months ended March 31, 2018 and 2017, respectively, were as follows:

	Three Months Ended March 31,	
	2018	2017
Cash proceeds from options exercised	$ —	$ 31
Aggregate intrinsic value of options exercised	$ —	$ 37

In connection with stock option awards granted to employees, the Company recognized approximately $2.1 million and $4.4 million in share-based compensation expense during the three months ended March 31, 2018 and 2017, respectively, net of expected forfeitures. As of March 31, 2018, there were approximately $12.6 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 2.4 years.

In connection with stock option awards granted to consultants, the Company recognized approximately $0.1 million and $0.1 million in share-based compensation expense during the three months ended March 31, 2018 and 2017, respectively, net of expected forfeitures. As of March 31, 2018, there were approximately $0.1 million of unrecognized compensation costs, net of estimated forfeitures, related to stock option awards granted to consultants, which are expected to be recognized over a remaining weighted average period of 1.6 years.

The following table presents a summary of the Company's outstanding RSU awards granted as of March 31, 2018 (in thousands except weighted average grant-date fair value):

	Restricted Stock Units	Weighted Average Grant-Date Fair Value
Outstanding, December 31, 2017	327	$ 51.08
Vested	(41)	$ 47.77
Forfeited	(58)	$ 47.17
Outstanding, March 31, 2018	228	$ 52.46

As of March 31, 2018, there were approximately 134,000 RSUs outstanding, net of estimated forfeitures, that are expected to vest. The weighted-average fair value of these RSUs was $49.37 per share; and the aggregate intrinsic value of these RSUs was approximately $0.4 million.

In connection with RSUs granted to employees, the Company recognized approximately $0.9 million and $1.3 million in share-based compensation expense during the three months ended March 31, 2018 and 2017, respectively, net of expected forfeitures. As of March 31, 2018, there was approximately $4.6 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 1.8 years. The total fair value of the RSUs that vested during the three months ended March 31, 2018 was $1.7 million.

In connection with RSUs granted to consultants, the Company recognized a de minimis amount of share-based compensation expense and $0.1 million in share-based compensation expense during the three months ended March 31, 2018 and 2017, respectively, net of expected forfeitures. As of March 31, 2018, 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 1.5 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 March 31, 2018 and 2017, respectively, net of expected forfeitures. As of March 31, 2018, 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 0.5 years. There were 12,229 shares of common stock issued under the ESPP during the three months ended March 31, 2018. Cash proceeds from ESPP purchases were $27 thousand during the three months ended March 31, 2018. There were 4,746 shares of common stock issued under the ESPP plan during the three months ended March 31, 2017. As of March 31, 2018, there were 971,413 shares available for future purchases under the ESPP.

10. Property and Equipment

Property and equipment as of March 31, 2018 and December 31, 2017 were as follows:

	Useful Life (Years)	March 31, 2018	December 31, 2017
Manufacturing and clinical equipment	7 - 10	$ 412	$ 412
Computer, software and other office equipment	5	933	933
		1,345	1,345
Accumulated depreciation		(877)	(827)
Property and equipment, net		$ 468	$ 518

For the three months ended March 31, 2018 and 2017, depreciation expense was $50 thousand and $808 thousand, respectively.

11. Commitments and Contingencies

The Company is party to an agreement with Archemix Corp., or Archemix, under which the Company in-licensed rights in certain patents, patent applications and other intellectual property related to Zimura and pursuant to which the Company may be required to pay sublicense fees and make milestone payments. Under the license agreement, for each anti-C5 aptamer product that the Company may develop under the agreement, including Zimura, the Company is obligated to make payments to Archemix of up to an aggregate of $57.5 million if the Company achieves specified development, clinical and regulatory milestones, with $30.5 million of such payments relating to a first indication, $24.5 million of such payments relating to second and third indications and $2.5 million of such payments relating to sustained delivery applications. Under the license agreement, the Company is also obligated to make additional payments to Archemix of up to an aggregate of $22.5 million if the Company achieves specified commercial milestones based on net product sales of all anti-C5 products licensed under the agreement. The Company is also obligated to pay Archemix a double-digit percentage of specified non-royalty payments the Company may receive from any sublicensee of its rights under the C5 agreement. The Company is not obligated to pay Archemix a running royalty based on net product sales in connection with the C5 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 agents 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. These cases were consolidated on March 13, 2018. The deadline for lead plaintiff to file an amended complaint is June 4, 2018. The Company intends to file a motion to dismiss the consolidated case on or before July 19, 2018.

On February 7, 2018, a shareholder derivative action was filed against the members of the Company's Board of Directors in the New York Supreme Court Commercial Division, captioned Cano v. Guyer, et al., No. 650601/2018. The complaint alleges that defendants breached their fiduciary duties to the Company by adopting a compensation plan that overcompensates the non-employee members of the Board relative to boards of companies of comparable market capitalization and size. 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 improve its corporate governance and internal procedures to comply with applicable laws, attorneys' fees, and other costs. The Company intends to file a motion to dismiss this case on or before May 14, 2018.

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 these matters 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. The reduction in personnel was substantially completed during 2017 with a limited number of departing employees scheduled to receive severance payments during 2018.

In January 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. On November 1, 2017, the Company and One Penn Plaza LLC executed a further amendment to the Lease extending the term of the Lease to the end of 2018. Payments under the further lease amendment will not constitute restructuring charges.

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 became effective February 2018, through which time the Company was 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.

In connection with the Company's restructuring activities, the Company recognized severance, stock compensation, other employee costs and lease termination costs, all of which have been reported in the Company's Statements of Operations, as follows:

	Three Months Ended March 31,	
	2018	2017
Research and development	$ —	$ 4,835
General and administrative	—	3,941
Total	$ —	$ 8,776

As of March 31, 2018, the Company's accrual balance for severance and benefit costs was $0.6 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 March 31, 2018 are expected to be paid through to December of 2018.

The following is a reconciliation of the severance-related accrual activity for the three months ended March 31, 2018:

	Accrued Severance and Other Employee Costs
Beginning Balance	$ 2,529
Accrued restructuring expenses	—
Payments	(1,898)
Ending Balance	$ 631

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 together with our financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and the audited financial statements and related notes and management's discussion and analysis of financial condition and results of operations for the year ended December 31, 2017 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 5, 2018. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties and should be read together with the "Risk Factors" section of this Quarterly Report on Form 10-Q for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a science-driven biopharmaceutical company specializing in the development of novel therapies to treat ophthalmic diseases, with a focus on age-related and orphan retinal diseases. Our multi-track strategy is to leverage our clinical experience and retina expertise to develop therapies for large market, age-related retinal diseases, where unmet medical needs remain for these patients, and for orphan eye diseases with a focus on underserved patients, and to utilize a disciplined business development approach to obtain additional products, product candidates and technologies in these disease areas. We believe that there are advantages to pursuing drug development for orphan indications, including the potential for regulatory exclusivity, the potential for clinical trials with smaller sample sizes and the potential for accelerated development timelines. Our team has significant ophthalmic drug development experience and deep relationships with global ophthalmology thought leaders. We have an extensive network of ophthalmic clinical trial sites, having worked with over 250 sites worldwide. We believe that the combination of these factors, together with our experience in designing and executing IND-enabling studies and clinical trials for eye diseases, and specifically back of the eye diseases, provide us a competitive advantage.

We are developing Zimura® (avacincaptad pegol), our complement C5 inhibitor, for dry and wet forms 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 loss of central vision, and autosomal recessive Stargardt disease, or STGD1, which is an orphan inherited retinal disease that also may result in loss of central and peripheral vision. In connection with our Stargardt clinical trial, which we recently initiated, we have expanded our network of thought leaders and clinical trial sites for orphan ophthalmic indications to include leading research university hospitals around the world, where patients with orphan retinal diseases are often referred.

We are actively engaged in extensive business development efforts to identify, evaluate and potentially obtain rights to and develop additional therapeutic and gene therapy product candidates that would complement our strategic goals and leverage our competitive advantages. We believe that our strategy will provide multiple potential opportunities to bring ophthalmic therapies to market.

Zimura

Based on our Zimura development experience to date, as well as scientific literature in the field, we believe there is a strong rationale to pursue the development of our C5 complement inhibitor, Zimura, in multiple ophthalmic diseases. Zimura is a chemically-synthesized, pegylated RNA aptamer. Aptamers are short molecules made up of a single stranded nucleic acid sequence or an amino acid sequence that bind molecular targets with high selectivity and specificity. We have multiple clinical development programs for Zimura ongoing or planned to initiate by the end of 2018. Our ongoing and planned clinical trials for Zimura, all of which are designed to obtain data to guide potential future development efforts, include the following:

- **OPH2003 (geographic atrophy (GA) secondary to dry AMD)**: an ongoing, randomized, double–masked, sham controlled, multi-center Phase 2b clinical trial evaluating the safety and efficacy of Zimura monotherapy in patients with geographic atrophy, or GA, secondary to dry AMD. GA, the end stage of dry AMD, is a disease characterized by retinal cell death and degeneration of retinal tissue.

