Ophthotech Corp.
Form 10-Q
November 13, 2013
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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2013

OR

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

Commission file number: 001-36080

Ophthotech Corporation

(Exact name of registrant as specified in its charter)

Delaware	**20-8185347**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)

One Penn Plaza, 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 x

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

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

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

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

As of November 8, 2013 there were 31,373,489 shares of Common Stock, $0.001 par value per share, outstanding.

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PART II OTHER INFORMATION

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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, po should, continue and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this Quarterly Report on Form 10-Q include, among other things, statements about:

- the timing, costs, conduct and outcome of our clinical trials of Fovista administered in combination with anti-VEGF drugs for the treatment of wet age-related macular degeneration, including statements regarding the timing of the initiation of, the availability of, and the costs to obtain, initial top-line results from, and the completion of such trials and the timing of regulatory filings;

- the timing of and our ability to obtain marketing approval of Fovista and our other product candidates, and the ability of Fovista and our other product candidates to meet existing or future regulatory standards;

- the potential receipt of revenues from future sales of Fovista;

- our plans to pursue research and development of other product candidates;

- the potential advantages of Fovista;

- the rate and degree of market acceptance and clinical utility of Fovista;

- our estimates regarding the potential market opportunity for Fovista;

- our sales, marketing and distribution capabilities and strategy;

- our ability to establish and maintain arrangements for manufacture of Fovista and our other product candidates;
- our ability to in-license or acquire approved products, additional product candidates or technologies;
- our intellectual property position;
- our expectations related to our use of available cash;
- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;
- the impact of governmental laws and regulations; and
- our competitive position.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in the Risk Factors section, that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

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PART I FINANCIAL INFORMATION

Item 1. Financial Statements.

Ophthotech Corporation

(A Development Stage Entity)

Unaudited Balance Sheets

(in thousands, except share and per share data)

	September 30, 2013	December 31, 2012
Assets		
Current assets		
Cash and cash equivalents	$ 236,079	$ 4,304
Prepaid expenses and other current assets	2,096	44
Other Assets		331
Security deposits	158	158
Total current assets	238,333	4,837
Property, plant and equipment, net	32	42
Other long term assets	11	
Total assets	$ 238,376	$ 4,879
Liabilities, Convertible Redeemable Series A, Series A-1, Series B, Series B-1 Preferred Stock and stockholders equity (deficit)		
Current liabilities		
Accrued clinical drug supplies and trial costs	$ 3,477	1,013
Accounts payable and accrued expenses	4,431	1,391
Notes payable		11,040
Warrant liability		966
Total current liabilities	7,908	14,410
Royalty purchase liability	41,667	
Total liabilities	49,575	14,410
Preferred Stock, Convertible and Redeemable:		
Series A - $0.001 par value, 73,094,000 shares authorized, 51,790,000 shares issued and outstanding at December 31, 2012		69,471
Series A-1 - $0.001 par value, 18,480,000 shares authorized, 6,000,000 shares issued and outstanding at December 31, 2012		8,460
Series B - $0.001 par value, 42,320,200 shares authorized, 30,000,000 shares issued and outstanding at December 31, 2012		35,456
Series B-1 - $0.001 par value, 700,000 shares authorized, 500,000 shares issued and outstanding at December 31, 2012		552
Total Preferred Stock, Convertible and Redeemable		113,939
Stockholders equity (deficit)		
Junior Series A Convertible Preferred Stock - $0.001 par value, 3,000,000 shares authorized, issued and outstanding at December 31, 2012		3,000

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, 31,250,817 shares issued and outstanding at September 30, 2013; 155,864,851 shares authorized, 1,469,798 shares issued and outstanding at December 31, 2012		31		1
Additional paid-in capital		351,431		
Deficit accumulated during development stage		(162,661)		(126,471)
Total stockholders equity (deficit)		188,801		(123,470)
Total liabilites and stockholders equity (deficit)	$	238,376	$	4,879

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

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OPHTHOTECH CORPORATION

(A Development Stage Entity)

Unaudited Statements of Operations

(in thousands, except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,		Period from January 5, 2007 (Inception) to September 30,
	2013	2012	2013	2012	2013
Costs and expenses:					
Research and development	$ 11,101	$ 1,595	$ 17,836	$ 4,794	$ 92,727
General and administrative	4,166	2,259	9,145	5,341	36,494
Total costs and expenses	15,267	3,854	26,981	10,135	129,221
Loss from operations	(15,267)	(3,854)	(26,981)	(10,135)	(129,221)
Interest expense		(230)	(1,454)	(256)	(1,964)
Interest and other income					481
Gain (loss) on extinguishment of debt	105		(1,091)		(1,091)
Other loss	(970)	(69)	(1,231)	(340)	(1,602)
Change in fair value related to investor rights liability					683
Net loss before income tax benefit	(16,132)	(4,153)	(30,757)	(10,731)	(132,714)
Income tax benefit					1,327
Net loss	(16,132)	(4,153)	(30,757)	(10,731)	(131,387)
Add: accretion of preferred stock dividends	(2,292)	(1,775)	(5,891)	(5,288)	(33,046)
Net loss attributable to common stockholders	$ (18,424)	$ (5,928)	$ (36,648)	$ (16,019)	$ (164,433)
Net loss attributable to common stockholders per share - basic and diluted	$ (10.26)	$ (4.07)	$ (23.21)	$ (11.07)	
Weighted average common shares outstanding:					
Basic and diluted	1,795	1,455	1,579	1,447	

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

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OPHTHOTECH CORPORATION

(A Development Stage Company)

Unaudited Statements of Cash Flows

(in thousands)

	Nine months ended September 30, 2013	2012	Period from January 5, 2007 (Inception) to September 30, 2013
Operating Activities			
Net loss	$ (30,757)	$ (10,731)	$ (131,387)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation	14	24	169
Amortization of debt issuance costs	88	24	135
Accretion of debt discount	87	30	146
Non-cash change in fair value of warrant liability	1,231	332	1,612
Non-cash change in fair value of investor rights liability			(683)
Loss on extinguishment of debt	1,091		1,091
Share-based compensation	1,607	283	2,797
Series A-1, Series B-1 and Junior Preferred Stock issued for acquired technology and licenses			9,500
Accrued interest expense converted to Series A Preferred Stock			2
Changes in operating assets and liabilities:			
Prepaid expense and other current assets	(2,052)	27	(2,107)
Other receivables		1,036	
Security deposits	(11)		(170)
Accrued clinical drug supplies and trial costs	2,464	(1,039)	3,477
Accounts payable and accrued expenses	3,040	787	4,431
Deferred rent		(22)	
Net cash used in operating activities	(23,198)	(9,249)	(110,987)
Investing Activities			
Purchase of marketable securities			(4,238)
Maturities of marketable securities			4,250
Purchase of property, equipment	(5)		(201)
Net cash used in investing activities	(5)		(189)
Financing Activities			
Payment of debt issuance costs	(43)	(263)	(421)
Proceeds from issuance of common stock		2	124
Proceeds from initial public offering, net	175,555		175,555
Proceeds from issuance of notes payable, net			210
(Repayment of) proceeds from issuance of venture debt facility, net	(11,900)	7,460	(512)
Proceeds from issuance of preferred stock, net	49,699		130,632
Proceeds from royalty purchase agreement	41,667		41,667
Net cash provided by financing activities	254,978	7,199	347,255
Net change in cash and cash equivalents	231,775	(2,050)	236,079
Cash and cash equivalents			
Beginning of period	4,304	6,396	
End of period	$ 236,079	$ 4,346	$ 236,079

Supplemental disclosures of cash flow information			
Conversion of preferred stock to common stock upon completion of IPO	$ 174,310	$	$ 174,310
Accreted dividends on Series A, Series A-1, Series of B, B-1 and Series C Preferred Stock	$ 5,891	$ 5,288	$ 33,046
Notes payable and accrued interest converted to Series A Preferred Stock	$	$	$ 212

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

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OPHTHOTECH CORPORATION

(A Development Stage Company)

Notes to Unaudited Financial Statements

(tabular dollars and shares in thousands, except per share data)

1. Business

Description of Business and Organization

Ophthotech Corporation (the Company or Ophthotech) was incorporated on January 5, 2007, in Delaware. The Company is a biopharmaceutical company specializing in the development of novel therapeutics to treat diseases of the eye. The Company s operations since inception have been limited to organizing and staffing the Company, acquiring rights to product candidates, business planning, raising capital and developing its product candidates. Accordingly, the Company is considered to be in the development stage as defined by Financial Accounting Standards Board Accounting Standards Codification (ASC) 915, *Development Stage Entities*. The Company operates in one business segment.

Capitalized terms not otherwise defined herein are defined in their respective agreements.

Liquidity

Since the Company s inception, it has incurred significant operating losses. The Company reported a net loss of $30.8 million for the nine months ended September 30, 2013 and $10.7 million for the nine months ended September 30, 2012. As of September 30, 2013, the Company had a deficit accumulated during the development stage of $162.7 million. To date, the Company has not generated any revenues and has financed its operations primarily through private placements of its preferred stock, venture debt borrowings, its royalty purchase and sale agreement with Novo A/S and its initial public offering (IPO), which closed on September 30, 2013. The Company issued and sold an aggregate of 8,740,000 shares of common stock in its IPO at a public offering price of $22.00 per share, including 1,140,000 shares pursuant to the exercise by the underwriters of an over-allotment option. The Company received net proceeds from the IPO of $175.6 million, after deducting underwriting discounts and commissions and other offering expenses. The Company has devoted substantially all of its financial resources and efforts to research and development and expects to continue to incur significant expenses and increasing operating losses over the next several years. The Company s net losses may fluctuate significantly from quarter to quarter and year to year.

To fully execute its business plan, the Company will need to complete certain research and development activities and clinical trials. Further, the Company s product candidates will require regulatory approval prior to commercialization. These activities may span many years and require substantial expenditures to complete and may ultimately be unsuccessful. Any delays in completing these activities could adversely impact the Company. The Company plans to meet its capital requirements primarily through a combination of equity and debt financings, collaborations, strategic alliances and marketing distribution or licensing arrangements and in the longer term, revenue from potential product sales. There can

be no assurance that such funds will be available, or if available, on terms favorable to the Company. The Company faces the normal risks associated with a development stage company, including but not limited to the risk that the Company s research and development activities will not be successfully completed, that adequate patent protection for the Company s technology will not be obtained, that any products developed will not obtain necessary government regulatory approval and that any approved products will not be commercially viable. In addition, the Company operates in an environment of rapid change in technology, substantial competition from pharmaceutical and biotechnology companies and is dependent upon the services of its employees and its consultants. The Company s capital requirements will depend on many factors, including the success of its development and commercialization of its product candidates and whether it pursues the development of additional product candidates. Even if the Company succeeds in developing and commercializing one or more of its product candidates, it may never achieve sufficient sales revenue to achieve or maintain profitability.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited financial information as of September 30, 2013, for the three and nine months ended September 30, 2013 and 2012, and for the period from January 5, 2007 (Inception) to September 30, 2013 has been prepared by the Company, pursuant to the rules and regulations of the Securities and Exchange Commission. Certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. generally accepted

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accounting principles have been condensed or omitted pursuant to such rules and regulations. The December 31, 2012 balance sheet was derived from the Company s audited financial statements. These interim financial statements should be read in conjunction with the 2012 audited annual financial statements and notes thereto in the final prospectus dated September 25, 2013 related to the Company s initial public offering.

In the opinion of management, the unaudited financial information as of September 30, 2013, for the three and nine months ended September 30, 2013 and 2012, and for the period from January 5, 2007 (Inception) to September 30, 2013, reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results or operations and cash flows. The results of operations for the three and nine months ended September 30, 2013 and 2012, are not necessarily indicative of the operating results for the full fiscal year or any future period.

Use of Estimates

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

Cash and Cash Equivalents

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

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, which, at times, exceed federally insured limits. The Company also maintains cash equivalents in money market funds that invest primarily in U.S. Treasury securities. The Company has not recognized any losses from credit risks on such accounts during any of the periods presented. The Company believes it is not exposed to significant credit risk on its cash and cash equivalents.

Foreign Currency Translation

The Company maintains a bank account in a foreign currency. The Company considers the U.S. dollar to be its functional currency. Expenses are translated at the exchange rate on the date the expense is incurred. The effect of exchange rate fluctuations on translating foreign currency assets and liabilities into U.S. dollars is included in the Statements of Operations. Foreign exchange transaction gains and losses are included in the results of operations and are not material in the Company s financial statements.

Financial Instruments

The carrying amounts of the Company s financial instruments, which include cash and cash equivalents, accounts payable and accrued expenses approximate their respective fair value due to their short maturities.

Property and Equipment

Property and equipment, which consist mainly of computers and other equipment, are carried at cost less accumulated depreciation. Depreciation is computed over the estimated useful lives of the respective assets, generally five to seven years, using the straight-line method.

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Research and Development

Research and development expenses consist of costs associated with the development and clinical testing of Fovista, an anti-PDGF aptamer the Company is developing for use in combination with anti-VEGF drugs for treatment of wet age-related macular degeneration, or wet AMD, and the Company s other product candidates. Research and development expenses consist of:

- external research and development expenses incurred under arrangements with third parties, such as contract research organizations, (CROs) and other vendors, contract manufacturing organizations and consultants; and

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

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

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

The Company anticipates that its research and development expenses will increase substantially as compared to prior periods in connection with initiating and conducting its pivotal Phase 3 clinical program for Fovista and if such trials are successful, seeking marketing approval for Fovista.

The Company does not currently utilize a formal time allocation system to capture expenses on a project-by-project basis because it records expenses by functional department. Accordingly, the Company does not allocate expenses to individual projects or product candidates, although it does allocate some portion of its research and development expenses by functional area and by compound.

Income Taxes

The Company utilizes the liability method of accounting for deferred income taxes, as set forth in ASC 740-10, *Income Taxes-Overall.* Under this method, deferred tax liabilities and assets are recognized for the expected future tax consequences of temporary differences between the carrying amounts and the tax basis of assets and liabilities. A valuation allowance is established against net deferred tax assets because, based on the weight of available evidence, it is more likely than not that some or all of the net deferred tax assets will not be realized. The Company maintains a full valuation allowance on its deferred tax assets. Accordingly, the Company has not recorded a benefit or provision for income taxes other than for the sale of a portion of its unused New Jersey State operating loss carryforwards through a program sponsored by the State

of New Jersey and the New Jersey Economic Development Authority in 2011. The Company s U.S. federal net operating losses have occurred since inception and as such, tax years subject to potential tax examination could apply from that date because carrying-back net operating loss opens the relevant year to audit.

Share-Based Compensation

The Company follows the provisions of the ASC 718, *Compensation Stock Compensation* which requires the measurement and recognition of compensation expense for all share-based payment awards made to employees and non-employee directors, including employee stock options. Share-based compensation expense is based on the grant date fair value estimated in accordance with the provisions of ASC 718 and is generally recognized as an expense over the requisite service period.

For stock options granted as consideration for services rendered by non-employees, the Company recognizes expense in accordance with the requirements of ASC 505-50, *Equity Based Payments to Non-Employees*. Non-employee option grants that do not vest immediately upon grant are recorded as an expense over the vesting period of the underlying stock options. At the end of each financial reporting period prior to vesting, the value of these options, as calculated using the Black-Scholes option-pricing model, will be re-measured using the fair value of the Company s common stock and the non-cash expense recognized during the period will be adjusted accordingly. Since the fair value of options granted to

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non-employees is subject to change in the future, the amount of the future expense will include fair value re-measurements until the stock options are fully vested.

Prior to the Company s initial public offering, the Company determined the estimated fair value of the common stock based on a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector and the prices at which the Company sold shares of its common stock.

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 was determined using the simplified method which is the midpoint between the vesting date and the end of the contractual term. The Company believes that it does not have sufficient reliable exercise data in order to justify the use of a method other than the simplified method of estimating the expected exercise term of employee stock option grants. The Company has paid no dividends to stockholders. The risk-free interest rate is based on the zero-coupon U.S. Treasury yield at the date of grant for a term equivalent to the expected term of the option.

Share-based compensation expense includes stock options granted to employees and non-employees and has been reported in the Company s statements of operations as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2013	2012	2013	2012
Research and development	$ 752	$ 96	$ 1,052	$ 182
General and administrative	395	54	555	101
Total	$ 1,147	$ 150	$ 1,607	$ 283

The Company had no shares of unvested restricted common stock granted to employees at September 30, 2013 or at December 31, 2012, respectively.

Reclassification

Certain amounts in prior periods have been reclassified to conform to the current year presentation. Such reclassifications did not have a material effect on the Company s financial condition or results of operations as previously reported.

JOBS Act

As an emerging growth company under the Jumpstart Our Business Startups Act of 2012, the Company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of

certain accounting standards until those standards would otherwise apply to private companies. The Company has elected to delay the adoption of such new or revised accounting standards. As a result of this election, the Company s financial statements may not be comparable to the financial statements of other public companies.

3. Capitalization

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

On September 30, 2013, the Company closed its initial public offering of 8,740,000 shares of common stock at a price of $22.00 per share. The net proceeds to the Company were $175.6 million, after deducting underwriters commissions and other offering expenses.In connection with the closing of the IPO, all of the Company s shares of redeemable convertible preferred stock outstanding at the time of the offering were automatically converted into 21,038,477 shares of common stock.

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In August 2013, the Company s Board of Directors and stockholders approved a restated certificate of incorporation which became effective following the closing of the Company s IPO on September 30, 2013. The restated certificate of incorporation increased the number of authorized shares of common stock to 200,000,000 and decreased the number of authorized shares of preferred stock to 5,000,000.

4. Net Loss Per Common Share

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

	Three months ended September 30,		Nine months ended September 30,	
	2013	2012	2013	2012
Basic and diluted net loss per common share calculation:				
Net loss	$ (16,132)	$ (4,153)	$ (30,757)	$ (10,731)
Accretion of preferred stock dividends	(2,292)	(1,775)	(5,891)	(5,288)
Net loss attributable to common shareholders	$ (18,424)	$ (5,928)	$ (36,648)	$ (16,019)
Weighted average common shares outstanding	1,795	1,455	1,579	1,447
Net loss per share of common stock - basic and diluted	$ (10.26)	$ (4.07)	$ (23.21)	$ (11.07)

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

	Three months ended September 30,		Nine months ended September 30,	
	2013	2012	2013	2012
Redeemable convertible preferred stock		16,513		16,513
Stock options outstanding	2,620	1,301	2,620	1,301
Warrants	101	78	101	78
Total	2,721	17,892	2,721	17,892

5. Fair Value Measurements

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

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

- Level 1 inputs to the valuation methodology are quoted prices (unadjusted) for an identical asset or liability in an active market.

- Level 2 inputs to the valuation methodology include quoted prices for a similar asset or liability in an active market or model-derived valuations in which all significant inputs are observable for substantially the full term of the asset or liability.

- Level 3 inputs to the valuation methodology are unobservable and significant to the fair value measurement of the asset or liability.

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The following table presents, for each of the fair value hierarchy levels required under ASC 820, the Company s assets and liabilities that are measured at fair value on a recurring basis as of September 30, 2013:

	Fair Value Measurement Using		
	Quoted prices in active markets for identical assets (Level 1)	**Significant other observable inputs (Level 2)**	**Significant unobservable inputs (Level 3)**
Assets			
Investments in U.S. Treasury money market funds*	$ 234,843	$	$
Liabilities			
Series A Warrant Liability	$	$	$
Series B Warrant Liabilty	$	$	$

* Investments in U.S. Treasury money market funds are reflected in cash and cash equivalents in the accompanying Balance Sheets.

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, 2012:

	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*	$ 524	$	$
Liabilities			
Series A Warrant Liability	$	$	$ 523
Series B Warrant Liabilty	$	$	$ 443

* Investments in money markets funds are reflected in cash and cash equivalents in the accompanying Balance Sheets.

Level 3 Valuation

The warrant liability was recorded in its own line item on the Company s Balance Sheets. The warrant liability was marked-to-market each reporting period with the change in fair value recorded to other loss in the Statement of Operations. The fair value of the warrant liability was estimated using a hybrid method between a probability-weighted expected return method, or PWERM, model and an option pricing model, which includes variables such as the expected volatility based on guideline public companies, the preferred stock value, and the estimated time to a liquidity event.

The significant assumptions used in preparing the option pricing model for valuing the Company s warrants for the Series A preferred shares as of December 31, 2012, include (i) volatility (47.2% 85.3%), (ii) risk free interest rate (0.05% 0.62%), (iii) strike price ($0.01), (iv) fair value of Series A preferred shares ($1.22 $4.34), (v) expected life (0.25 years to

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4.5 years) and (vi) expected outcome probability weighting of three outcome scenarios: merger (65%); dissolution (20%) and an initial public offering (15%).

The significant assumptions used in preparing the option pricing model for valuing the Company s warrants for the Series B preferred shares as of December 31, 2012, include (i) volatility (47.2% 80.1%), (ii) risk free interest rate (0.05% 1.68%), (iii) strike prices ($1.00 $2.50), (iv) fair value of Series B preferred shares ($1.18 $4.22), (v) expected life (0.25 years to 9.5 years) and (vi) expected outcome probability weighting of three outcome scenarios: merger (65%); dissolution (20%) and an initial public offering (15%).

The table presented below is a summary of changes in the fair value of the Company s Level 3 valuation for the Series A and Series B warrant liabilities for the period ended September 30, 2013:

	Level 3	
	Series A Warrant Liability	**Series B Warrant Liability**
Balance at December 31, 2012	523	443
Warrants issued in connection with venture debt facility		32
Change in fair value of warrant liability	379	852
Conversion of warrant liability to equity	(902)	(1,327)
Balance at September 30, 2013	$	$

Upon completion of the Company s IPO on September 30, 2013, the underlying preferred stock was converted to common stock and the preferred stock warrants became exercisable for common stock. The fair value of the warrant liability was re-measured immediately prior to the completion of the Company s IPO, and the fair value of the warrant liability at that time was reclassified to additional paid-in capital. Based on the initial public offering price of $22.00 per share, the fair value of the warrant liability that was reclassified to additional paid-in capital was $2.2 million. The Company recorded a related charge of approximately $1.0 million and $1.2 million as other loss in its results of operations for the three and nine months ended September 30, 2013, respectively.

6. Notes Payable

In June 2012, December 2012 and March 2013, the Company issued secured promissory notes (the Notes) in the amount of $7.5 million and $4.0 million and $1.5 million, respectively, to the same lender. The Notes bore interest on the outstanding principal amount thereof from the Closing Date until paid in full at a rate per annum equal to the sum of (i) the greater of (A) the LIBOR Rate in effect for the applicable Interest Period and (B) 3.0%, plus (ii) the LIBOR Rate Margin adjusted on the first day of each Interest Period and fixed for the duration of each such Interest Period.

As of December 31, 2012, the Company classified the debt with the lender as a current liability since the Company intended to pay down the balance in its entirety within twelve months. The Company repaid in full the outstanding principal, interest and related prepayment fees in May 2013. The repayment of the Notes resulted in a loss on extinguishment of debt in the amount of $1.1 million for the nine months ended September 30, 2013. In addition, the Company made payments of $0.8 million which, in accordance with the Notes, were required upon the earlier of the maturity date or the prepayment date of the Notes. These payments were recorded as interest expense for the nine months ended

September 30, 2013.

7. Stock Option and Compensation Plans

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

During the nine months ended September, 30, 2013, the Company s Board of Directors and stockholders increased the number of shares authorized under the 2007 Plan by 1,878,343 shares.

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During the nine months ended September 30, 2013, the Company s board of directors also adopted and the Company s stockholders also approved the 2013 stock incentive plan (the 2013 Plan), which became effective immediately prior to the closing of the Company s initial public offering. The 2013 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, and other stock-based awards. Upon effectiveness of the 2013 Plan, the number of shares of the Company s common stock that were reserved for issuance under the 2013 Plan was the sum of (1) such number of shares (up to approximately 3,359,641 shares) as is equal to the sum of 739,317 shares (the number of shares of the common stock then available for issuance under the 2007 Plan), and such number of shares of the Company s common stock that are subject to outstanding awards under the 2007 Plan that expire, terminate or are otherwise surrendered, canceled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right plus (2) an annual increase, to be added the first day of each fiscal year, beginning with the fiscal year ending December 31, 2014 and continuing until, and including, the fiscal year ending December 31, 2023, equal to the lowest of 2,542,372 shares of the Company s common stock, 4% of the number of shares of the Company s common stock outstanding on the first day of the fiscal year and an amount determined by our 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.

As of September 30, 2013, the Company had approximately 2,620,000 stock options outstanding under the 2007 Plan and approximately 739,000 shares available for grant under the 2013 Plan.

The Company recognized share-based compensation of approximately $1.1 million for the three months ended September 30, 2013 and approximately $0.2 million during the three months ended September 30, 2012. The Company recognized share-based compensation of approximately $1.6 million for the nine months ended September 30, 2013 and approximately $0.3 million during the nine months ended September 30, 2012. As of September 30, 2013, there was approximately $11.6 million of total unrecognized share-based compensation expense that is expected to be recognized over a weighted average period of approximately 3.4 years.