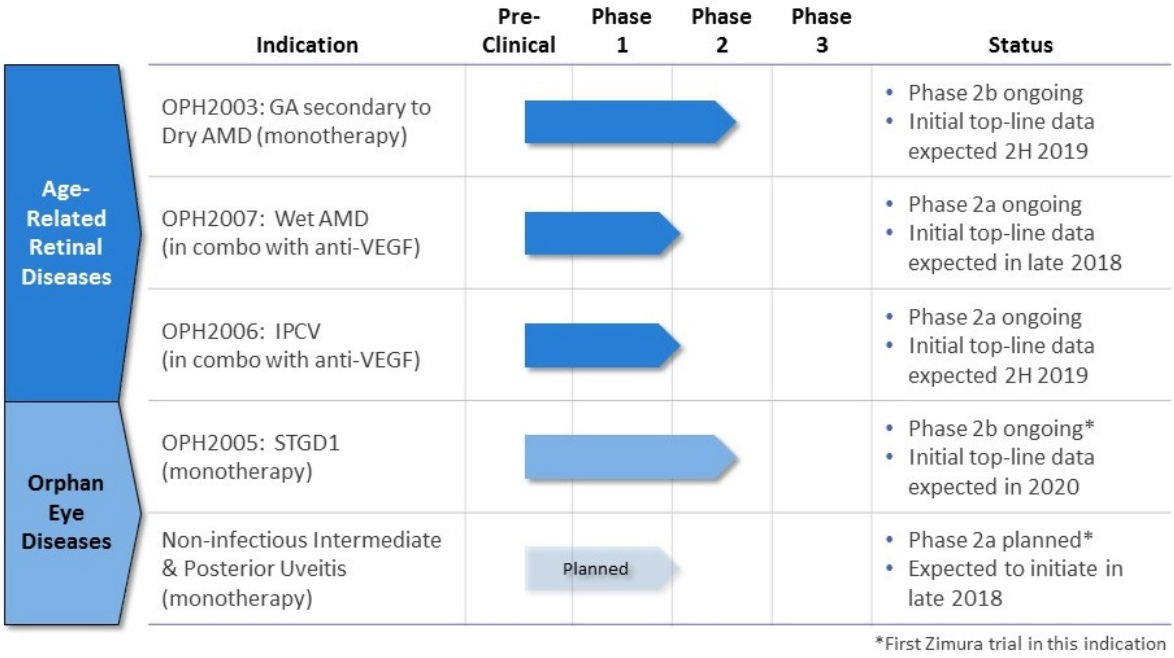
- **OPH2007 (wet AMD)**: an ongoing, randomized, dose-ranging, open-label, multi-center Phase 2a clinical trial of Zimura in combination with the anti-vascular endothelial growth factor, or anti-VEGF, agent Lucentis® (ranibizumab) for the treatment of wet AMD in patients who have not previously been treated with anti-VEGF agents, referred to as treatment-naïve patients. Wet AMD is characterized by the presence and growth of abnormal new blood vessels under and through the retina. In May 2018, we announced completion of enrollment for this trial with a total of 64 patients enrolled.

- **OPH2006 (IPCV)**: an ongoing, randomized, dose-ranging, open-label Phase 2a clinical trial of Zimura in combination with the anti-VEGF agent Eylea® (aflibercept) for the treatment of idiopathic polypoidal choroidal vasculopathy, or IPCV, in patients who have not responded to Eylea monotherapy. IPCV is an age-related retinal disease involving the choroidal vasculature characterized by the presence of polypoidal lesions, which leads to vision loss. We are at a very early stage of site initiation and patient recruitment for this trial.

- **OPH2005 (autosomal recessive Stargardt disease (STGD1))**: an ongoing, randomized, double-masked, sham controlled, multi-center Phase 2b clinical trial evaluating the safety and efficacy of Zimura monotherapy for the treatment of autosomal recessive Stargardt disease, referred to as STGD1. We are at an early stage of patient recruitment for this trial.

- **Non-infectious intermediate and posterior uveitis**: a planned open-label Phase 2a clinical trial of Zimura monotherapy for the treatment of non-infectious intermediate and posterior uveitis, a rare inflammatory disease of the back of the eye.

The following table summarizes the current status of these ongoing and planned Zimura development programs:



On-going Business Development and Pipeline Expansion Activities

Since early 2017, we have been engaged in extensive business development efforts. Without limiting any option, the principal focus of this plan, based on our deep expertise and experience in ophthalmic drug development, has been to actively explore obtaining rights to additional products, product candidates and technologies to treat ophthalmic diseases, particularly those in the back of the eye. We evaluated a large number of assets and platforms during 2017 and continue to actively review assets, platforms and other compelling ophthalmology opportunities that would complement our strategic goals. We 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. Our selection criteria are based on several factors. In general, we are looking for:

- compelling science;

- an identified unmet medical need based on the current standard of care;

- a meaningful commercial opportunity based on existing treatment options and treatment options known to be in development; and
- areas where we believe we can apply our competitive advantages.

Based on our work to date, among the novel technologies we have evaluated, we believe that gene therapy solutions may be particularly well-suited for our strategy as potential treatments for both orphan and age-related eye diseases. We remain committed to being opportunistic and will consider other compelling opportunities that may emerge.

Gene Therapy Research Programs

In February 2018, we announced that an element of our strategy will include initiating collaborative gene therapy programs focused on discovering and developing novel gene therapy technologies to treat retinal diseases. We intend to investigate promising gene therapy product candidates through collaborations with leading companies and academic and research institutions in the United States and internationally.

For our first gene therapy research collaboration, we have entered into a series of sponsored research agreements with the University of Massachusetts Medical School, or UMMS, and its Horae Gene Therapy Center to utilize novel gene delivery methods and UMMS's "minigene" therapy approach to target retinal diseases. AAV vectors are generally limited as a delivery vehicle by the size of their genetic cargo, which is restricted to approximately 4,700 base pairs of genetic code. The use of "minigenes" as a novel therapeutic strategy seeks to deliver a shortened but still functional form of a larger gene packaged into a standard-size AAV delivery vector. The "minigene" strategy may offer an innovative solution for diseases that would otherwise be difficult to address through conventional AAV gene replacement therapy where the size of the gene of interest exceeds the transgene packaging capacity of conventional AAV vectors. Furthermore, one of the differentiating advantages of the "minigene" approach is that it could potentially provide a treatment that is independent of a patient's specific mutation. The scope of the UMMS collaboration addresses Leber Congenital Amaurosis type 10, or LCA10, which is the most common type of LCA and is caused by mutations in the CEP290 gene, and STGD1, which is caused by mutations in the ABCA4 gene. LCA10 and STGD1 are both orphan inherited degenerative retinal diseases that lead to vision loss without any FDA or EMA approved treatment. As a condition of each sponsored research agreement, UMMS has granted us an option to obtain an exclusive license to any patents or patent applications that result from the sponsored research. Our aggregate financial commitment for the sponsored research agreements is in the low, single-digit millions of dollars.

Fovista Wind-down

In December 2016 and August 2017, we received initial top-line data from our three pivotal clinical trials, referred to as OPH1002, OPH1003 and OPH1004, evaluating the anti-platelet derived growth factor, or anti-PDGF, aptamer Fovista® (pegpleranib) administered in combination with anti-VEGF agents for the treatment of wet AMD, indicating that these trials failed to achieve their pre-specified primary endpoints. We have terminated these trials, as well as several other smaller Fovista trials in wet AMD, which we have referred to as the Fovista Expansion Studies. The National Eye Institute and an academic pre-clinical program are evaluating various uses of Fovista for the treatment of retinal capillary hemangiomas associated with the orphan disease Von-Hippel-Lindau Syndrome, and for the treatment of retinoblastoma, a rare cancer of the eye in children, respectively. We have completed our commitments to these two programs, which primarily involved providing Fovista drug product and drug substance that we had on hand for use in the studies.

Therefore, we do not currently expect any further development activity for Fovista going forward, as we have no intentions to resume development of Fovista in wet AMD and our supply commitments for the two external studies are complete.

Prior Novartis Agreement

In May 2014, we entered into a licensing and commercialization agreement with Novartis Pharma AG, or Novartis, 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 agent 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 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 agent to which Novartis has

rights, as well as a pre-filled syringe presentation of such products and to use commercially reasonable efforts, subject to obtaining marketing approval, to commercialize licensed products in the Novartis Territory in accordance with agreed development and marketing plans. Novartis paid us a $200.0 million upfront fee upon execution of the Novartis Agreement, as well as $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 $330.0 million. In July 2017, we and Novartis entered into a letter agreement to streamline the process and timeline for evaluating data from the final Fovista Phase 3 clinical trial once it became available. On October 23, 2017, following the failure of the Phase 3 Fovista program and pursuant to the terms of the July 2017 letter agreement, Novartis elected to terminate the Novartis Agreement with immediate effect.

Financial matters

As of March 31, 2018, we had cash and cash equivalents of $154.9 million. We estimate our year end 2018 cash and cash equivalents to range between $112.0 million and $117.0 million based on our current 2018 business plan and planned capital expenditures. This estimate includes continuation of our development programs for Zimura and the continuation of our collaborative gene therapy research programs as currently planned.

As a result of our ongoing reassessment of our development programs and potential business development opportunities and pipeline expansion activities, we may modify, expand or terminate some or all of our research or 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, will likely 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 2018, our revenue resulted from payments received under the Novartis Agreement as modified by the July 2017 letter agreement we entered into with Novartis in relation to the Novartis Agreement, both of which are described below under "—Liquidity and Capital Resources—Prior Licensing and Commercialization Agreement with Novartis Pharma AG." We used the relative selling price method to allocate arrangement consideration to our performance obligations under the Novartis Agreement. We completed the deliverables under the Novartis Agreement during the third quarter of 2017.