The intrinsic value of the Company s approximately 1,091,000 vested options as of September 30, 2013 was $30.8 million, based on a per share price of $29.71, the closing price of the Company s stock at September 30, 2013, and a weighted average exercise price of $1.36 per share. The intrinsic value of the Company s approximately 1,529,000 unvested options as of September 30, 2013 was $30.5 million, based on a per share price of $29.71, the closing price of the Company s stock at September 30, 2013, and a weighted average exercise price of $9.76 per share.

8. Royalty Agreement and Series C Agreement

In May 2013, the Company entered into a Purchase and Sale Agreement (the Purchase and Sale Agreement) with Novo A/S, providing for the Company to sell, and Novo A/S to purchase, the right, title, and interest in a portion of the revenues from the sale of (a) Fovista, (b) Fovista-Related Products, and (c) Other Products (as defined in the Purchase and Sale Agreement), calculated as low to mid-single digit percentages of net sales.

The Purchase and Sale Agreement provides for up to three separate purchases for a purchase price of $41.7 million each, at a first, second and third closing, for an aggregate purchase price of $125.0 million. In each purchase, Novo A/S acquires rights to a low single digit percentage of net sales. If all royalty interests under the Purchase and Sale Agreement are purchased, Novo A/S will have a right to receive royalties on net sales at a mid-single digit percentage.

In May 2013, the Company received cash proceeds of $41.7 million for the royalty entitlement related to the first closing on the date of the Purchase and Sale Agreement. Receipt of cash proceeds for the second and third purchases is contingent upon certain triggers and conditions detailed in the Purchase and Sale Agreement, none of which have occurred prior to this filing.

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

Under the terms of the Purchase and Sale 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 Purchase and Sale Agreement, have the right or obligation to prepay Novo A/S in connection with a change of control of the Company or otherwise.

The proceeds from the first financing tranche under the Purchase and Sale Agreement were recorded as a liability on the Company s Balance Sheet as of September 30, 2013, in accordance with ASC 730. Because there is a significant

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related party relationship between the Company and Novo A/S, the Company is treating its obligation to make royalty payments under the Purchase and Sale Agreement as an implicit obligation to repay the funds advanced by Novo A/S. As the Company makes royalty payments in accordance with the Purchase and Sale Agreement, it will reduce the liability balance. At the time that such royalty payments become probable and estimable, and if such amounts exceed the liability balance, the Company will impute interest accordingly on a prospective basis based on such estimates, which would result in a corresponding increase in the liability balance.

The Purchase and Sale 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.

Also in May 2013, the Company entered into a Series C Preferred Stock Purchase Agreement (the Series C Agreement) with certain of its existing investors for the sale and issuance, upon the satisfaction of certain conditions, of an aggregate of 20,000,000 shares of the Company s Series C Preferred Stock at a price of $2.50 ($14.75 on a post-reverse stock split basis) per share. The Company issued 6,666,667 shares of Series C Preferred Stock at $2.50 ($14.75 on a post-reverse stock split basis) per share in a closing that occurred in May 2013, simultaneous with entry into the Series C Agreement. In August 2013, the Company amended the Series C Agreement to provide for the acceleration of the sale and issuance of the remaining 13,333,333 shares issuable thereunder, the purchase and sale of which closed on August 7, 2013 at $2.50 ($14.75 on a post-reverse stock split basis) per share for aggregate proceeds of $33.3 million. There are no further rights or obligations for the issuance of Series C Preferred Stock under the Series C Agreement.

As the Series C Agreement was entered into in conjunction with the Purchase and Sale Agreement, the Company s management considered whether the consideration received for the issuance of Series C Preferred Stock or the consideration received for the sale of the royalty entitlement at the first closing under the Purchase and Sale Agreement should be allocated in the Company s financial statements in a manner different than the prices stated in the respective agreements. The Company s management, with the assistance of an outside valuation specialist, determined that the $2.50 ($14.75 on a post-reverse stock split basis) per share price approximated the fair value of a share of Series C Preferred Stock, and therefore concluded that the consideration received under the agreements should be allocated in accordance with the terms of the respective agreements. In connection with entering into the Series C Agreement, the minimum public offering price per share in an underwritten public offering of common stock required for the automatic conversion of outstanding shares of Series A Preferred Stock, Series A-1 Preferred Stock, Series B Preferred Stock, Series B-1 Preferred Stock, Series C Preferred Stock and Junior Series A Preferred Stock pursuant to the Company s certificate of incorporation was adjusted to $14.75 per share (subject to further adjustment as a result of any stock dividend, stock split, combination or similar recapitalization of the common stock).

The Company has determined that in accordance with ASC 470-20-20, at the time of the initial closing under the Series C Agreement on May 23, 2013, there was a firm commitment from the Series C Preferred Stock investors with respect to the significant terms of the financing, including the quantity of shares to be issued, the fixed price of the shares and the timing of the transaction. In addition, the Company has concluded that the Series C Agreement and the Company s certificate of incorporation includes a disincentive feature for non-performance that was sufficiently large enough to make investor performance at subsequent closings probable. As such, the Company s measurement of any beneficial conversion feature occurred at the time of the initial closing. Based on a $10.03 per share valuation of the Company s common stock as of the date of the initial closing of the sale of the Series C Preferred Stock, as well as the fact that the Series C Preferred Stock featured a common stock conversion price of $14.75 per share (implying a one-to-one conversion into shares of common stock), the Company determined that there was no beneficial conversion feature associated with the issuance of its Series C Preferred Stock.

The proceeds received from Novo A/S under the Purchase and Sale Agreement will be reported as revenue for income tax purposes. Notwithstanding the Company s receipt of $41.7 million in proceeds under the Purchase and Sale Agreement in May 2013, the Company has

forecasted a tax loss for the 2013 tax year. Based upon the Company s cumulative history of losses and expected future losses, the Company recorded a full valuation allowance against all net federal and state deferred tax assets.

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

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and related accompanying notes appearing elsewhere in this Quarterly Report on Form 10-Q. 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 financings, which includes forward looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the Risk Factors section of this Quarterly Report on Form 10-Q, our actual results could 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 biopharmaceutical company specializing in the development of novel therapeutics to treat diseases of the eye. Our most advanced product candidate is Fovista, which we are developing for use in combination with anti-VEGF drugs that represent the current standard of care for the treatment of wet age-related macular degeneration, or wet AMD. We have completed one Phase 1 and one Phase 2b clinical trial of Fovista administered in combination with the anti-VEGF drug Lucentis.

Our Phase 3 clinical program for Fovista consists of three separate Phase 3 clinical trials to evaluate the safety and efficacy of Fovista administered in combination with anti-VEGF drugs for the treatment of wet AMD compared to anti-VEGF monotherapy. Two of these trials will evaluate Fovista in combination with Lucentis and the other will evaluate Fovista in combination with each of Eylea or Avastin. The three trials are planned to enroll a total of approximately 1,866 patients in approximately 225 centers internationally.

In July 2013, we submitted protocols for the three trials in our Phase 3 clinical program to the U.S. Food and Drug Administration, or FDA. In August 2013, we initiated enrollment in the United States in the two trials evaluating Fovista administered in combination with Lucentis. The third trial in this Phase 3 clinical program is targeted to initiate enrollment in the United States in the first quarter of 2014.

Outside the United States, we have made regulatory submissions in selected countries to initiate the two Phase 3 clinical trials of Fovista administered in combination with Lucentis and have begun to obtain approvals to proceed. In the European Union, in addition to filing in selected countries with regulatory agencies referred to as National Competent Authorities, which are responsible for approving clinical trial applications, we are also continuing interactions with the European Medicine Agency s Committee for Medicinal Products for Human Use, or CHMP, which is the committee responsible for preparing opinions on questions concerning medicines for human use.

The CHMP recently provided scientific advice on our proposed Phase 3 clinical program for Fovista and our plan to seek regulatory approval for Fovista in which the CHMP advised us that the planned primary endpoint for each of the Fovista Phase 3 clinical trials, mean change from baseline in best corrected visual acuity, was acceptable. In addition, the CHMP confirmed that carcinogenicity studies are not needed. The CHMP also advised us that we should justify our proposal to initiate, at the Phase 3 clinical trial stage, previously untested combinations of Fovista with Avastin or Eylea, and that we should consider conducting toxicity studies with Fovista administered in combination with Avastin or Eylea prior to initiating our corresponding Phase 3 clinical trial. In addition, the CHMP informed us that the final label for Fovista, if it receives marketing approval, may be required to specify the licensed anti-VEGF agents that were studied in combination with Fovista, given that Avastin is not licensed for intravitreal use, rather than a broad label specifying Fovista for use in combination with any anti-VEGF agent. The CHMP further advised us that there will be a requirement for additional data to bridge the results from our Phase 3 clinical trials of Fovista administered

in combination with Lucentis to the less frequent dosing regimens of Lucentis and Eylea approved in the European Union. We intend to have further scientific discussion with the CHMP on these issues and do not currently expect that additional toxicity studies will be required. We plan to adjust the dosing schedule in our Phase 3 clinical program for Fovista administered in combination with Eylea so that no bridging study would be needed for this combination. Although we are still considering the possible design of a bridging study for Fovista administered in combination with Lucentis and discussing with the CHMP the need to conduct any such study, we expect that, if we determine to conduct such a study, it would not have a material impact on our plan, based on our estimates regarding patient enrollment in our Phase 3 clinical program, to have initial, top-line data from our Phase 3 clinical program for Fovista available in 2016. We also anticipate that our existing cash and cash equivalents and potential funding under our royalty purchase and sale agreement, or royalty agreement, with Novo A/S will be sufficient to enable us to fund our operating expenses and capital expenditure requirements, including any additional costs not previously contemplated for a possible bridging study for Fovista administered in combination with Lucentis, through at least the second quarter of 2016.

If the results of this Phase 3 clinical program are favorable, we plan to submit applications for marketing approval for Fovista in both the United States and the European Union before the end of 2016. We also are evaluating the conduct of small, exploratory clinical trials to assess the potential therapeutic benefit of Fovista in other ophthalmic conditions and further clinical development of our product candidate ARC1905 for the treatment of wet AMD.

We were incorporated and commenced active operations in early 2007. Our operations to date have been limited to organizing and staffing our company, acquiring rights to product candidates, business planning, raising capital and developing Fovista and our other product candidates. We acquired our rights to Fovista from (OSI) Eyetech, Inc., or Eyetech, in July 2007. The acquisition included an assignment of license rights and obligations under an agreement with Archemix Corp. We have licensed rights to our product candidate ARC1905 from Archemix Corp. Since inception, we have incurred significant operating losses. Our net loss was $30.8 million for the nine months ended September 30, 2013 and $10.7 million for the nine months ended September 30, 2012. As of September 30, 2013, we had a deficit accumulated during the development stage of $162.7 million. To date, we have not generated any revenues and have financed our operations primarily through private placements of our preferred stock, venture debt borrowings, our royalty agreement with Novo A/S and our initial public offering, which we closed in September 2013. We issued and sold an aggregate of 8,740,000 shares of common stock in our initial public offering at a public offering price of $22.00 per share, including 1,140,000 shares pursuant to the exercise by the underwriters of an over-allotment option. We received net proceeds from the initial public offering of $175.6 million, after deducting underwriting discounts and commissions and other offering expenses payable by us. Our ability to become and remain profitable depends on our ability to generate revenue. We do not expect to generate significant revenue unless, and until, we obtain marketing approval for, and commercialize, Fovista.

We expect our expenses to increase substantially as compared to prior periods in connection with our ongoing activities, particularly as we continue the development of and seek marketing approval for Fovista and, possibly, other product candidates. In addition, if we obtain marketing approval for Fovista or any other product candidate that we develop, we expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. Furthermore, following our initial public offering, we expect to incur additional costs associated with operating as a public company, hiring additional personnel and expanding our facilities. We expect that these costs will include significant legal, accounting, investor relations and other expenses that we did not incur as a private company. Moreover, additional rules and regulations applicable to public companies will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. We currently estimate that we will incur incremental annual costs, including costs for additional personnel, of approximately $2.0 million associated with operating as a public company, although it is possible that our actual incremental costs will be higher than we currently estimate. The increased costs will increase our net loss. We will 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.

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Financial Operations Overview

Revenue

To date, we have not generated any revenues. Our ability to generate product revenues, which we do not expect will occur before 2017, at the earliest, will depend heavily on our obtaining marketing approval for and commercializing Fovista.