On October 23, 2017, following the failure of the Fovista Phase 3 program and pursuant to the terms of the July 2017 letter agreement, Novartis elected to terminate the Novartis Agreement with immediate effect. As we have no products approved for sale, we will not receive any revenue from any product candidates that we develop until we obtain regulatory approval and commercialize such products, or until we potentially enter into agreements with third parties for the development and commercialization of product candidates. If our development efforts for any of our product candidates result in regulatory approval or we enter into collaboration agreements with third parties, we may generate revenue from product sales or from such third parties.

Research and Development Expenses

Research and development expenses primarily consist of costs associated with the development and clinical testing and manufacturing of Zimura and, historically, 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 academic research collaborators, 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 for employees dedicated to research and development activities, 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. We expect that research and development expenses in the future will also include the costs of our collaborative gene therapy research programs, including the costs of our research collaboration with UMMS, entered into in February 2018.

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 months ended March 31, 2018 and 2017:

	Three months ended March 31,	
	2018	2017
	(in thousands)	
Fovista	$ 29	$ 10,440
Zimura	3,975	5,632
Personnel-related	1,881	10,952
Share-based compensation	1,440	4,150
Other	361	805
	$ 7,686	$ 31,979

We expect to continue to incur significant research and development expenses as we pursue the development of Zimura as currently planned. We also expect very limited research and development expenses related to Fovista in the future, as we have terminated our Fovista development programs and have no plans for the future development of Fovista. As we pursue our ongoing and planned Zimura development programs and our collaborative gene therapy research programs, or as we commence any new development efforts in relation to additional product candidates we may in-license or acquire as we pursue our business plan, we expect that our overall research and development expenses will begin to increase from the current level of expenditure.

Our expenses may exceed our expectations if we experience any unforeseen issue in our ongoing clinical trials, such as delays in enrollment or issues with the availability of drug supply or, if we further expand the scope of our clinical trials or collaborative research programs. Our costs may also exceed our expectations for other reasons, for example, if we experience issues with process development, establishing or scaling-up of manufacturing activities or activities to enable and qualify second source suppliers or if we decide to increase preclinical and clinical research and development activities, including by entering into new collaborative research programs, in-licensing or acquiring, and pursuing the development of, additional product candidates, building internal research capabilities or pursuing internal research efforts.

The future development of our product candidates is highly uncertain. 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 clinical development, to complete process development and manufacturing scale-up and validation activities or to potentially seek marketing approval with respect to our product candidates.

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 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 32 of this Quarterly Report on Form 10-Q for more information regarding our current and future financial resources and our expectations regarding our research and development expenses and funding requirements.

General and Administrative Expenses

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

We anticipate that our general and administrative expenses will decrease in future periods as compared to 2017 levels as a result of a reduction in personnel to focus on our revised business plan, which we expect will involve a total expected workforce of approximately 40 employees. We substantially completed the reduction in personnel during 2017 as part of implementing our revised business plan. The expected decreases in our general and administrative expenses as compared to 2017 levels may be partially offset by expenses related to business development activities.

Interest Income

We currently have invested our cash and cash equivalents in money market funds, 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 academic research collaborators, CROs, CMOs and other vendors in connection with research and development and manufacturing activities.

We base our expenses related to academic research collaborators, 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

In May 2014, we received an upfront payment of $200.0 million in connection with our entry into the Novartis Agreement, which was not recorded as revenue due to the existence of a contingency with respect to our right to terminate the agreement in certain circumstances and the associated termination fee equivalent to the entire $200.0 million upfront payment, which we would have been required to pay if we elected to exercise this termination option. 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 in milestones, under the Novartis Agreement. We used the relative selling price method to allocate these payments to contract deliverables based on our performance obligations under the Novartis Agreement.

The July 2017 letter agreement with Novartis resolved the contingency with respect to our termination right, allowing us to immediately recognize as revenue the portion of the upfront payment allocated using the relative selling price method to deliverables completed during prior periods. During the third quarter of 2017, we completed the remaining deliverables under the Novartis Agreement and the July 2017 letter agreement and recognized as revenue the balance of all of the payments previously received from Novartis related to licensing, research and development, manufacturing and joint operating committee activities that had been previously deferred using the relative selling price method. In total, during the third quarter of 2017, we recognized $206.7 million in previously deferred collaboration revenue in connection with the Novartis Agreement. The recognition of this revenue during the period did not impact our cash balance. On October 23, 2017, following the failure of the Fovista Phase 3 program and pursuant to the terms of the July 2017 letter agreement, Novartis elected to terminate the Novartis Agreement with immediate effect.

Below is a summary of the components of our collaboration revenue for the three months ended March 31, 2018 and 2017:

	Three months ended March 31,	
	2018	2017
	(in thousands)	
License revenue	$ —	$ —
Research and development activity revenue	—	1,658
API transfer revenue	—	—
Joint operating committee revenue	—	4
Total collaboration revenue	$ —	$ 1,662

Royalty Purchase Liability

The proceeds from the financing we received under our Fovista royalty financing agreement with Novo A/S, or the Novo Agreement, have been recorded as a liability on our Balance Sheet in accordance with ASC 730, *Research and Development*. Although there is no explicit repayment obligation contained in the Novo Agreement, because there was a significant related party relationship between us and Novo A/S at the time the Novo Agreement was entered into, 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. In the event that we make royalty payments to Novo A/S, 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 employee stock purchase plan. 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 months ended March 31,

2018 and 2017:

	Three months ended March 31,	
	2018	2017
Expected common stock price volatility	81%	81%
Risk-free interest rate	2.39%-2.65%	2.10%-2.38%
Expected term of options (years)	5.9	6.2
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 also 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 $3.1 million and $6.1 million for the three months ended March 31, 2018 and 2017, respectively. As of March 31, 2018, we had $17.4 million of total unrecognized share-based compensation expense, which we expect to recognize over a weighted-average remaining vesting period of approximately 2.2 years. We expect our share-based compensation expense for our equity awards to employees, non-employee directors and consultants to increase as a result of recognizing our existing unrecognized share-based compensation for awards that will vest and as we issue additional equity awards to attract and retain our employees.

For the three months ended March 31, 2018 and 2017, we allocated share-based compensation as follows:

	Three months ended March 31,	
	2018	2017
	(in thousands)	
Research and development	$ 1,440	$ 4,150
General and administrative	1,642	1,914
Total	$ 3,082	$ 6,064

Income Taxes

On December 22, 2017, the U.S. Tax Cuts and Jobs Act, or the TCJA, was enacted reducing the corporate tax rate from 35% to 21% effective for tax years beginning on or after January 1, 2018. As a result of the passage of the TCJA, the value of our deferred tax assets and related valuation allowance was reduced by a provisional amount of approximately $54.6 million. Additionally, under the TCJA, the Corporate Alternative Minimum Tax, or AMT, was repealed. Accordingly, our previously recorded AMT credits of approximately $3.5 million are now refundable over a four-year period beginning in 2018 and the previously recorded valuation allowance for these AMT credits was reversed as a result of the TCJA during the fourth quarter of 2017. During the three months ended March 31, 2018, we reduced our estimate of refundable AMT credits to $3.3 million to reflect the impact of sequestration as required by the Balanced Budget and Emergency Deficit Control Act of 1985, as amended.

The deferred tax assets associated with our losses incurred to date in 2018 have a full valuation allowance recorded against them due to our history of losses and the lack of other positive evidence to support future taxable income against which these losses could be applied. See Note 7 to our financial statements in Part I-Item 1 of this Quarterly Report on Form 10-Q for further information regarding our expectations with respect to our income tax provision.

Results of Operations

Comparison of Three Month Periods Ended March 31, 2018 and 2017

	Three months ended March 31,		Increase (Decrease)
	2018	2017	
	(in thousands)		
Statements of Operations Data:			
Collaboration revenue	$ —	$ 1,662	$ (1,662)
Operating expenses:			
Research and development	7,686	31,979	(24,293)
General and administrative	5,645	13,159	(7,514)
Total operating expenses	13,331	45,138	(31,807)
Loss from operations	(13,331)	(43,476)	(30,145)
Interest income	473	378	95
Other expense	(16)	(21)	(5)
Loss before income tax provision	(12,874)	(43,119)	(30,245)
Income tax provision	199	3	196
Net loss	$ (13,073)	$ (43,122)	$ (30,049)

Collaboration Revenue

We recognized no collaboration revenue for the three months ended March 31, 2018, a decrease of $1.7 million compared to $1.7 million for the three months ended March 31, 2017. Collaboration revenue for the three months ended March 31, 2018 decreased as we completed all deliverables required under the Novartis Agreement during the year ended December 31, 2017.

Collaboration revenue for the three months ended March 31, 2017 was $1.7 million, which was allocated to research and development activities performed under the Novartis Agreement.

Research and Development Expenses

Our research and development expenses were $7.7 million for the three months ended March 31, 2018, a decrease of $24.3 million compared to $32.0 million for the three months ended March 31, 2017. The decrease in research and development expenses for the three months ended March 31, 2018 was primarily due to an $11.8 million decrease in personnel costs which included a $2.7 million decrease in stock compensation costs and a $4.8 million decrease in costs related to our previously announced reduction in force. Additionally, there was a $10.4 million decrease in costs associated with our Fovista program, including our Fovista Phase 3 clinical program and our Fovista Expansion Studies, a $1.7 million decrease associated with our Zimura program, and a $0.2 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 Fovista Phase 3 clinical trials and the Fovista Expansion Studies. The decreased costs for our Zimura program included lower costs related to the Zimura manufacturing activities offset by the increased costs related to clinical trial activities as a result of start-up costs and increased enrollment.

General and Administrative Expenses

Our general and administrative expenses were $5.6 million for the three months ended March 31, 2018, a decrease of $7.5 million, compared to $13.2 million for the three months ended March 31, 2017. The decrease in general and administrative expenses for the three months ended March 31, 2018 was primarily due to a decrease in costs to support our operations and infrastructure as a result of our reduction in force and the termination of facilities leases completed during 2017. General and administrative expenses for the three months ended March 31, 2017 included approximately $3.9 million in costs related to our previously announced reduction in force and the termination of facilities leases.

Interest Income

Interest income for the three months ended March 31, 2018 was $0.5 million compared to interest income of $0.4 million for the three months ended March 31, 2017.

Income Tax Provision

We recorded an income tax provision of $199 thousand and $3 thousand for the three months ended March 31, 2018 and 2017, respectively. The income tax provision recorded for the three months ended March 31, 2018 primarily related to a reduction in the amount of deferred tax assets that we expect will be realized in the future.

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.

Cash Flows

As of March 31, 2018, we had cash and cash equivalents totaling $154.9 million and no debt. We currently have invested our cash and cash equivalents in money market funds.

The following table shows a summary of our cash flows for the three months ended March 31, 2018 and 2017:

	Three months ended March 31,	
	2018	2017
	(in thousands)	
Net cash (used in) provided by:		
Operating Activities	$ (12,088)	$ (61,512)
Investing Activities	—	20,105
Financing Activities	27	31
Net change in cash and cash equivalents	$ (12,061)	$ (41,376)

Cash Flows from Operating Activities

Net cash used in operating activities for the three months ended March 31, 2018 was $12.1 million and relates primarily to net cash used to fund our Zimura research and development activities and our general and administrative operations.

Net cash used in operating activities for the three months ended March 31, 2017 was $61.5 million and related 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.

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

We had no net cash provided by investing activities for the three months ended March 31, 2018. Net cash provided by investing activities for the three months ended March 31, 2017 was $20.1 million and relates primarily to proceeds from the maturity of marketable securities totaling $32.1 million offset by purchases of marketable securities totaling $12.0 million.

Cash Flows from Financing Activities

Net cash provided by financing activities of $27 thousand for the three months ended March 31, 2018 and $31 thousand for the three months ended March 31, 2017 related to the proceeds from stock option plan exercises and purchases made under our employee stock purchase plan.

Funding Requirements

Our product candidate Zimura is in clinical development. We expect to continue to incur significant research and development expenses as we pursue the development of Zimura as currently planned. We could also 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 in additional indications, beyond those already in development, and as we evaluate and potentially in-license or acquire, and undertake development of, additional product candidates, including any promising product candidates that emerge from our collaborative gene therapy research programs. 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. We are party to an agreement with Archemix that imposes significant milestone payment obligations on us in connection with our achievement of specific clinical, regulatory and commercial milestones with respect to Zimura. 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 currently planned 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;
- pursue our collaborative gene therapy research programs;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, manufacturing, quality control, quality assurance and scientific personnel, especially if we are successful in acquiring or in-licensing rights to additional products, product candidates or technologies or progressing the clinical development of any of our product candidates or if we decide to establish internal gene therapy capabilities;
- seek marketing approval for any product candidates that successfully complete clinical trials;
- expand our outsourced manufacturing activities or establish commercial operations or sales, marketing and distribution capabilities, if we receive, or expect to receive, marketing approval for any of our product candidates; and
- expand our general and administrative functions to support future growth of the company.

As of March 31, 2018, we had cash and cash equivalents of $154.9 million. We estimate our year end 2018 cash and cash equivalents to range between $112.0 million and $117.0 million based on our current 2018 business plan and planned capital expenditures. This estimate includes continuation of our development programs for Zimura and the continuation of our collaborative gene therapy research programs as currently planned. We also had $133.5 million in total liabilities as of March 31, 2018, of which $125.0 million related to the Novo Agreement, which we are required to show as a liability on our balance sheets under generally accepted accounting principles but which does not correspond to any contractual repayment obligation.

We believe that our cash and cash equivalents 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 additional sponsored research or 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 scope of any additional collaborative research programs, 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 any unforeseen issue in our ongoing clinical trials, such as delays in enrollment or with the availability of drug supply or if we further expand the scope or size of our clinical trials or collaborative research programs. Our costs may also exceed our expectations for other reasons, for example, if we experience issues with manufacturing or 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 our product candidates is highly uncertain. 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 clinical development, to complete process development and manufacturing scale-up and validation activities or to potentially seek marketing approval with respect to our product candidates.

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

- the scope, costs and results of our ongoing Zimura clinical programs, as well as any additional clinical trials we undertake to obtain data sufficient to seek marketing approval for Zimura in any indication;
- the extent to which we in-license or acquire rights to, and undertake research or development of products, product candidates or technologies, including any product candidate or other technologies we may evaluate as part of our collaborative gene therapy research programs;
- 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 develop;
- the costs and timing of process development, manufacturing scale-up and validation activities and ongoing stability studies associated with Zimura or any other product candidates that we may develop;
- 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.

We do not have any committed external source of funds. 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 may not be available for at least several years, if at all. In addition, if approved, our product candidates 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. 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.

Prior 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 agent 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.

In July 2017, we and Novartis entered into a letter agreement to streamline the process and timeline for evaluating data from the OPH1004 trial once it became available. 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. On October 23, 2017, following the failure of the Phase 3 Fovista program and pursuant to the terms of the Letter Agreement, Novartis elected to terminate the Novartis Agreement with immediate effect.

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. 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, pursuant to a license, manufacturing and supply agreement that we agreed to terminate with Nektar in October 2017.

Royalty Financing Agreement with Novo A/S

In May 2013, we entered into the Novo Agreement, pursuant to which we had the ability to obtain 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 complete the Phase 3 development of Fovista, to file, prosecute and maintain certain patent rights and, in our reasonable judgment, to pursue claims of infringement of our intellectual property rights. The Novo Agreement also places certain restrictions on our business, including restrictions on our ability to grant security interests in our intellectual property to third parties, to sell, transfer or out-license intellectual property, or to grant others rights to receive royalties on sales of Fovista and certain other products. We reimbursed Novo A/S for specified legal and other expenses and are required to provide Novo A/S with certain continuing information rights. We have agreed to indemnify Novo A/S and its representatives with respect to certain matters, including with respect to any third-party infringement or product liability claims relating to our products. Our obligations under the Novo agreement are secured by a lien on certain of our intellectual property and other rights related to Fovista and other anti-PDGF products we may develop.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations as of March 31, 2018:

	Payments Due by Period				
	Total	Less than 1 year	1 - 3 years	3 - 5 years	More than 5 years
	(in thousands)				
Sponsored Research (1)	$ 2,450	$ 1,648	$ 802	$ —	$ —
Operating Leases (2)	581	537	44	—	—
Severance and Other Employee Benefits (3)	631	631	—	—	—
Total (4)	$ 3,662	$ 2,816	$ 846	$ —	$ —

(1) The table above includes our contracted obligations under our sponsored research agreements. We have engaged academic research collaborators to conduct research that has potential to create or enhance technologies to assist our development and commercialization of new products or processes.

(2) The table above includes our continuing rent obligations through February 2020. On November 1, 2017, we and One Penn Plaza LLC entered into an amendment to the lease for office space at One Penn Plaza in New York, New York extending the term of our lease, which was scheduled to expire in January 2018, through the end of December 2018.

(3) Severance and Other Employee Benefits represents our commitments under the Board of Directors' approved plan to implement a reduction in personnel that involved approximately 80% of our workforce based on the number of employees at the time the plan was approved. The reduction in personnel was substantially completed during 2017 with a limited number of departing employees scheduled to receive severance payments during 2018.

(4) This table does not include (a) any royalty payments, sublicense fees or 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) anticipated expenditures under supply agreements for periods for which we are not yet bound under binding purchase orders, (c) contracts that are entered into in the ordinary course of business that are not material in the aggregate in any period presented above and (d) our royalty purchase liability of $125.0 million as of March 31, 2018, due to the fact that royalty payment obligations are not expected given our lack of plans for the future development of Fovista or any other anti-PDGF product that would fall under our royalty obligation.

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

- Under a license agreement with Archemix, for each anti-C5 aptamer product that we may develop under the agreement, including Zimura, we are obligated to make additional payments to Archemix of up to an aggregate of $57.5 million if we achieve specified development, clinical and regulatory milestones, with $30.5 million of such payments relating to a first indication, $24.5 million of such payments relating to second and third indications and $2.5 million of such payments relating to sustained delivery applications. Under the C5 agreement, we are also obligated to make additional payments to Archemix of up to an aggregate of $22.5 million if we achieve specified commercial milestones based on net product sales of all anti-C5 products licensed under the agreement. 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 the C5 agreement. We are not obligated to pay Archemix a running royalty based on net product sales in connection with the C5 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 2018 annual meeting of stockholders, as filed with the SEC on April 17, 2018.