Research and Development Expenses

Research and development expenses consist of costs associated with the development and clinical testing of Fovista and our other product candidates. Our research and development expenses consist of:

- external research and development expenses incurred under arrangements with third parties, such as contract research organizations, or CROs, and other vendors, contract manufacturing organizations and consultants; and

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

All research and development costs are charged to operations as incurred in accordance with 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. From inception through September 30, 2013, we have incurred approximately $92.7 million of total research and development expenses.

To date, the large majority of our research and development work has been related to Fovista, ARC1905 and a product candidate, volociximab, that we were previously developing for the treatment of wet AMD. We licensed rights to volociximab in January 2008 and then terminated the license agreement in May 2012 to focus on the development of Fovista. We anticipate that our research and development expenses will increase substantially as compared to prior periods in connection with initiating and conducting our pivotal Phase 3 clinical program for Fovista and if such trials are successful, seeking marketing approval for Fovista.

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

The following table summarizes our research and development expenses for the three and nine months ended September 30, 2013 and 2012:

	Three months ended September 30,		Nine months ended September 30,	
	2013	2012	2013	2012
	(in thousands)			
Fovista	$ 9,219	$ 854	$ 14,073	$ 2,530
ARC1905	6	8	13	31
Volociximab	1	4	7	19
Personnel related	1,334	678	2,971	2,068
Share-based compensation	522	49	753	124
Other	19	2	19	22
	$ 11,101	$ 1,595	$ 17,836	$ 4,794

We recorded research and development expenses from inception to September 30, 2013 of approximately $38.4 million related to Fovista, approximately $11.1 million related to ARC1905 and approximately $5.6 million related to volociximab.

As of September 30, 2013, we estimate that we will incur costs, including clinical development related employee expenses and external research and development expenses, of approximately $166.0 million to obtain initial, top-line data from our Phase 3 clinical program for Fovista. We expect these data to be available in 2016. We also estimate that additional funds of approximately $46.0 million will be required to fund our other development programs and for general corporate purposes and working capital until we obtain initial, top-line data from our Phase 3 program. Costs related to our current Phase 3 clinical program for Fovista could exceed these estimates if we experience delays in our clinical trials, including the timing of our patient enrollment, the availability of drug supply for our clinical trials or for other reasons. These costs will also increase if we decide to expand the scope of our Fovista clinical program or our other development programs, or increase other corporate activities and staffing.

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Our Phase 3 clinical program for Fovista is expected to continue through at least 2017, and substantial expenditures to complete the Phase 3 clinical program will be required after the receipt of initial, top-line data. At this time, we cannot reasonably estimate the remaining costs necessary to complete the Phase 3 clinical program for Fovista, complete process development and manufacturing scale-up activities associated with Fovista and seek marketing approval after we obtain initial, top-line data, or the nature, timing or costs of the efforts necessary to complete the development of any other product candidate.

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

- the scope, rate of progress and expense of our research and development activities;
- the potential benefits of our product candidates over other therapies;
- our ability to market, commercialize and achieve market acceptance for any of our product candidates;
- clinical trial results;
- the terms and timing of regulatory approvals; and
- the expense of filing, prosecuting, defending and enforcing patent claims and other intellectual property rights.

A change in the outcome of any of these variables with respect to the development of Fovista or any other product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if regulatory authorities were to require us to conduct clinical trials beyond those which we currently anticipate will be required for the completion of clinical development of Fovista or any other product candidate or if we experience significant delays in enrollment in any clinical trials, we could be required to expend significant additional financial resources and time on the completion of the clinical development.

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, finance and business development functions. Other general and administrative expenses include facility costs and professional

fees for legal, patent, consulting and accounting services.

We anticipate that our general and administrative expenses will increase in future periods to support increases in our research and development and commercialization activities and as a result of increased headcount, including management personnel to support our clinical and manufacturing activities, expanded infrastructure, increased legal, compliance, accounting and investor and public relations expenses associated with being a public company and increased insurance premiums, among other factors.

Change in Fair Value of Warrant Liability

In connection with our series A financing and our venture debt financing, we issued warrants for the purchase of shares of our series A preferred stock and series B preferred stock. We determined that these warrants were financial instruments that could have required a transfer of assets because of the redemption features of the underlying preferred stock. We classified these warrants as liabilities that were re-measured to fair value at each balance sheet date, and we recorded the changes in the fair value of the warrant liability as other loss. Upon completion of our initial public offering, or IPO, the underlying preferred stock was converted to common stock and the preferred stock warrants became exercisable for common stock. We re-measured the fair value of the warrant liability immediately prior to the completion of our IPO, and the fair value of the warrant liability at that time was reclassified to additional paid-in capital. Based on the initial public offering price of $22.00 per share, the fair value of the warrant liability that was reclassified to additional paid-in capital was $2.2 million. We recorded a related charge of approximately $1.0 million and $1.2 million as other loss in our results of operations for the three

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and nine months ended September 30, 2013, respectively. The warrants were reclassified to stockholders equity upon the closing of our IPO.

Interest Income

Our cash and cash equivalents are invested primarily in money market accounts, which generate a small amount of interest income. We expect to continue that investment philosophy as we obtain more financing proceeds.

Critical Accounting Policies and Significant Judgments and Estimates

Our management s discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued expenses and share-based compensation described in greater detail below. We base our estimates on our limited historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

Our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this Quarterly Report on Form 10-Q. However, we believe that the following accounting policies are the most critical to aid investors 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 fees paid or payable to CROs and other vendors in connection with research and development activities for which we have not yet been invoiced.

We base our expenses related to CROs on our estimates of the services received and efforts expended pursuant to quotes and contracts with CROs that conduct research and development on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the research and development expense. In accruing service fees, we estimate the time

period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepayment 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.

Royalty Purchase Liability

The proceeds from the first financing tranche under our royalty agreement with Novo A/S have been recorded as a liability on our balance sheet in accordance with Financial Accounting Standards Board Accounting Standards Codification, or ASC, Topic 730. Because there is a significant related party relationship between us and Novo A/S, we are treating our obligation to make royalty payments under the royalty 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 Sheets. As we make royalty payments to Novo A/S in accordance with the royalty agreement, we will reduce the liability balance. At the time that such royalty payments become probable and estimable, and if such amounts exceed the liability

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balance, we will impute interest accordingly on a prospective basis based on such estimates, which would result in a corresponding increase in the liability balance.

Share-Based Compensation

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

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

We use the Black-Scholes option pricing model to value our stock option awards. Use of this valuation methodology requires that we make assumptions as to the volatility of our common stock, the expected term of our stock options, the risk free interest rate for a period that approximates the expected term of our stock options and our expected dividend yield. As a new public company, we do not have sufficient history to estimate the volatility of our common stock price or the expected life of the options. We calculate expected volatility based on reported data for similar publicly traded companies for which historical information is available and will continue to do so until the historical volatility of our common stock is sufficient to measure expected volatility for future option grants.

We use the simplified method as prescribed by the Securities and Exchange Commission Staff Accounting Bulletin No. 107, *Share-Based Payment*, to calculate the expected term of stock option grants to employees as we do not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term of stock options granted to employees. 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 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. The weighted-average assumptions used to estimate the fair value of stock options using the Black-Scholes option pricing model were as follows for the three and nine months ended September 30, 2013 and 2012:

	Three months ended September 30,		Nine months ended September 30,	
	2013	2012*	2013	2012
Expected common stock price volatility	82%		82%	81%
Risk-free interest rate	1.66%-2.69%		0.89%-2.69%	1.16%-1.51%
Expected term of options (years)	6.5		6.2	6.3
Expected annual dividend per share	$	$	$	$

* No stock options were granted during three months ended September 30, 2012

We are also required to estimate forfeitures at the time of grant, and revise those estimates in subsequent periods if actual forfeitures differ from our estimates. We use historical data to estimate pre-vesting option forfeitures and record share-based compensation expense only for those awards that are expected to vest. To the extent that actual forfeitures differ from our estimates, the difference is recorded as a cumulative adjustment in the period the estimates were revised. Through September 30, 2013, actual forfeitures have not been material.

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For the three and nine months ended September 30, 2013 and 2012, we allocated share-based compensation as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2013	2012	2013	2012
	(in thousands)			
Research and development	$ 752	$ 96	$ 1,052	$ 182
General and administrative	395	54	555	101
Total	$ 1,147	$ 150	$ 1,607	$ 283

Pre-IPO Valuations

Prior to our initial public offering, our valuations utilized the probability-weighted expected return method, or PWERM, to allocate the enterprise value to the common stock. Under this method, the per share fair market value of the common stock was estimated based upon the probability-weighted present value of expected future equity values for our common stock, under various possible future liquidity event scenarios, in light of the rights and preferences of each class of stock, discounted for a lack of marketability. The future liquidity event scenarios were primarily: (1) IPO; (2) a strategic merger or sale of our company; (3) a sale of our company at a value below the cumulative liquidation preference of the preferred stockholders; or (4) a dissolution of the company. The timing of the future liquidity event scenarios was determined based primarily on input from our board of directors and management. The future values of our common stock in the IPO scenarios and the strategic merger or sale scenarios were estimated by application of the market approach based on certain key assumptions, including the following:

- for our June 2010 valuation, our expected pre-money IPO valuation to the investors on their invested capital;

- for our December 2011, November 2012, May 2013 and August 2013 valuations, recently completed IPOs of similar stage biotechnology companies;

- estimated third-party trade sale values based on a range of returns to the investors on their invested capital; and

- expected dates for a future exit or liquidity event based on key events and company timelines.

JOBS Act

As an emerging growth company under the Jumpstart Our Business Startups Act of 2012, we can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain

accounting standards until those standards would otherwise apply to private companies. We are electing to delay our adoption of such new or revised accounting standards. As a result of this election, our financial statements may not be comparable to the financial statements of other public companies.

Results of Operations

Comparison of Three Month Periods Ended September 30, 2013 and 2012

Revenue

We did not recognize any revenue for the three months ended September 30, 2013 or for the three months ended September 30, 2012.

Research and Development Expenses

Our research and development expenses were $11.1 million for the three months ended September 30, 2013, an increase of $9.5 million compared to $1.6 million for the three months ended September 30, 2012. The increase was primarily due to milestone payments and clinical trial startup costs as we continued to progress the Fovista Phase 3 clinical program.

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General and Administrative Expenses

Our general and administrative expenses for the three months ended September 30, 2013 were $4.2 million, an increase of $1.9 million compared to $2.3 million for the three months ended September 30, 2012. The increase was primarily due to an increase in intellectual property related expenses, professional services and consulting fees, and personnel costs, including additional management and corporate staffing to support our public company infrastructure.

Interest Expense

Interest expense for the three months ended September 30, 2013 was de minimis compared to $0.2 million for the three months ended September 30, 2012. The amounts recorded in the three months ended September 30, 2012 were related to interest associated with our venture debt facility that we entered into in June 2012. The debt facility was paid off in May 2013 and as such, there was no corresponding interest expense during the three months ended September 30, 2013.

Other Loss

Other loss was $1.0 million for the three months ended September 30, 2013 compared to $0.1 million for the three months ended September 30, 2012. The $0.9 million increase was due to the change in fair value of the preferred stock warrants.

Comparison of Nine Month Periods Ended September 30, 2013 and 2012

Revenue

We did not recognize any revenue for the nine months ended September 30, 2013 or for the nine months ended September 30, 2012.

Research and Development Expenses

Our research and development expenses were $17.8 million for the nine months ended September 30, 2013, an increase of $13.0 million compared to $4.8 million for the nine months ended September 30, 2012. The increase was primarily due to milestone payments, manufacturing activity and clinical trial startup costs as we continued to progress the Fovista Phase 3 clinical program.

General and Administrative Expenses

Our general and administrative expenses for the nine months ended September 30, 2013 were $9.1 million, an increase of $3.8 million compared to $5.3 million for the nine months ended September 30, 2012. The increase was primarily due to an increase in intellectual property related expenses, professional services and consulting fees and personnel costs, including additional management and corporate staffing to support our public company infrastructure.

Interest Expense

Interest expense for the nine months ended September 30, 2013 was $1.5 million compared to $0.3 million for the nine months ended September 30, 2012. The amounts in both 2013 and 2012 were related to interest associated with our venture debt facility that we entered into in June 2012 and paid off in May 2013. The related interest expense for the nine months ended September 30, 2013 included a payment of $0.8 million that was required upon the earlier of the maturity date or the date of repayment of the venture debt facility.

Loss on Extinguishment of Debt

In May 2013, we repaid the outstanding balance on our venture debt facility. The associated $1.1 million loss on extinguishment of debt represents the related prepayment penalties and an expense for deferred costs and unamortized debt discount, in each case, related to the venture debt facility.