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 and cash equivalents of $154.9 million as of March 31, 2018, consisting of cash and investments in money market funds. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because a significant portion of our investments are in short-term securities. Our available for sale securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

We contract with CROs and CMOs globally. We may be subject to fluctuations in foreign currency rates in connection with certain of these agreements. Transactions denominated in currencies other than the U.S. dollar are recorded based on exchange rates at the time such transactions arise. As of March 31, 2018, substantially all of our total liabilities were denominated in the U.S. dollar.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of March 31, 2018. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Based on the evaluation of our disclosure controls and procedures as of March 31, 2018, 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 March 31, 2018 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II

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 agents 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 moved to consolidate the Micholle and Wasson actions. These cases were consolidated on March 13, 2018. The deadline for lead plaintiff to file an amended complaint is June 4, 2018. We intend to file a motion to dismiss on or before July 19, 2018.

On February 7, 2018, a shareholder derivative action was filed against the members of our Board of Directors in the New York Supreme Court Commercial Division, captioned Cano v. Guyer, et al., No. 650601/2018. The complaint alleges that defendants breached their fiduciary duties to our company by adopting a compensation plan that overcompensates the non-employee members of the Board relative to boards of companies of comparable market capitalization and size. 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 improve our corporate governance and internal procedures to comply with applicable laws, attorneys' fees, and other costs. We intend to file a motion to dismiss on or before May 14, 2018.

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 Business Plan, Financial Position and Need for Additional Capital

We are in the process of implementing a business plan that may continue to evolve as we await relevant clinical data and evaluate new opportunities. Our 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.

In early 2017, we began a process to review 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.

In July 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 also determined that we would continue our Zimura programs in age-related retinal diseases. In February 2018, we announced that an element of our strategy will include initiating gene therapy collaborations focused on discovering and developing novel gene therapy technologies to treat retinal diseases. We intend to investigate promising gene therapy product candidates through collaborations with leading companies and academic and research institutions in the United States and internationally. We continue to be actively engaged in extensive business development efforts to identify, evaluate and potentially obtain rights to and develop additional therapeutic and gene therapy product candidates that would complement our strategic goals and leverage our competitive advantages.

This 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 limited or 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, converting early stage gene therapy research efforts into clinical development opportunities, building internal or outsourced gene therapy capabilities and designing and executing a pre-clinical and/or clinical development 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 business plan. We are also continuing to consider other alternatives, including mergers or other transactions involving our company as a whole or other collaboration transactions. We cannot be sure when or if this 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 primarily dependent on the progress of our ongoing and planned clinical development programs for Zimura and the outcome of our ongoing business development efforts and pipeline expansion activities, together with the amount of our remaining available cash. The development of Zimura and the outcome of our ongoing business development efforts and pipeline expansion activities are 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 agents 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 autosomal recessive Stargardt disease, referred to as STGD1, or non-infectious intermediate and posterior uveitis. Our prior Zimura trials were not powered to demonstrate the efficacy of Zimura therapy with statistical significance. We determined the size of the OPH2003 trial in GA based on our best estimates of the size of trial required to demonstrate a potential clinical benefit for Zimura. This estimate incorporates our assumptions regarding the potential performance of Zimura in this indication based in part on available third-party clinical data and our statistical analysis of this data. In addition, we determined the size of the OPH2005 trial in STGD1 based on the number of patients with STGD1 that we believe could potentially be enrolled within a reasonable period of time. This number may be increased or decreased in light of the actual enrollment rate during the trial. As STGD1 is an orphan indication, to our knowledge there is only very limited natural history data currently available regarding the variability for our planned primary efficacy endpoint in the STGD1 patient population we plan to enroll in this trial. Given the information above, these trials could be underpowered to demonstrate a potential clinical benefit for Zimura in these indications.

With respect to business development efforts and pipeline expansion activities, our research collaboration with the University of Massachusetts Medical School, or UMMS, is for very early stage technology which may never translate into clinical development programs. We may be unable to secure additional collaborations, partnerships or in-licensing or acquisition opportunities.

We may continue to reassess and make changes to our existing development programs and pipeline expansion strategy. Our future plans for our Zimura development program may be affected by the results of competitors' clinical trials of complement inhibitors or other product candidates that may compete with ours. Our plans for our business development efforts and pipeline expansion activities may be affected by the results of competitors' ongoing research and development efforts. We may modify, expand or terminate some or all of our development programs, clinical trials or collaborative research programs at any time as a result of new competitive information or as the result of changes to our product pipeline or business development strategy.

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

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 expand our clinical pipeline would likely impair our ability to grow.

An important 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. In addition, we have recently added gene therapy research as an area of interest for our strategy. Because we expect generally that we will not engage directly in internal early stage research and drug discovery efforts, 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, including any promising product candidates that may emerge from our collaborative gene therapy research programs, including, for example, our collaboration with UMMS, for which we have an option to obtain an exclusive license to patents and patent applications resulting from the sponsored research but for which we have not yet agreed to license terms. 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. We believe that other companies may be particularly active in pursuing opportunities to in-license or acquire promising gene therapy opportunities. 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 therapeutic modalities, 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 business plan.

Our business plan may not be successful, or we may be unsuccessful in effectively executing our 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.

We are a clinical-stage company. The relatively early stage of our business may make it difficult for our stockholders to assess our viability as a potential commercial-stage company in the future.

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 Zimura, Fovista and other product candidates. We have not yet demonstrated our ability to successfully complete a large-scale, pivotal clinical trial with safety and efficacy 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. 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 a history of significant operating losses. 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. To date, we have not generated any revenues from commercial 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 Fovista Licensing and Commercialization Agreement with Novartis Pharma, AG, which we refer to as the Novartis Agreement, which we entered into in May 2014 and which was terminated by Novartis in October 2017. As of March 31, 2018, we had an accumulated deficit of $497.8 million. Our net loss was $13.1 million for the three months ended March 31, 2018 and we expect to continue to incur significant operating losses for the foreseeable future.

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. Although we are no longer pursuing the development of Fovista, we expect to continue to incur significant expenses and operating losses over the next few years as we continue the development of Zimura and potentially add to our product portfolio through in-licensing or acquisition of additional product candidates. Our net losses may fluctuate significantly from quarter to quarter and year to year.

Our product candidate Zimura is in clinical development. We expect to continue to incur significant research and development expenses as we pursue the development of Zimura as currently planned. We could also 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 in additional indications, beyond those already in development, and as we evaluate and potentially in-license or acquire, and undertake development of, additional product candidates, including any promising product candidates that emerge from our collaborative gene therapy research programs. 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. We are party to an agreement with Archemix that imposes significant milestone payment obligations on us in connection with our achievement of specific clinical, regulatory and commercial milestones with respect to Zimura. 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 currently planned 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;
- pursue our collaborative gene therapy research programs;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, manufacturing, quality control, quality assurance and scientific personnel, especially if we are successful in acquiring or in-licensing rights to additional products, product candidates or technologies or progressing the clinical development of any of our product candidates or if we decide to establish internal gene therapy capabilities;
- seek marketing approval for any product candidates that successfully complete clinical trials;
- expand our outsourced manufacturing activities or establish commercial operations or sales, marketing and distribution capabilities, if we receive, or expect to receive, marketing approval for any of our product candidates; and
- expand our general and administrative functions to support future growth of the company.

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 March 31, 2018, we had cash and cash equivalents of $154.9 million. We estimate our year end 2018 cash and cash equivalents to range between $112.0 million and $117.0 million based on our current 2018 business plan and planned capital expenditures. This estimate includes continuation of our development programs for Zimura and the continuation of our collaborative gene therapy research programs as currently planned. We also had $133.5 million in total liabilities as of March 31, 2018, of which $125.0 million related to the Novo Agreement, which we are required to show as a liability on our balance sheets under generally accepted accounting principles but which does not correspond to any contractual repayment obligation.

We believe that our cash and cash equivalents 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 additional sponsored research or 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 scope of any additional collaborative research programs, 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 any unforeseen issue in our ongoing clinical trials, such as delays in enrollment or with the availability of drug supply, or if we further expand the scope or size of our clinical trials or collaborative research programs. Our costs may also exceed our expectations for other reasons, for example, if we experience issues with manufacturing or 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 our product candidates is highly uncertain. 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 clinical development, to complete process development and manufacturing scale-up and validation activities or to potentially seek marketing approval with respect to our product candidates.

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

- the scope, costs and results of our ongoing Zimura clinical programs, as well as any additional clinical trials we undertake to obtain data sufficient to seek marketing approval for Zimura in any indication;
- the extent to which we in-license or acquire rights to, and undertake research or development of products, product candidates or technologies, including any product candidate or other technologies we may evaluate as part of our collaborative gene therapy research programs;
- 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 develop;
- the costs and timing of process development, manufacturing scale-up and validation activities and ongoing stability studies associated with Zimura or any other product candidates that we may develop;

- 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.

We do not have any committed external source of funds. 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 may not be available for at least several years, if at all. In addition, if approved, our product candidates 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.

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.

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 a purported consolidated putative class action lawsuit initiated in 2017 that 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 agents for the treatment of wet AMD. The members of our Board of Directors have also been named as defendants in a shareholder derivative action initiated on February 7, 2018, which generally alleges that defendants breached their fiduciary duties to our company by adopting a compensation plan that overcompensates the non-employee members of the Board relative to the boards of companies of comparable market capitalization and size. These complaints seek equitable and/or injunctive relief, unspecified damages, attorneys' fees, and other costs. The defendants 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 and our Board of Directors' 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.

The recently passed comprehensive tax reform bill could adversely affect our business and financial condition.