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Other Loss

Other loss was $1.2 million for the nine months ended September 30, 2013 compared to $0.3 million for the nine months ended September 30, 2012. The $0.9 million increase was due to the change in fair value of the preferred stock warrants.

Liquidity and Capital Resources

Sources of Liquidity

To date, we have not generated any revenues and have financed our operations primarily through private placements of our preferred stock, venture debt borrowings, our royalty agreement with Novo A/S and our initial public offering, which we closed on September 30, 2013. We issued and sold an aggregate of 8,740,000 shares of common stock in our initial public offering at a public offering price of $22.00 per share, including 1,140,000 shares pursuant to the exercise by the underwriters of an over-allotment option. We received net proceeds from the initial public offering of $175.6 million, after deducting underwriting discounts and commissions and other offering expenses payable by us. Our royalty agreement, which is described in more detail below, provides for financing of up to $125.0 million in the aggregate in return for the sale to Novo A/S of royalty interests in worldwide sales of Fovista. We received $41.7 million of this royalty financing in May 2013. Our receipt of additional amounts is subject to enrollment of specified numbers of patients in our Phase 3 clinical trials of Fovista and our satisfying additional closing conditions and other obligations. In May 2013, we issued and sold an aggregate of 6,666,667 shares of our series C preferred stock at a price per share of $2.50, for an aggregate purchase price of $16.7 million. In August 2013, we issued and sold an aggregate of 13,333,333 additional shares of our series C preferred stock to the same purchasers at a price per share of $2.50, for an aggregate purchase price of $33.3 million.

Cash Flows

As of September 30, 2013, we had cash and cash equivalents totaling $236.1 million and no debt. We primarily invest our cash and cash equivalents in U.S. Treasury securities and money market funds that invest in U.S. Treasury securities.

The following table shows a summary of our cash flows for the nine months ended September 30, 2013 and 2012:

	Nine months ended September 30,	
	2013	2012
	(in thousands)	
Net cash (used in) provided by:		
Operating Activities	$ (23,198)	$ (9,249)
Investing Activities	(5)	
Financing Activities	254,978	7,199
Net change in cash and cash equivalents	$ 231,775	$ (2,050)

Cash Flows from Operating Activities

Net cash used in operating activities in all periods resulted primarily from our net losses adjusted for non-cash charges and changes in the components of working capital. The increase in net cash used in the nine months ended September 30, 2013 compared to the nine months ended September 30, 2012 primarily related to our efforts to advance Fovista into Phase 3 clinical trials, including increased spending on Phase 3 clinical trial start-up costs, manufacturing activity for Fovista and milestone payments, partially offset by the elimination of spending on our Phase 2b clinical trial for Fovista.

We expect cash used in operating activities to continue to increase substantially compared to prior periods and for the foreseeable future as we continue the development of and seek marketing approval for Fovista and, possibly, other product candidates. In August 2013, we initiated our pivotal Phase 3 clinical program for Fovista that will consist of three separate clinical trials.

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Cash Flows from Investing Activities

Net cash used in investing activities for the nine months ended September 30, 2013 and for the nine months ended September 30, 2012 was de minimis in both periods.

Cash Flows from Financing Activities

Net cash provided by financing activities was $255.0 million for the nine months ended September 30, 2013 and $7.2 million for the nine months ended September 30, 2012. Net cash provided by financing activities for the nine months ended September 30, 2013 consisted primarily of proceeds of $175.6 million from our initial public offering in September 2013, proceeds of $50.0 million from our Series C financing in May 2013 and August 2013, and proceeds of $41.7 million from our royalty agreement with Novo A/S in May 2013. These proceeds were offset by the repayment of all outstanding principal, interest and fees under our venture debt facility. Net cash provided by financing activities for the nine months ended September 30, 2012 was $7.2 million, consisting primarily of borrowings under our venture debt facility.

Funding Requirements

Our lead product candidate, Fovista, is still in clinical development. We expect our expenses to increase substantially as compared to prior periods in connection with our ongoing activities, particularly as we continue the development of and seek marketing approval for Fovista and, possibly, other product candidates. In addition, if we obtain marketing approval for Fovista or any other product candidate that we develop, we expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. Furthermore, following our initial public offering we expect to incur additional costs associated with being a public company, including legal, compliance, accounting and investor and public relations expenses as well as increased insurance premiums. We are party to agreements, specifically an acquisition agreement with OSI (Eyetech), Inc., or Eyetech, which agreement is now held by OSI Pharmaceuticals, Inc., or OSI Pharmaceuticals, a subsidiary of Astellas US, LLC, and license agreements with Archemix Corp., or Archemix, and Nektar Therapeutics, or Nektar, that impose significant milestone payment obligations on us in connection with our achievement of specific clinical, regulatory and commercial milestones with respect to Fovista.

Our expenses also will increase if and as we:

- pursue the development of Fovista for the treatment of additional indications or for use in other patient populations or, if it is approved, seek to broaden the label for Fovista;

- pursue the clinical development of ARC1905 for the treatment of wet AMD;

- in-license or acquire the rights to other products, product candidates or technologies for the treatment of ophthalmic diseases;

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

- establish sales, marketing, distribution and outsourced manufacturing capabilities, if we receive, or expect to receive, marketing approval for Fovista;

- maintain, expand and protect our intellectual property portfolio;

- hire additional clinical, quality control and scientific personnel; and

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

We expect that our existing cash and cash equivalents of $236.1 million as of September 30, 2013 and our potential future funding of $83.3 million under our royalty agreement with Novo A/S, will enable us to fund our operating expenses and capital expenditure requirements through at least the second quarter of 2016. We estimate that such funds will be sufficient to enable us to obtain initial, top-line data from our Phase 3 clinical program for Fovista. 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. This estimate assumes, among other things, that we satisfy the conditions of our royalty agreement and that we receive

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the full financing amount available under our royalty agreement with Novo A/S on a timely basis. The royalty agreement with Novo A/S provides that we will use the remaining proceeds we received and future proceeds, if any, under the royalty agreement primarily to support clinical development and regulatory activities for Fovista and for certain other permitted purposes. As of September 30, 2013, we estimate that we will incur costs, including clinical development related employee expenses and external research and development expenses, of approximately $166.0 million to obtain initial, top-line data from our Phase 3 clinical program for Fovista. We expect these data to be available in 2016. We also estimate that additional funds of approximately $46.0 million will be required to fund our other development programs and for general corporate purposes and working capital until we obtain initial, top-line data from our Phase 3 program. Costs related to our current Phase 3 clinical program for Fovista could exceed these estimates if we experience delays in our clinical trials, including the timing of our patient enrollment, the availability of drug supply for our clinical trials or for other reasons. These costs will also increase if we decide to expand the scope of our Fovista clinical program or our other development programs, or increase other corporate activities and staffing.

Our Phase 3 clinical program for Fovista is expected to continue through at least 2017, and substantial expenditures to complete the Phase 3 clinical program will be required after the receipt of initial, top-line data. At this time, we cannot reasonably estimate the remaining costs necessary to complete the Phase 3 clinical program for Fovista, complete process development and manufacturing scale-up activities associated with Fovista and seek marketing approval after we obtain initial, top-line data, or the nature, timing or costs of the efforts necessary to complete the development of any other product candidate.

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

- the progress, costs and results of our Phase 3 clinical program for Fovista;
- the costs and timing of process development and manufacturing scale-up activities associated with Fovista;
- the costs, timing and outcome of regulatory review of Fovista;
- the costs of commercialization activities for Fovista if we receive, or expect to receive, marketing approval, including the costs and timing of establishing product sales, marketing, distribution and outsourced manufacturing capabilities;
- subject to receipt of marketing approval, net revenue received from commercial sales of Fovista, after milestone payments and royalties;
- the costs of developing Fovista for the treatment of additional indications or for use in other patient populations;

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

- the scope, progress, results and costs of product development of ARC1905 and other product candidates;

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

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

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. The potential funding pursuant to our royalty agreement with Novo A/S is subject to enrollment of specified numbers of patients in our Phase 3 clinical trials of Fovista and our satisfying additional closing conditions and other obligations. To the extent that we raise additional capital through the sale of equity or convertible debt securities, stockholders ownership interest would be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Our pledge of assets, including intellectual property rights, as collateral to secure our obligations under our royalty agreement with Novo A/S may limit our ability to obtain debt financing. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product

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development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Royalty Financing

In May 2013, we entered into our royalty agreement with Novo A/S, pursuant to which we may obtain royalty financing in three tranches in an amount of up to $125.0 million in return for the sale to Novo A/S of aggregate royalties at low to mid-single-digit percentages of worldwide sales of Fovista, with the royalty percentage determined by the amount of funding provided by Novo A/S. The first tranche of the royalty financing, in which Novo A/S purchased a low single-digit royalty interest and paid us $41.7 million, closed concurrently with our entry into the royalty agreement. Under the royalty agreement, Novo A/S agreed to purchase from us, and we agreed to sell to Novo A/S, two additional low single-digit royalty interests on worldwide sales of Fovista, in each case, for a purchase price of $41.7 million, or $83.3 million in the aggregate for both additional tranches. If all royalty interests under the royalty agreement are purchased, Novo A/S will have a right to receive royalties on worldwide sales of Fovista at a mid-single-digit percentage. The closing of each of the two subsequent financing tranches is subject to the enrollment of a specified number of patients in our Phase 3 clinical trials of Fovista and our satisfying additional closing conditions and other obligations.

Under specified circumstances, including terminations, suspensions or delays of our Phase 3 clinical trials for Fovista, the failure of certain closing conditions to be satisfied or transactions involving a change of control of us in which the acquiring party does not meet certain specifications, Novo A/S has the option to cancel the subsequent purchase and sale of the additional royalty interests. We also have the option to cancel the subsequent purchase and sale of the additional royalty interests in specified circumstances, including terminations, suspensions or delays in our Phase 3 clinical trials for Fovista, any change of control of us or the completion of equity financings meeting specified thresholds.

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 our agreement with Novo A/S 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 royalty agreement to repay in full an aggregate of $14.4 million of outstanding principal, interest and fees under our venture debt facility. The royalty agreement provides that we will use the remaining proceeds we received, and future proceeds, if any, from the sale of royalty interests under the royalty agreement, primarily to support clinical development and regulatory activities for Fovista and, to the extent applicable, other specified products we may develop pursuant to the terms of the royalty agreement, and for general corporate expenses.

The royalty agreement requires the establishment by us and Novo A/S of a joint oversight committee in relation to the development of Fovista in the event that Novo A/S does not continue to have a representative on our board of directors. The royalty agreement also contains customary representations and warranties, as well as certain covenants relating to the operation of our business, including covenants requiring us to use commercially reasonable efforts to continue our development of Fovista, to file, prosecute and maintain certain patent rights and, in our reasonable judgment, to pursue claims of infringement of our intellectual property rights. The royalty 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 are required to reimburse Novo A/S for specified legal and other expenses and 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 royalty 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.

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Contractual Obligations and Commitments

The following table summarizes our contractual obligations as of September 30, 2013:

	Total	Payments Due by Period Less than 1 year	1-3 years	3-5 years	More than 5 years
			(in thousands)		
Operating Leases (1)	$ 208	$ 68	$ 140	$	$
Total (2)	$ 208	$ 68	$ 140	$	$

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

(2) This table does not include (a) any milestone payments which may become payable to third parties under license agreements as the timing and likelihood of such payments are not known with certainty, (b) any royalty payments to third parties as the amounts, timing and likelihood of such payments are not known, (c) contracts that are entered into in the ordinary course of business which are not material in the aggregate in any period presented above and (d) the royalty purchase liability of $41.7 million due to the fact that the royalty payment period is not known.

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

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

- Under a license agreement with Archemix Corp., or Archemix, with respect to pharmaceutical products comprised of or derived from any anti-PDGF aptamer, we are obligated to make future payments to Archemix of up to an aggregate of $14.0 million if we achieve specified clinical and regulatory milestones with respect to Fovista, up to an aggregate of $3.0 million if we achieve specified commercial milestones with respect to Fovista and, for each other anti-PDGF aptamer product that we may develop under the agreement, up to an aggregate of approximately $18.8 million if we achieve specified clinical and regulatory milestones and up to an aggregate of $3.0 million if we achieve specified commercial milestones. No royalties are payable to Archemix under this license agreement. From inception through September 30, 2013, we have made payments of approximately $4.8 million resulting from this agreement, including a $2.5 million payment to Archemix that was triggered by the initiation of our Phase 3 clinical program for Fovista in August 2013.