On December 22, 2017, United States President Donald J. Trump signed into law new legislation that significantly revised the Internal Revenue Code of 1986, as amended. The newly enacted federal income tax law, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modification or repeal of many business deductions and credits. Notwithstanding the reduction in the corporate income tax rate, the overall impact of the new federal tax law is uncertain and our business and financial condition could be adversely affected. In addition, it is uncertain how various states will respond to the newly enacted federal tax law. The impact of this tax reform on holders of our common stock is also uncertain and could be adverse. We urge our stockholders to consult with their legal and tax advisors with respect to this legislation and the potential tax consequences of investing in or holding our common stock.

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 successfully completing pre-clinical development activities, including pre-clinical efficacy and IND-enabling studies, for our product candidates or product candidates we are interested in in-licensing or acquiring, including product candidates we may evaluate as part of our collaborative gene therapy research programs;
- 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 and 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. Prior to initiating clinical trials, a sponsor must complete extensive pre-clinical testing of a product candidate, including, in most cases, pre-clinical efficacy experiments as well IND-enabling toxicology studies. These experiments and studies may be time-consuming and expensive to complete. The necessary pre-clinical testing may not be completed successfully for a pre-clinical product candidate and a potentially promising product candidate may therefore never be tested in humans. Once it commences, 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 Phase 3 Fovista program for the treatment of wet AMD.

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 STGD1 or non-infectious intermediate and posterior uveitis.

Given that we have limited data regarding the effect of Zimura in GA, we determined the size of the OPH2003 trial in GA based on our best estimates of the size of trial required to demonstrate a potential clinical benefit for Zimura. This estimate incorporates our assumptions regarding the potential performance of Zimura in this indication based in part on available third-party clinical data and our statistical analysis of this data. In addition, we determined the size of the OPH2005 trial in STGD1 based on the number of patients with STGD1 that we believe could potentially be enrolled within a reasonable period of time. This number may be increased or decreased in light of the actual enrollment rate during the trial. As STGD1 is an orphan indication, to our knowledge there is only very limited natural history data currently available regarding the variability for our planned primary efficacy endpoint in the STGD1 patient population we plan to enroll in this trial. Given the information above, these trials could be underpowered to demonstrate a potential clinical benefit for Zimura in these indications.

Furthermore, our current and planned Zimura clinical trials are evaluating or will evaluate Zimura dosing regimens that we have not studied before, which may increase the risk that patients in these trials experience adverse events and/or serious adverse events (either ocular, systemic or both) that we have not observed or at rates that we have not observed in prior trials. For a further discussion of the safety risks in our trials, see the risk factor herein entitled "*If serious adverse or unacceptable side effects are identified during the development of our product candidate, we may need to abandon or limit our development of such product candidate.*"

Moreover, the failure of prior clinical trials evaluating complement inhibition in GA, including a competitor's two Phase 3 clinical trials evaluating an investigational anti-complement factor D antibody administered via intravitreal injections, a second competitor's Phase 2 clinical trial evaluating an investigational anti-C5 antibody administered via intravitreal injections and a third competitor's Phase 2 clinical trial evaluating an anti-C5 antibody administered systemically, may call into question the hypothesis underlying the use of a complement inhibitor as a method for treating GA. In addition, the competitor's 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-experienced 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, especially in cases where we are working with clinical research organizations or clinical trial sites we have not worked with previously;
- our contract research organizations, clinical trial sites, contract manufacturers and packagers and analytic testing service providers 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, including the selection of the primary efficacy endpoint for a pivotal clinical trial, 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. These risks may be heightened for clinical trials in orphan diseases, for which the natural history of the disease is less understood, making it more difficult to predict the drug effect required to adequately demonstrate efficacy, and because there are fewer affected patients available to participate in clinical trials;
- the cost of clinical trials of our product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or product candidates we are investigating 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 in our wet AMD and IPCV trials, may be insufficient or inadequate or we may face

delays in the manufacture and supply of our product candidates as a result of a 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; and

- we may face delays in the manufacture and supply of any product candidates we are investigating in our collaborative gene therapy research programs as a result of our inability to establish new manufacturing capabilities or processes.

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.

We have no experience in gene therapy clinical development. Our lack of experience may limit our ability to be successful or may delay our development efforts.

Gene therapy is an emerging field of drug development with only one gene replacement therapy having received FDA approval to date. The novel gene therapy delivery technologies and "minigene" therapy approaches we are evaluating in our collaboration with UMMS are particularly early-stage in their development. Even with promising pre-clinical efficacy data for a new gene therapy product candidate, there will remain several areas of drug development risk, including translational science, manufacturing techniques, safety concerns, regulatory pathway, clinical trial design and the approach to ocular gene therapy administration through either sub-retinal surgery or intravitreal delivery, which will likely pose particular uncertainty given the relatively limited development history for gene therapies. Although we believe gene therapy is a promising area for ophthalmic drug development, we do not have any internal gene therapy development experience or specific gene therapy capabilities. In entering this new area, we will need to build significant technical capabilities, including translational, manufacturing, process development, and other capabilities. We will either need to hire internally for these capabilities or establish them through outside service providers. We believe that gene therapy is an area of significant investment by biotechnology and pharmaceutical companies and that there may be a scarcity of talent available to us in these areas. If we are not able to establish our own internal or outsourced gene therapy capabilities, we may not be able to develop promising product candidates that emerge from our collaborative gene therapy research programs, which would limit our prospects for future growth.

If serious adverse or unacceptable side effects are identified during the development of our product candidate, 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.

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 STGD1 or non-infectious intermediate and posterior uveitis. Our clinical trials for Zimura involve dosing regimens that we have not studied before, which may increase the risk that patients in these trials experience adverse events and/or serious adverse events (either ocular, systemic or both) that we have not observed or at rates that we have not observed in prior trials. In addition, 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. For these reasons, 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 Zimura monotherapy or Zimura in combination with anti-VEGF therapy. Because we currently have only one product candidate in clinical development, it is possible that a safety issue in any of our ongoing clinical trials for Zimura could impact all of our ongoing clinical trials.

In addition, there are several known safety risks specific to gene therapy, including inflammation resulting from a patient's immune response to the administration of viral vectors and the potential for toxicity as a result of chronic exposure to the expressed protein. In the event that we in-license or acquire a gene therapy product candidate and progress it into clinical development, we may experience delays or other challenges for our development programs as a result of safety issues.

Our experience manufacturing Zimura is limited. In addition, we have no experience manufacturing gene therapy product candidates. Manufacturing issues, including technical or quality issues or issues securing capacity, may arise that could cause delays in our development programs or increase costs. Furthermore, we may experience delays in regulatory approval of our product candidates if we do not satisfy applicable manufacturing regulatory requirements.

We do not have any internal manufacturing facilities, personnel or other capabilities and are dependent on outside contract manufacturers to manufacture Zimura and any other product candidates that we would acquire or in-license as part of pursuing our 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 API for Zimura and a different, single third-party manufacturer, Ajinomoto Althea, to provide fill/finish services for Zimura. In order to obtain and maintain regulatory approval for Zimura, our third-party manufacturers will be required to consistently produce the API used in Zimura 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. 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. 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. The lack of uniform manufacturing and quality standards among regulatory agencies may delay regulatory approval of 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.

Gene therapy drug products are complex and difficult to manufacture. A number of factors common to the manufacturing of most biologics and drugs could also cause production interruptions, including raw materials shortages, raw material failures, growth media failures, equipment malfunctions, facility contamination, labor problems, natural disasters, disruption in utility services, terrorist activities, or acts of god that are beyond our control.

We believe that the high demand for clinical gene therapy material and a scarcity of potential contract manufacturers may cause long lead times for establishing manufacturing capabilities for gene therapy drug development activities. There may also be long lead times to purchase manufacturing materials, including for GMP compliant material needed for clinical trials. It is often the case that early stage research is conducted with materials that are not manufactured using GMP techniques or processes and which are not subject to the same level of analysis that would be required for clinical grade material. Therefore, to the extent that we in-license or acquire a new gene therapy product candidate, we may need to devote significant time and financial resources to establishing manufacturing processes that are sufficient for clinical supplies. In addition, because early stage, pilot manufacturing is often done on a small scale, we may face challenges scaling up any early stage manufacturing to the scale necessary to support supply for clinical trials. If we are not able to establish gene therapy manufacturing or related processes, our development plans may be delayed or stalled and our business may be materially harmed.

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 Zimura. 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 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 the use of our combination therapy product candidates, such as Zimura for the treatment of wet AMD or IPCV, limiting their use in combination with particular standard of care drugs, such as a particular anti-VEGF drug;
- restrictions in the label imposing a waiting period in between intravitreal injections;
- 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 50;
- 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 or to the extent our product candidates require invasive procedures for administration, such as subretinal surgery;
- prevalence and severity of any side effects or perceived safety concerns, especially for new therapeutic modalities such as gene therapy; 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.

For each of our Zimura trials where patients will receive multiple intravitreal injections on the same day, either Zimura in combination with an anti-VEGF agent or multiple Zimura injections, we have provided for a delay in the second intravitreal injection to minimize the risk of an unacceptable increase in intraocular pressure as a result of the volume of the multiple injections. If Zimura receives marketing approval for a particular indication and the approved label requires a waiting period, the potential market opportunity for Zimura may be limited to the extent that physicians and patients find such a waiting period unacceptable.