- Under a license agreement with Archemix with respect to pharmaceutical products comprised of or derived from anti-C5 aptamers, for each anti-C5 aptamer product that we may develop under the agreement, including ARC1905, we are obligated to make future payments to Archemix of up to an aggregate of $57.5 million if we achieve specified development, clinical and regulatory milestones and, as to

all anti-C5 products under the agreement collectively, up to an aggregate of $22.5 million if we achieve specified commercial milestones. We are also obligated to pay Archemix a double-digit percentage of specified non-royalty payments we may receive from any sublicensee of our rights under this license agreement. No royalties are payable to Archemix under this license agreement. From inception through September 30, 2013, we have made payments totaling $2.0 million under this agreement.

- Under a license, manufacturing and supply agreement with Nektar Therapeutics, or Nektar, for specified pegylation reagents used to manufacture Fovista, we are obligated to make future payments to Nektar of up to an aggregate of $4.5 million if we achieve specified clinical and regulatory milestones, and an additional payment of $3.0 million if we achieve a specified commercial milestone with respect to Fovista. We are obligated to pay Nektar tiered royalties at low to mid-single-digit percentages of net sales of any licensed product we successfully commercialize, with the royalty percentage determined by our level of licensed product sales, the extent of patent coverage for the licensed product and whether we have granted a third-party commercialization rights to the

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licensed product. We have agreed to pay Nektar a low double-digit percentage of any upfront payment we receive in connection with granting any third-party commercialization rights to a licensed product less certain milestone events the company has previously paid, and a higher double-digit percentage of other specified amounts, such as milestone payments, we receive in connection with any such commercialization agreement, subject to agreed minimum and maximum amounts. From inception through September 30, 2013, we have made approximately $1.8 million in payments resulting from this agreement, including a $1.0 million payment to Nektar that was triggered by the initiation of our Phase 3 clinical program for Fovista in August 2013.

- Under our royalty agreement with Novo A/S with respect to Fovista, we are obligated to pay Novo A/S a low to mid-single-digit percentage royalty based on worldwide sales of Fovista, with the royalty percentage determined by the amount of funding provided by Novo A/S. See Royalty Financing above for further information about our royalty agreement with Novo A/S.

We also have employment agreements with certain employees which require the funding of a specific level of payments, if certain events, such as a change in control or termination without cause, occur.

In addition, in the course of normal business operations, we have agreements with contract service providers to assist in the performance of our research and development and manufacturing activities. Expenditures to CROs represent a significant cost in clinical development. We can elect to discontinue the work under these agreements at any time. We could also enter into additional collaborative research, contract research, manufacturing, and supplier agreements in the future, which may require upfront payments and even long-term commitments of cash.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under Securities and Exchange Commission rules.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are exposed to market risk related to changes in interest rates. We had cash and cash equivalents of $236.1 million as of September 30, 2013, consisting of cash and 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 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 short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

We contract with CROs and contract manufacturers globally. We may be subject to fluctuations in foreign currency rates in connection with certain of these agreements. Transactions denominated in currencies other than the U.S. dollar are recorded based on exchange rates at the time such transactions arise. As of September 30, 2013, substantially all of our total liabilities were denominated in the U.S. dollar.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2013. The term disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-

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benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2013, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(d) and 15d-15(d) under the Exchange Act) occurred during the three months ended September 30, 2013 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

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PART II OTHER INFORMATION

Item 1. Legal Proceedings.

We are not currently subject to any material legal proceedings.

Item 1A. Risk Factors.

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

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant operating losses since our inception. We expect to incur losses for at least the next several years and may never achieve or maintain profitability.

Since inception, we have incurred significant operating losses. Our net loss was $30.8 million for the nine months ended September 30, 2013 and $10.7 million for the nine months ended September 30, 2012. As of September 30, 2013, we had a deficit accumulated during the development stage of $162.7 million. To date, we have not generated any revenues and have financed our operations primarily through private placements of our preferred stock, venture debt borrowings, our royalty purchase and sale agreement with Novo A/S and our initial public offering, which we closed in September 2013. We issued and sold an aggregate of 8,740,000 shares of common stock in our initial public offering at a public offering price of $22.00 per share, including 1,140,000 shares pursuant to the exercise by the underwriters of an over-allotment option. We received net proceeds from the initial public offering of $175.6 million, after deducting underwriting discounts and commissions and other offering expenses payable by us. We have devoted substantially all of our financial resources and efforts to research and development. We expect to continue to incur significant expenses and increasing operating losses over the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year.

Our lead product candidate, Fovista, is still in clinical development. We expect our expenses to increase substantially as compared to prior periods in connection with our ongoing activities, particularly as we continue the development of and seek marketing approval for Fovista and, possibly, other product candidates. In addition if we obtain marketing approval for Fovista or any other product candidate that we develop, we expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. Furthermore, following our initial public offering, we expect to incur additional costs associated with being a public company, including legal, compliance, accounting and investor and public relations expenses, as well as increased insurance premiums. We are party to agreements, specifically an acquisition agreement with OSI (Eyetech), Inc., or Eyetech, which agreement is now held by OSI Pharmaceuticals, Inc., or OSI Pharmaceuticals,

a subsidiary of Astellas US, LLC, and license agreements with Archemix Corp., or Archemix, and Nektar Therapeutics, or Nektar, that impose significant milestone payment obligations on us in connection with our achievement of specific clinical, regulatory and commercial milestones with respect to Fovista.

Our expenses also will increase if and as we:

- pursue the development of Fovista for the treatment of additional indications or for use in other patient populations or, if it is approved, seek to broaden the label for Fovista;

- pursue the clinical development of our product candidate ARC1905 for the treatment of wet and dry AMD;

- in-license or acquire the rights to other products, product candidates or technologies for the treatment of ophthalmic diseases;

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

- establish sales, marketing, distribution and outsourced manufacturing capabilities if we receive, or expect to receive, marketing approval for Fovista;

- maintain, expand and protect our intellectual property portfolio;

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- hire additional clinical, quality control and scientific personnel; and

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

If we are required by the U.S. Food and Drug Administration, or FDA, the European Medicines Agency, or EMA, or regulatory authorities in other jurisdictions to perform clinical trials or studies in addition to those we currently expect to conduct, or if there are any delays in completing the clinical trials of Fovista or the development of any of our other product candidates, our expenses could increase.

Our ability to become and remain profitable depends on our ability to generate revenue. We do not expect to generate significant revenue unless and until we obtain marketing approval for, and commercialize, Fovista, which we do not expect will occur before 2017, if ever. This will require us to be successful in a range of challenging activities, including:

- initiating and obtaining favorable results from our Phase 3 clinical program for Fovista;

- subject to obtaining favorable results from our Phase 3 clinical program, applying for and obtaining marketing approval for Fovista;

- establishing sales, marketing and distribution capabilities to effectively market and sell Fovista in the United States with our own specialty sales force targeting retinal specialists;

- establishing collaboration, distribution or other marketing arrangements with third parties to commercialize Fovista in markets outside the United States;

- protecting our rights to our intellectual property portfolio related to Fovista; and

- ensuring the manufacture of commercial quantities of Fovista.

We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our

research and development efforts, diversify our product offerings or continue our operations. A decline in the value of our company could also cause our stockholders to lose all or part of their investment.

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

We are an early-stage company. We were incorporated and commenced active operations in 2007. Our operations to date have been limited to organizing and staffing our company, acquiring rights to product candidates, business planning, raising capital and developing Fovista and our other product candidates. We have not yet demonstrated our ability to successfully complete a large-scale, pivotal clinical trial, obtain marketing approval, manufacture at commercial scale, or arrange for a third party to do so on our behalf, or conduct sales, marketing and distribution activities necessary for successful product commercialization. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history.

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

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

We expect our expenses to increase substantially as compared to prior periods in connection with our ongoing activities, particularly as we continue the development of and seek marketing approval for Fovista and, possibly, other product candidates. Our expenses will increase if we suffer any delays in our Phase 3 clinical program for Fovista, including delays in receipt of regulatory clearance to begin our Phase 3 clinical trials or delays in enrollment of patients. If we obtain marketing approval for Fovista or any other product candidate that we develop, we expect to incur significant

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commercialization expenses related to product sales, marketing, distribution and manufacturing. Furthermore, following our initial public offering, we expect to incur additional costs associated with operating as a public company, hiring additional personnel and expanding our facilities. Accordingly, we will 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.

We expect that our existing cash and cash equivalents of $236.1 million as of September 30, 2013, together with potential future funding of $83.3 million under our royalty agreement with Novo A/S, will enable us to fund our operating expenses and capital expenditure requirements through at least the second quarter of 2016. 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. This estimate assumes, among other things, that we receive the full financing amount available under our royalty agreement with Novo A/S on a timely basis. The royalty agreement with Novo A/S provides that we will use the remaining proceeds we received and future proceeds, if any, under the royalty agreement primarily to support clinical development and regulatory activities for Fovista and for certain other permitted purposes. As of September 30, 2013, we estimate that we will incur costs, including clinical development related employee expenses and external research and development expenses, of approximately $166.0 million to obtain initial, top-line data from our Phase 3 clinical program for Fovista. We expect these data to be available in 2016. We also estimate that additional funds of approximately $46.0 million will be required to fund our other development programs and for general corporate purposes and working capital until we obtain initial, top-line data from our Phase 3 program. Costs related to our current Phase 3 clinical program for Fovista could exceed these estimates if we experience delays in our clinical trials, including the timing of our patient enrollment, the availability of drug supply for our clinical trials or for other reasons. These costs will also increase if we decide to expand the scope of our Fovista clinical program or our other development programs, or increase other corporate activities and staffing.

Our Phase 3 clinical program for Fovista is expected to continue through at least 2017, and substantial expenditures to complete the Phase 3 clinical program will be required after the receipt of initial, top-line data. At this time, we cannot reasonably estimate the remaining costs necessary to complete the Phase 3 clinical program for Fovista, complete process development and manufacturing scale-up activities associated with Fovista and seek marketing approval after we obtain initial, top-line data, or the nature, timing or costs of the efforts necessary to complete the development of any other product candidate.

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

- the progress, costs and results of our Phase 3 clinical program for Fovista;
- the costs and timing of process development and manufacturing scale-up activities associated with Fovista;
- the costs, timing and outcome of regulatory review of Fovista;
- the costs of commercialization activities for Fovista if we receive, or expect to receive, marketing approval, including the costs and timing of establishing product sales, marketing, distribution and outsourced manufacturing capabilities;

- subject to receipt of marketing approval, revenue received from commercial sales of Fovista, after milestone payments and royalties;

- the costs of developing Fovista for the treatment of additional indications or for use in other patient populations;

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

- the scope, progress, results and costs of product development of ARC1905 and any other product candidates that we may develop;

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

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

Our commercial revenues, if any, will be derived from sales of Fovista or any other products that we successfully develop, none of which do we expect to be commercially available for several years, if at all. In addition, if approved, Fovista or any other product candidate that we develop or any product that we in-license may not achieve commercial success. Accordingly, we will need to obtain substantial additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all. In addition, we may seek additional capital due to

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favorable market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans.

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

In May 2013, we entered into a royalty purchase and sale agreement, or royalty agreement, with Novo A/S for a financing of up to $125.0 million in return for the sale to Novo A/S of royalty interests in worldwide sales of Fovista. We received approximately $41.7 million of this royalty financing in May 2013. We are obligated to pay Novo A/S royalties at low to mid-single-digit percentages of worldwide sales of Fovista, with the royalty percentage determined by the amount of funding provided by Novo A/S.

We are subject to diligence and other obligations under our royalty agreement with Novo A/S. If we fail to enroll the specified numbers of patients in our Phase 3 clinical trials of Fovista and satisfy additional closing conditions under the royalty agreement or fail to satisfy our other obligations, Novo A/S will have no further obligation to pay additional funds to us under the royalty agreement. We would then need to raise substantial additional funding through public or private equity offerings, debt financings, corporate collaboration and licensing arrangements or other financing alternatives. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay or terminate our research and development programs, including for Fovista, or any future commercialization efforts.

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

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

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. The potential funding pursuant to our royalty agreement with Novo A/S is subject to enrollment of specified numbers of patients in our Phase 3 clinical trials of Fovista and our satisfying additional closing conditions and other obligations. We do not have any other committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our existing stockholders rights as a holders of our common stock. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

Our pledge of assets, including intellectual property rights, as collateral to secure our obligations under our royalty agreement with Novo A/S may limit our ability to obtain debt financing.

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

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

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

Risks Related to Product Development and Commercialization

We depend heavily on the success of our lead product candidate, Fovista, which we are developing to be administered in combination with anti-VEGF drugs for the treatment of patients with wet AMD. If we are unable to complete our Phase 3 clinical program and obtain marketing approvals for Fovista, or thereafter we fail to commercialize Fovista or experience significant delays in doing so, our business will be materially harmed.