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 products approved and marketed for wet AMD or orphan retinal diseases, 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, as well as generic and biosimilar 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 GA, wet AMD, Stargardt disease, non-infectious intermediate and posterior uveitis or other disease indications for which we may develop Zimura. 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. In particular, many companies are pursuing gene therapy approaches for age-related and orphan retinal diseases.

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 method of administration of Zimura, intravitreal injection, is commonly used to administer ophthalmic drugs for the treatment of severe disease and is generally accepted by patients facing the prospect of severe visual loss or blindness. A therapy that offers a less invasive method of administration, however, might have a competitive advantage over one administered by intravitreal injection, depending on the relative safety of the other method of administration.

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.

Competitive considerations for Dry AMD and GA:

- There are a number of products in preclinical research and clinical development by third parties to treat dry AMD, including several that are in development for GA secondary to 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 perfusion enhancers. Based on publicly available information, we are aware that Apellis Pharmaceuticals, Inc., Novartis AG and MorphoSys AG, Hemera Biosciences, Inc., Achillion Pharmaceuticals, Inc. and Catalyst Biosciences, Inc. each have complement inhibitors in development, the most advanced of which we believe is Apellis's pegylated, synthetic peptide targeting complement factor C3. Apellis announced positive Phase 2 results for its product candidate and has announced plans to initiate a Phase 3 program during 2018. If Apellis's Phase 3 program for its complement factor C3 product candidate is successful, it is likely that Apellis may obtain marketing approval for its product candidate 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 system.

Competitive considerations for wet AMD:

- 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 multiple mechanisms of action are in clinical or pre-clinical development for wet AMD, including angiopoietin-2 inhibitors, tyrosine kinase inhibitors, integrin inhibitors, novel VEGF inhibitors and complement inhibitors, as well as a few remaining PDGF inhibitors. Within the complement system, we are aware that Apellis is planning a Phase 1b/2 clinical trial with their C3 inhibitor in combination with anti-VEGF therapy. 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 topical dosing of therapeutic agents such as anti-VEGF agents or integrin inhibitors through eye-drops or to allow for a less frequent intravitreal dosing schedule than currently used for standard of care anti-VEGF agents.

- The commercial opportunity for Zimura in wet AMD in particular 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, since we are only developing Zimura for wet AMD as a combination therapy with anti-VEGF agents. Moreover, we expect that if Zimura 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 Zimura in combination with these drugs. This could limit sales of Zimura for this indication.

Competitive considerations for Stargardt disease:

- 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., Vision Medicines, Inc., Lin BioScience, Inc., Nightstar Therapeutics plc and ProQR Therapeutics N.V. each have development programs in Stargardt disease. In addition, several academic organizations have early programs in Stargardt disease. Four of these programs, Acucela, Alkeus, Vision Medicines and Lin BioScience, are exploring the use of oral therapeutics, while Sanofi, with technology provided by Oxford BioMedica plc, and Nightstar are each using a gene therapy approach and ProQR is using an RNA interference 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. The Orphan Drug Act only provides exclusivity periods for the specific drug granted orphan designation for a specific indication. In addition, 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.

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. 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.

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, pricing dynamics, 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, including increasing scrutiny of drug prices, 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.

In addition, even in countries where pharmaceuticals are not subject to strict pricing regulations through a governmental review and approval process, we may nonetheless face an unfavorable pricing environment as a result of political pressure or market dynamics. The pricing for products intended to treat orphan diseases in particular may be perceived as too high to be justified. The perceived high cost for pharmaceutical products to treat orphan diseases may attract increased political and public scrutiny. Moreover, if we obtain marketing approval for a product candidate, such as Zimura, in more than one indication, including, for example in an orphan indication such as STGD1 and a non-orphan indication such as GA secondary to dry AMD, or in a monotherapy indication, such as GA secondary to dry AMD and a combination therapy indication such as wet AMD, such a product candidate likely would only be sold at one price in any given country, regardless of the indications for which it is prescribed. This dynamic may result in our charging a price that does not generate profits in each indication for which the product is approved.

Our ability and the ability of any commercialization partner to commercialize a 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 any 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 Zimura for the treatment of wet AMD or IPCV, our drug would 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 Zimura to the cost of treatment with anti-VEGF drugs for the treatment of wet AMD or IPCV. 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 Zimura for the treatment of wet AMD or IPCV, 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 any 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 future 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 future commercialization partner will face an even greater risk if we commercially sell any products that we develop or in-license. Because our Zimura wet AMD and IPCV trials involve or will involve the administration of Zimura in combination with an anti-VEGF drug, we also face an inherent risk of product liability exposure related to the testing of such anti-VEGF drug. 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 a commercialization or collaboration partner 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 collaborations to advance our development programs, 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. In addition, the commercialization of a product candidate in markets outside of the United States requires regulatory expertise and commercial capabilities that are specific to the local market. 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. These collaborations carry numerous risks. 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. 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 will depend on our collaborators' abilities and efforts to successfully perform the functions assigned to them in these arrangements.

Collaborations involving our product candidates could pose numerous risks to us, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations and may not perform their obligations as expected;
- collaborators may deemphasize or not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus, changes in product candidate priorities or available funding or changes in priorities as a result of a merger, acquisition or other corporate restructuring or transaction;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- we could grant exclusive rights to our collaborators, which would prevent us from collaborating with others;
- disagreements or disputes with collaborators, including disagreements or disputes over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of products or product candidates, might lead to additional responsibilities for us with respect to product candidates or might result in litigation or arbitration, any of which would divert management attention and resources, be time-consuming and be expensive;
- collaborators with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- collaborators may not properly maintain or defend our intellectual property rights, may infringe the intellectual property rights of third parties, may misappropriate our trade secrets or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to litigation and potential liability; and
- collaborations may be terminated for the convenience of the collaborator, our breach of the terms of the collaboration or other reasons and, if terminated, we may need to raise additional capital to pursue further development or commercialization of the applicable product candidates.

If a collaborator of ours 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 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, in part, on third-party researchers to advance our pipeline expansion efforts. These arrangements may not ultimately yield any promising product candidates for clinical development.

Part of our pipeline expansion strategy involves collaborative sponsored research to be performed by third-party research institutions. Although we seek to direct this research and advise on the design of these projects as well as critical development decisions, this research is being performed by individuals that are not our employees and the timeline and quality of the research efforts are outside of our direct control. Academic investigators and other researchers may have different priorities than we do as a biopharmaceutical drug development company. Confidential information and new inventions derived from these research efforts may be disclosed through publications or other means prior to our being able to protect such intellectual property through the filing of patent applications. Our third-party research partners may not be able to obtain or maintain full ownership of inventions that are derived from the research or associated rights, which may limit their ability to provide us with a license to all relevant intellectual property on terms and conditions that are acceptable to us. Even if our collaborative research efforts yield promising results or new technological advances, they may not ultimately result in our being able to develop or exploit the resulting intellectual property.

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 in the past and expect in the future to rely upon third parties, such as CROs, clinical data management organizations, biostatisticians, academic research collaborators, medical institutions (including reading centers) and clinical investigators, in conducting our pre-clinical testing and 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 pre-clinical testing and 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, label 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 our Zimura for clinical trials and expect to continue to do so in connection with its potential commercialization and for materials for development activities and commercialization of any other product candidates that we 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 or quality, 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 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 or differing priorities on the part of our existing or future manufacturers could delay clinical development or marketing approval.

We currently rely exclusively upon a single third-party manufacturer to provide supplies of Zimura API and a different single third-party manufacturer to provide fill/finish services for Zimura. We do not currently have any contractual commitments for the supply of Zimura API. We also do not currently have arrangements in place for redundant supply or a second source for API for Zimura or for a redundant supply or a second source for fill/finish services. We purchase the proprietary polyethylene glycol, or PEG, reagent used to modify the chemically synthesized aptamer in Zimura on a purchase order basis from a single third-party supplier. We do not currently have any contractual commitments for supply of the PEG reagent we use for Zimura. 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. We currently expect to rely on third-party manufacturers for any manufacturing needs for our collaborative gene therapy research programs or future gene therapy clinical trials.

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, differing priorities, 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 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 Zimura. We may enter into similar arrangements with respect to future product candidates. Termination of these rights or the failure by us or our licensees, including our potential future commercialization or collaboration partners, to comply with obligations under these or other agreements could materially harm our business and prevent us from developing or commercializing our products and product candidates.

We are party to a license agreement with Archemix on which we depend for rights to Zimura. This agreement imposes diligence, development and commercialization timelines, milestone payment, royalty, insurance and other obligations on us. Generally, the diligence obligations contained in the agreement require us to use commercially reasonable efforts to develop, seek regulatory approval for and commercialize Zimura in the United States, the European Union, Japan and such other markets where it would be commercially reasonable to do so. Under our license agreement with Archemix 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 expect to enter into acquisition or licensing agreements in the future that would

impose similar obligations on us, particularly as we pursue our business plan to acquire or in-license additional products, product candidates or other technologies and expand our product pipeline.

If we fail to comply with our obligations under current or future acquisition and licensing agreements, or otherwise breach an acquisition or licensing 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. 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 Zimura and other important intellectual property or technology. Any of the foregoing could prevent us from commercializing 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 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. 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 Zimura and other product candidates may be materially harmed. 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 or do not 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 U.S. patent rights covering Zimura as a composition of matter are expected to expire in 2025. The U.S. patent rights covering methods of treating certain complement protein mediated disorders with Zimura are expected to expire in 2026. The European patent rights covering the composition of matter of Zimura and methods of treating certain complement protein mediated disorders with Zimura are expected to expire in 2025. As we expect the clinical development of Zimura to continue for at least the next several years, these expiration dates may be prior to the date by which we would be able to commercialize Zimura in the United States or Europe if we seek and obtain marketing approval. Once our patents expire, 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 patent term restoration for one of our patents in the United States under the Hatch-Waxman Act, which 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, but we can provide no assurances that such a restoration term will be obtained. 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.

Certain of our licensed patent rights for Zimura are method-of-treatment patents. 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 Zimura or any other product candidates we may develop would limit our ability to generate revenue from the sale of 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 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 Zimura or such other product candidate in that jurisdiction so long as these competitors do not infringe any other of our patents covering 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, 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 our product candidates, if they are ultimately approved.

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 or take other actions 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 any future 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. New patent applications in the field of biotechnology and pharmaceuticals, and gene therapies in particular, are being filed at a rapid pace.

There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries. We or any future 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. There is a lag between the filing of a patent application, which generally establishes the priority date of a patent claim, and the publication of such patent application. During the period between filing of a patent application and publication of the application, we would not otherwise have a means of discovering the existence or extent of the claimed inventions contained in a filed but unpublished patent application. Patent applications are often drafted broadly, and the scope of patent claims that may ultimately issue may not be known until several years after a patent application is filed and published. We may make development or pipeline decisions based on our belief that our product candidates can be distinguished from patent claims contained in published patent applications or issued patents, that patent claims contained in published patent applications are unlikely to issue as drafted, or that claims contained in issued patents are invalid. These positions regarding third-party intellectual property may not ultimately be successful in litigation. Thus, we do not know with certainty that our product candidates, or our intended commercialization thereof, does not and will not infringe or otherwise violate any third party's intellectual property.

If we are or any of our future 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 future 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 future 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 future 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 future 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 Zimura from Archemix, we must rely upon Archemix's and its successors' practices, and those of its 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 Zimura from Archemix, we must rely upon Archemix's and its successors' practices, and those of its predecessors, with regard to the protection of Zimura-related trade secrets before we acquired it. 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 any of our product candidates 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 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 a 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. In the case of Zimura for the treatment of wet AMD or IPCV, the FDA or other regulatory authority may limit the approval of Zimura to use with only specified anti-VEGF drugs that are approved for the treatment of wet AMD or IPCV rather than with all anti-VEGF drugs. Such limitation could limit sales of Zimura for the treatment of wet AMD or IPCV.

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 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 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 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 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.

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 a product has received fast track designation and may be eligible for priority review status, a sponsor may not ultimately 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 potential 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 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, which 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 future 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, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of any collaborators, to profitably sell any products 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 any future collaborators, may receive for any approved products.

In March 2010, President Barack H. Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education 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;
- 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;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements to report certain financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report product samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. These changes include the Budget Control Act of 2011, which, among other things, led to aggregate reductions to Medicare payments to providers of up to 2% per fiscal year that started in 2013 and, due to subsequent legislative amendments to the statute, will stay in effect through 2025 unless additional congressional action is taken, and the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several types of 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. Further, there have been several recent U.S. congressional inquiries and proposed state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products.

We expect that these healthcare reforms, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. Since enactment of the ACA, there have been numerous legal challenges and congressional actions to repeal and replace provisions of the law. In May 2017, the U.S. House of Representatives passed legislation known as the American Health Care Act of 2017. Thereafter, the Senate Republicans introduced and then updated a bill to replace the ACA known as the Better Care Reconciliation Act of 2017. The Senate Republicans also introduced legislation to repeal the ACA without companion legislation to replace it, and a "skinny" version of the Better Care Reconciliation Act of 2017. In addition, the Senate considered proposed healthcare reform legislation known as the Graham-Cassidy bill. None of these measures was passed by the U.S. Senate.

The Trump Administration has also taken executive actions to undermine or delay implementation of the ACA. In January 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. In October 2017, the President signed a second Executive Order allowing for the use of association health plans and short-term health insurance, which may provide fewer health benefits than the plans sold through the ACA exchanges. At the same time, the Administration announced that it will discontinue the payment of cost-sharing reduction, or CSR, payments to insurance companies until Congress approves the appropriation of funds for such CSR payments. The loss of the CSR payments is expected to increase premiums on certain policies issued by qualified health plans under the ACA. A bipartisan bill to appropriate funds for CSR payments was introduced in the Senate, but the future of that bill is uncertain.

More recently, with enactment of the Tax Cuts and Jobs Act of 2017, which was signed by the President on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, will become effective in 2019. According to the Congressional Budget Office, the repeal of the individual mandate will cause 13 million fewer Americans to be insured in 2027 and premiums in insurance markets may rise. Further, each chamber of the Congress has put forth multiple bills designed to repeal or repeal and replace portions of the ACA. Although none of these measures has been enacted by Congress to date, Congress may consider other legislation to repeal and replace elements of the ACA. The Congress will likely consider other legislation to replace elements of the ACA during the next congressional session.

We will continue to evaluate the effect that the ACA and its possible repeal and replacement could have on our business. It is possible that 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. Accordingly, such reforms, if enacted, could have an adverse effect on anticipated revenue from product candidates that we may successfully develop and for which we may obtain marketing approval and may affect our overall financial condition and ability to develop or commercialize product candidates. While the timing and scope of any potential future legislation to repeal and replace ACA provisions is highly uncertain in many respects, it is also possible that some of the ACA provisions that generally are not favorable for the research-based pharmaceutical industry could also be repealed along with ACA coverage expansion provisions.

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. In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our products. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs.

Finally, legislative and regulatory proposals have also been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us and any future collaborators to more stringent product labeling and post-marketing testing and other requirements.

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

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

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

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

We and our third-party manufacturers are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials,

including chemicals and biological materials, and produce hazardous waste products. We cannot eliminate the risk of contamination or injury 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 recently completed a substantial reduction in personnel, the effects of which could disrupt our operations. In addition, we may experience difficulties in retaining key employees.

During the year ended December 31, 2017, our workforce was reduced by 122 employees in connection with a reduction in personnel following the failure of our pivotal trials of Fovista as well as natural attrition. Nonetheless, 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.

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, 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 business plan.

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, pipeline expansion and commercialization strategies. 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.

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 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 personal and other 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, business partners and employees. In particular, we rely on contract research organizations and other third parties to store and manage information from our clinical trials. 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 compromise, damage, loss or exfiltration from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. In particular, companies and other entities and individuals have been increasingly subject to a wide variety of security incidents, cyber-attacks, phishing scams and other attempts to gain unauthorized access to systems and information, including through social engineering. These threats can come from a variety of sources, ranging in sophistication from individual hackers to state-sponsored attacks. Cyber threats may be broadly targeted, 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. We might not 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 or unauthorized exposure of personal information of our clinical trial patients, our employees or others. Cyber-attacks and the measures we implement to prevent, detect, and respond to them could cause us to incur significant remediation costs, result in product development delays, disrupt key business operations, expose us

to contractual damages and/or regulatory liability, require us to make certain breach notifications, divert the attention of our management and key information technology resources, harm our reputation and deter patients, clinical investigators or other business partners from participating in our clinical trials or otherwise working with us. Any 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 the first two of our pivotal 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 and declined further thereafter. The closing price of our common stock was $2.62 on May 8, 2018. 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 following our announcement of the initial, top-line results. See “Part II, Item 1—Legal Proceedings” and “—Risks Related to Our 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.” These proceedings 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.

PART IV

Item 6. Exhibits and Financial Statement Schedules

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.

(2) Financial Statement Schedules

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

(3) Exhibits

Exhibit Number	Description of Exhibit
3.1	Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.3 to Amendment No. 2 of the Registrant's Registration Statement on Form S-1 (File No. 333-190643) filed with the SEC on September 9, 2013)
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.4 to Amendment No. 2 of the Registrant's Registration Statement on Form S-1 (File No. 333-190643) filed with the SEC on September 9, 2013)
31.1	Certification of principal executive officer pursuant to Rule 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended
31.2	Certification of principal financial officer pursuant to Rule 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended
32.1	Certification of principal executive officer pursuant to 18 U.S.C. §1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of principal financial officer pursuant to 18 U.S.C. §1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS*	XBRL Instance Document
101.SCH*	XBRL Taxonomy Extension Schema Document
101.CAL*	XBRL Taxonomy Calculation Linkbase Document
101.DEF*	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	XBRL Taxonomy Label Linkbase Document
101.PRE*	XBRL Taxonomy Presentation Linkbase Document

***** Submitted electronically herewith.

Attached as Exhibit 101 to this report are the following formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheets at March 31, 2018 (unaudited) and December 31, 2017, (ii) Statements of Operations (unaudited) for the three month periods ended March 31, 2018 and 2017, (iii) Statements of Cash Flows (unaudited) for three month periods ended March 31, 2018 and 2017 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.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

OPHTHOTECH CORPORATION

Date: May 9, 2018

By: /s/ David F. Carroll
David F. Carroll
Chief Financial Officer
(Principal Financial and Accounting Officer)

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 March 31, 2018 of Ophthotech Corporation;

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

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

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

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

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: May 9, 2018

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 March 31, 2018 of Ophthotech Corporation;

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

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

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

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

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: May 9, 2018

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 March 31, 2018 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 Rule 13a-14(b) and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 9, 2018

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 March 31, 2018 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 Rule 13a-14(b) and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 9, 2018

By: /s/ David F. Carroll

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