We have invested a significant portion of our efforts and financial resources in the development of Fovista to be administered in combination with anti-VEGF drugs for the treatment of patients with wet AMD. There remains a significant risk that we will fail to successfully develop Fovista. The results of our Phase 2b clinical trial may not be predictive of the results of our Phase 3 clinical program due, in part, to the fact that we have no clinical data on Fovista combination therapy in any clinical trial longer than 24 weeks, that we have no clinical data on the effects of Fovista when administered in combination with Avastin or Eylea and that we plan to conduct our Phase 3 clinical trials at many clinical centers that were not included in our Phase 2b clinical trial.

We do not expect to have initial, top-line data from our Phase 3 clinical program for Fovista available until 2016. The timing of the availability of such top-line data and the completion of our Phase 3 clinical program is dependent, in part, on our ability to locate and enroll a sufficient number of eligible patients in our Phase 3 clinical program on a timely basis. The timing of the availability of initial, top-line data from our Phase 3 clinical trial evaluating the safety and efficacy of Fovista administered in combination with Avastin or Eylea may be subject to particular variability because we have no clinical experience testing Fovista administered in combination with Avastin or Eylea. If we ultimately obtain statistically significant, positive results from our Phase 3 clinical program, we do not expect to submit applications for marketing approval for Fovista until the end of 2016.

If we are not able to obtain data from our Phase 3 clinical trial evaluating Fovista administered in combination with each of Avastin or Eylea when data from our other two Phase 3 clinical trials evaluating Fovista administered in combination with Lucentis are available, we may nonetheless decide to proceed with submitting applications for marketing approval for Fovista administered only in combination with Lucentis. If we submit applications for marketing approval for Fovista only in combination with Lucentis, we may determine either to delay seeking approval of Fovista in combination with Avastin or Eylea until after regulatory authorities have considered and acted on our applications for Fovista in combination with Lucentis, or to amend our applications once data from our third Phase 3 clinical trial become available. If we were to delay seeking approval of Fovista in combination with Avastin or Eylea pending regulatory action on our applications for Fovista in combination with Lucentis, the FDA or other regulatory authorities could defer taking action on our applications while data remain outstanding from our third Phase 3 clinical trial. Moreover, if we subsequently amend our applications for marketing approval when data from our third Phase 3 clinical trial become available, we may experience further delays in our application process. Additionally, we expect that our Phase 3 clinical trials will continue in accordance with their protocols after we submit applications for marketing approval, and the conclusions of those trials may yield data that are inconsistent with the initial data used to support our applications. As a result of these and other factors, we cannot

accurately predict when or if Fovista will prove effective or safe in humans or will receive marketing approval. We do not know precisely the timing of clinical trials or marketing approvals for other product candidates.

Our ability to generate product revenues, which we do not expect will occur before 2017, if ever, will depend heavily on our obtaining marketing approval for and commercializing Fovista. The success of Fovista will depend on several factors, including the following:

- obtaining favorable results from clinical trials;

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- making arrangements with third-party manufacturers and receiving regulatory approval of our manufacturing processes and our third-party manufacturers facilities from applicable regulatory authorities;

- receipt of marketing approvals from applicable regulatory authorities for the use of Fovista in combination with anti-VEGF drugs for the treatment of wet AMD, particularly which anti-VEGF drugs are included in any such approval;

- launching commercial sales of Fovista, if and when approved, whether alone or in collaboration with others;

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

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

- effectively competing with other therapies, including the existing standard of care;

- maintaining a continued acceptable safety profile of Fovista following approval;

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

- protecting our rights in our intellectual property portfolio.

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

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize Fovista in combination with anti-VEGF drugs for the treatment of wet AMD or for any additional indication, which would materially harm our business.

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

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

Our Phase 2b clinical trial evaluated a combination of Fovista and Lucentis. In this trial, patients treated with a combination of 0.3 mg of Fovista and Lucentis did not achieve statistically significant superiority compared to Lucentis monotherapy based on the pre-specified primary endpoint of mean change in visual acuity from baseline at the 24 week timepoint. Although a combination of 1.5 mg of Fovista and Lucentis demonstrated statistically significant superiority in this trial compared to Lucentis monotherapy based on the pre-specified primary endpoint of mean change in visual acuity from baseline at the 24 week timepoint, we may nonetheless fail to achieve success in our Phase 3 clinical trials involving a combination of 1.5 mg of Fovista and Lucentis for a variety of potential reasons.

- The primary endpoint of mean change in visual acuity in our Phase 2b clinical trial was measured 24 weeks after the first dose of Fovista. The primary endpoint of mean change in visual acuity in our Phase 3 clinical program will be measured 12 months after the first dose of Fovista. We have no clinical data on Fovista combination therapy in any clinical trial longer than 24 weeks. If the positive results we observed at 24 weeks in our Phase 2b clinical trial are not observed at 12 months, we likely will not receive marketing approval for Fovista.

- Retrospective subgroup analyses that we performed on the results of our Phase 2b clinical trial may not be predictive of the results of our Phase 3 clinical program. Although we believe that the retrospective analyses further support the results from our primary endpoint and our proposed mechanism of action, retrospective analyses performed after

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unblinding trial results can result in the introduction of bias and are given less weight by regulatory authorities than pre-specified analyses.

- We plan to conduct our Phase 3 clinical trials at many clinical centers that were not included in our Phase 2b clinical trial. The introduction of new centers, and the resulting involvement of new treating physicians, can introduce additional variability into the conduct of the trials in accordance with their protocols and may result in greater variability of patient outcomes, which could adversely affect our ability to detect statistically significant differences between patients treated with 1.5 mg of Fovista administered in combination with an anti-VEGF drug and anti-VEGF drug monotherapy.

- Our Phase 3 clinical program involves two Phase 3 clinical trials testing a combination of 1.5 mg of Fovista and Lucentis for the treatment of wet AMD and one trial testing a combination of 1.5 mg of Fovista with each of Avastin or Eylea for the treatment of wet AMD. We have no clinical efficacy data on the effects of Fovista when administered in combination with Avastin or Eylea for the treatment of patients with wet AMD. Avastin is not approved for such use.

Fovista administered in combination with Lucentis was generally well tolerated in our Phase 1 and Phase 2b clinical trials. However, the results of these clinical trials may not be predictive of the results of our Phase 3 clinical program for Fovista due, in part, to the fact that we have no clinical safety data on patient exposure to Fovista administered in combination with any anti-VEGF drug for longer than 24 weeks and that we have no clinical safety data on the effects of Fovista when administered in combination with Avastin or Eylea.

In general, the FDA and similar regulatory authorities outside the United States require two adequate and well controlled clinical trials demonstrating safety and effectiveness for marketing approval. If a combination of 1.5 mg of Fovista and Lucentis fails to achieve superiority over Lucentis monotherapy with statistical significance on the primary endpoint of mean change in visual acuity from baseline at 12 months in both of our Phase 3 clinical trials evaluating the safety and efficacy of this combination, we likely will not receive marketing approval for Fovista even if the combination of 1.5 mg of Fovista with Avastin or Eylea achieves superiority over Avastin or Eylea monotherapy with statistical significance on the primary endpoint in one of our Phase 3 clinical trials. There are a variety of other possible outcomes of our Phase 3 clinical trials. As described below, positive outcomes in one or more of our Phase 3 clinical trials may not be sufficient for the FDA or similar regulatory authorities outside the United States to grant marketing approval for Fovista.

- If a combination of 1.5 mg of Fovista and Lucentis achieves superiority over Lucentis monotherapy with statistical significance on the primary endpoint in only one of our Phase 3 clinical trials and the combination of 1.5 mg of Fovista with Avastin or Eylea does not achieve superiority over Avastin or Eylea monotherapy with statistical significance on the primary endpoint in our other Phase 3 clinical trials, we likely will not receive marketing approval for Fovista.

- If a combination of 1.5 mg of Fovista and Lucentis achieves superiority over Lucentis monotherapy with statistical significance on the primary endpoint in only one of our Phase 3 clinical trials and the combination of 1.5 mg of Fovista with Avastin or Eylea achieves superiority over Avastin or Eylea monotherapy with statistical significance on the primary endpoint in our other Phase 3 clinical trial, the FDA or similar regulatory authorities outside the United States may nonetheless not grant marketing approval for Fovista.

- Even if a combination of 1.5 mg of Fovista and an anti-VEGF drug achieves superiority over an anti-VEGF drug monotherapy with statistical significance on the primary endpoint in two or all three of our Phase 3 clinical trials, the FDA or similar regulatory authorities outside the United States may nonetheless not grant marketing approval for Fovista if such regulatory authorities do not believe that the benefits offered by Fovista administered in combination with an anti-VEGF drug are clinically meaningful or that such benefits outweigh the observed or potential risks.

In the United States, Avastin and Eylea are two of the most widely used anti-VEGF drugs for the treatment of wet AMD. If a combination of 1.5 mg of Fovista with Avastin or Eylea does not achieve superiority over Avastin or Eylea monotherapy with statistical significance on the primary endpoint of mean change in visual acuity from baseline at 12 months in our Phase 3 clinical program, our ability to successfully commercialize Fovista in combination with any anti-VEGF drug could be harmed materially. In addition, any failure of Fovista administered in combination with Avastin or Eylea to achieve superiority over Avastin or Eylea monotherapy with statistical significance on the primary endpoint could cause the FDA or similar regulatory authorities outside the United States to require additional clinical trials or other research before granting

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marketing approval of Fovista for use in combination with any anti-VEGF drug, including Lucentis, for the treatment of patients with wet AMD.

The protocols for our Phase 3 clinical trials and other supporting information are subject to review by the FDA and regulatory authorities outside the United States. The FDA is not obligated to comment on our protocols within any specified time period or at all or to affirmatively clear or approve our Phase 3 clinical program. We have submitted the protocols for our Phase 3 clinical trials to the FDA and have initiated two of the trials in our Phase 3 clinical program in the United States, both of which are evaluating the safety and efficacy of Fovista administered in combination with Lucentis, without waiting for any such comments. The FDA or other regulatory authorities may request additional information, require us to conduct additional non-clinical trials or require us to modify our proposed Phase 3 clinical program, including its endpoints, patient enrollment criteria or selection of anti-VEGF drugs, to receive clearance to initiate such program or to continue such program once initiated. The FDA, the EMA or other regulatory authorities may be more likely to request any such modification with respect to our Phase 3 clinical trial evaluating the safety and efficacy of Fovista administered in combination with Avastin or Eylea because we have no clinical data on the effects of Fovista when administered in combination with Avastin or Eylea.

In September 2013, the EMA, through the Scientific Advice Working Party, provided initial input in connection with our seeking scientific advice from the EMA regarding our Phase 3 clinical program for Fovista. More recently, we received scientific advice regarding our Phase 3 clinical program for Fovista, and our plan to seek regulatory approval for Fovista, from the Committee for Medicinal Products for Human Use, or CHMP, of the EMA, which is the committee responsible for preparing opinions on questions concerning medicines for human use. The CHMP advised us that the planned primary endpoint for each of the Fovista Phase 3 clinical trials, mean change from baseline in best corrected visual acuity, was acceptable. In addition, the CHMP confirmed that carcinogenicity studies are not needed. The CHMP also advised us that we should justify our proposal to initiate, at the Phase 3 clinical trial stage, previously untested combinations of Fovista with Avastin or Eylea and that we should consider conducting toxicity studies with Fovista administered in combination with Avastin or Eylea prior to initiating our corresponding Phase 3 clinical trial. In addition, the CHMP informed us that the final label for Fovista, if it receives marketing approval, may be required to specify the licensed anti-VEGF agents that were studied in combination with Fovista, given that Avastin is not licensed for intravitreal use, rather than a broad label specifying Fovista for use in combination with any anti-VEGF agent. The CHMP further advised us that there will be a requirement for additional data to bridge the results from our Phase 3 clinical trials of Fovista administered in combination with Lucentis to the less frequent dosing regimens of Lucentis and Eylea approved in the European Union. We intend to have further scientific discussion with the CHMP on these issues and do not currently expect that additional toxicity studies will be required. We plan to adjust the dosing schedule in our Phase 3 clinical program for Fovista administered in combination with Eylea so that no bridging study would be needed for this combination.

We may not receive clearance from regulatory authorities in jurisdictions outside the United States to initiate our Phase 3 clinical program in those jurisdictions on a timely basis, or at all. In addition, any modifications to our Phase 3 clinical program for Fovista may result in our incurring increased expense or in a delay in the enrollment or completion of such program. Although we are still considering the possible design of a bridging study for Fovista administered in combination with Lucentis and discussing with the CHMP the need to conduct any such study, we expect that, if we determine to conduct such a study, it would not have a material impact on our plan, based on our estimates regarding patient enrollment in our Phase 3 clinical program, to have initial, top-line data from our Phase 3 clinical program for Fovista available in 2016. We also anticipate that our existing cash and cash equivalents and potential funding under our royalty agreement with Novo A/S will be sufficient to enable us to fund our operating expenses and capital expenditure requirements, including any additional costs not previously contemplated for a possible bridging study for Fovista administered in combination with Lucentis, through at least the second quarter of 2016.

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

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

- not obtain marketing approval at all;

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

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

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

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

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

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

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- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;

- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;

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

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

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

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

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

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

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

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

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

Patient enrollment is affected by other factors including:

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

Additional financing under our royalty agreement with Novo A/S is contingent upon enrolling specified numbers of patients in our Phase 3 clinical trials of Fovista and our satisfying additional closing conditions and other obligations. Novo A/S will not be required to provide the additional royalty financing unless we enroll the specified numbers of patients. In addition, our inability to locate and enroll a sufficient number of patients for our clinical trials would result in significant delays in our clinical trials, could require us to abandon one or more clinical trials altogether and could delay or prevent our

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receipt of necessary regulatory approvals. Enrollment delays in our clinical trials also may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

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

If Fovista or any other 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. Although, Fovista administered in combination with Lucentis was generally well tolerated in our Phase 1 clinical trial and our Phase 2b clinical trial, we have no clinical safety data on patient exposure to Fovista administered in combination with Lucentis for longer than 24 weeks, and we have no clinical safety data on the effects of Fovista when administered in combination with Avastin or Eylea. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause side effects that prevented further development of the compound. Our Phase 3 clinical program for Fovista involves the administration of Fovista in combination with anti-VEGF drugs, and the safety results of our trials are dependent, in part, on the safety and tolerability of the co-administered anti-VEGF drug. Avastin is not approved for the treatment of wet AMD, and according to third-party clinical studies, may be associated with a greater risk of serious adverse events or undesirable side effects as compared to Lucentis.

Even if Fovista or any other product candidate that we develop receives marketing approval, it 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 Fovista may be smaller than we estimate.

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

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

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

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

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

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

- our ability to offer our products at competitive prices, particularly in light of the additional cost of Fovista together with an anti-VEGF drug;

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

- willingness of the target patient population to try new therapies and of physicians to prescribe these therapies, particularly in light of the existing available standard of care;

- prevalence and severity of any side effects;

- whether alternatives are more convenient or easier to administer; and

- strength of our marketing and distribution support.

In addition, the potential market opportunity for Fovista is difficult to estimate precisely. If Fovista receives marketing approval for the treatment of wet AMD, it will be used solely in combination with an anti-VEGF drug. The market opportunity for Fovista will be dependent upon the continued use of anti-VEGF drugs in the treatment of wet AMD and the market share of such anti-VEGF drugs for which Fovista is approved as a combination therapy. In addition, because physicians, patients and third-party payors may be sensitive to the addition of the cost of Fovista to the cost of treatment with anti-VEGF drugs, we may experience downward pressure on the price we can charge for Fovista.

Our Phase 3 clinical program excludes from enrollment wet AMD patients with pure occult choroidal neovascularization. Based on enrollment of wet AMD patients in third-party clinical trials, the pure occult subtype accounts for approximately 40% of the cases of subfoveal wet AMD. If Fovista receives marketing approval for the treatment of wet AMD and the approved label excludes patients with pure occult lesions, the potential market opportunity for Fovista will be limited to the extent that physicians do not prescribe Fovista for such patients.

Our Phase 3 clinical program provides for a 30-minute delay in the injection of Fovista after the anti-VEGF drug to minimize the risk in our clinical trials of an unacceptable increase in intraocular pressure as a result of the amount of the two agents injected. If Fovista receives marketing approval for the treatment of wet AMD and the approved label requires such a waiting period, the potential market opportunity for Fovista may be limited to the extent that physicians and patients find such a waiting period unacceptable.

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

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

The development and commercialization of new drug products is highly competitive. We face competition with respect to Fovista from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of product candidates for the treatment of wet AMD or other disease indications for which we may develop Fovista. 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

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

The current standard of care for wet AMD is monotherapy administration of anti-VEGF drugs, principally Avastin, Lucentis and Eylea. We are developing Fovista for administration in combination with these anti-VEGF drugs. These drugs are well established therapies and are widely accepted by physicians, patients and third-party payors. When used for the treatment of wet AMD, Avastin is inexpensive. Physicians, patients and third-party payors may not accept the addition of Fovista to their current treatment regimens for a variety of potential reasons, including:

- if they do not wish to incur the additional cost of Fovista;
- if they perceive an additional injection to administer Fovista as undesirable;
- if they perceive the addition of Fovista to be of limited benefit to patients; or
- if they wish to treat with anti-VEGF drugs as monotherapy first and add Fovista only if and when resistance to continued anti-VEGF therapy limits further enhancement of visual outcome with anti-VEGF monotherapy.

There are also a number of products in preclinical research and clinical development by third parties to treat wet AMD, including product candidates that inhibit the function of PDGF, the molecule whose function Fovista also inhibits, product candidates that inhibit the function of both VEGF and PDGF that could obviate the separate use of an anti-PDGF agent, such as Fovista, and anti-VEGF gene therapy products that may substantially reduce the number and frequency of intravitreal injections when treating wet AMD. These companies include pharmaceutical companies, biotechnology companies, and specialty pharmaceutical and generic drug companies of various sizes, such as Regeneron Pharmaceuticals, Inc., Allergan, Inc., Xcovery Vision LLC, Neurotech Pharmaceuticals, Inc., Avalanche Biotechnologies, Inc, Somalogic, Inc, and others.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient to use or are less expensive than Fovista or other products that we may develop. The commercial opportunity for Fovista also could be reduced or eliminated if our competitors develop and commercialize products that reduce or eliminate the use of anti-VEGF drugs for the treatment of patients with wet AMD. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

In addition, our ability to compete may be affected in many cases by insurers or other third-party payors, particularly Medicare, seeking to encourage the use of less expensive or more convenient products. We expect that if Fovista is approved, the cost of treatment of wet AMD with a combination of Fovista with an anti-VEGF drug will be significantly higher than the cost of treatment of wet AMD with Avastin, Lucentis or

Eylea monotherapy. Insurers and other third-party payors may encourage the use of anti-VEGF drugs as monotherapy and discourage the use of Fovista in combination with these drugs. This could limit sales of Fovista.

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

We have no experience manufacturing Fovista or ARC1905 at commercial scale. As a result, delays in regulatory approval of Fovista or ARC1905 may occur. Also, manufacturing issues may arise that could cause delays or increase costs.

We have no experience manufacturing the chemically synthesized aptamers comprising the active pharmaceutical ingredients of Fovista or ARC1905 at commercial scale. We currently rely on a single third-party manufacturer to supply us with Fovista drug substance on a purchase order basis. In order to obtain regulatory approval for Fovista, this third-party

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manufacturer will be required to consistently produce the active pharmaceutical ingredient used in Fovista in commercial quantities and of specified quality on a repeated basis and document its ability to do so. This is referred to as process validation. If this third-party manufacturer is unable to satisfy this requirement, our business will be materially and adversely affected.

Our third-party manufacturer has made only a limited number of lots of Fovista to date and has not made any commercial lots. The manufacturing processes for Fovista have never been tested at commercial scale, and the process validation requirement has not yet been satisfied. These manufacturing processes and our third-party manufacturer s facility will be subject to inspection and approval by the FDA before we can commence the manufacture and sale of Fovista. Our third-party manufacturer has never been inspected by the FDA and has not been through the FDA approval process for a commercial product. If our third-party manufacturer is unable to pass such inspection and otherwise satisfactorily complete the FDA approval regimen, our business will be materially and adversely affected.

The standards of the International Conference on Harmonization of Technical Requirements for Registration of Pharmaceuticals for Human Use, which establishes basic guidelines and standards for drug development in the United States, the European Union, Japan and other countries, do not apply to oligonucleotides, including aptamers. As a result, there is no established generally accepted manufacturing or quality standard for the production of Fovista or ARC1905. Even though the FDA has reviewed the quality standards for Fovista to be used in our Phase 3 clinical program, the FDA has the ability to modify these standards at any time and foreign regulatory agencies may impose differing quality standards and quality control on the manufacture of Fovista. The lack of uniform manufacturing and quality standards among regulatory agencies may delay regulatory approval of Fovista or ARC1905.

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

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

We do not have a sales, marketing or distribution infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product, we must either develop a sales, marketing and distribution organization or outsource those functions to third parties. If Fovista receives marketing approval, we plan to commercialize it in the United States with our own focused, specialty sales force targeting retinal specialists. In addition, we expect to utilize a variety of types of collaboration, distribution and other marketing arrangements with third parties to commercialize Fovista in markets outside the United States.

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 or persuade adequate numbers of physicians to prescribe our products;

- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and

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- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

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

Even if we are able to commercialize Fovista or any other product candidate that we develop, the product may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Our ability to commercialize Fovista or any other product candidate successfully also will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A major trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors, particularly Medicare, have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for Fovista or any other product that we commercialize, and, even if these are available, the level of reimbursement may not be satisfactory.

Reimbursement may affect the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining and maintaining adequate reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician and because, in the case of Fovista, our drug will be administered in combination with other drugs that may carry high prices. In addition, physicians, patients and third-party payors may be sensitive to the addition of the cost of Fovista to the cost of treatment with anti-VEGF drugs. We may be required to conduct expensive pharmacoeconomic studies to justify coverage and reimbursement or the level of reimbursement relative to other therapies, including in the case of Fovista, relative to monotherapy with anti-VEGF drugs. If coverage and adequate reimbursement are not available or reimbursement is available only to limited levels, we may not be able to successfully commercialize Fovista or any other product candidate for which we obtain marketing approval.

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

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

Our strategy of obtaining rights to product candidates and approved products for the treatment of a range of ophthalmic diseases through in-licenses and acquisitions may not be successful.

We may expand our product pipeline through opportunistically in-licensing or acquiring the rights to other products, product candidates or technologies for the treatment of ophthalmic diseases. Because we expect generally that we will not engage in early stage research and drug discovery, the future growth of our business will depend in significant part on our ability to in-license or acquire the rights to approved products, additional product candidates or technologies. However, we may be unable to in-license or acquire the rights to any such products, product candidates or technologies from third parties. The in-licensing and acquisition of pharmaceutical products is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire products, product candidates or technologies that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to in-license or acquire the rights to the relevant product, product candidate or technology on terms that would allow us to make an appropriate return on our investment. Furthermore, we may be unable to identify suitable products, product candidates or technologies within our area of focus. If we are unable to successfully obtain rights to suitable products, product candidates or technologies, our business, financial condition and prospects for growth could suffer.

Product liability lawsuits against us could divert our resources, cause us to incur substantial liabilities and limit commercialization of any products that we may develop or in-license.

We face an inherent risk of product liability exposure related to the testing of Fovista and any other product candidate that we develop in human clinical trials and will face an even greater risk if we commercially sell any products that we develop or in-license. Because our Phase 3 clinical program for Fovista involves the administration of Fovista in combination with anti-VEGF drugs, including off-label use by intravitreal injection of Avastin provided by us, we also face an inherent risk of product liability exposure related to the testing of such anti-VEGF drugs. If we cannot successfully defend ourselves against claims that our product candidates, co-administered anti-VEGF drugs or our products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

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

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

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

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Risks Related to Our Dependence on Third Parties

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

If Fovista receives marketing approval, we plan to commercialize it in the United States with our own focused, specialty sales force targeting retinal specialists. In addition, we expect to utilize a variety of types of collaboration, distribution and other marketing arrangements with third parties to commercialize Fovista in markets outside the United States. We also may seek third-party collaborators for development and commercialization of other product candidates. 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. We are not currently party to any such arrangement. However, if we do enter into any such arrangements with any third parties in the future, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators abilities and efforts to successfully perform the functions assigned to them in these arrangements.

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

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

- collaborators may deemphasize or not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators strategic focus, product and product candidate priorities or available funding;

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

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

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

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

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

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

- 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, the foregoing risks would be heightened, and the business combination may divert attention or resources or create competing priorities. The collaborator may delay or terminate our product development or commercialization program. If one of our collaborators terminates its agreement with

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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.

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

The potential commercialization of Fovista and the development and potential commercialization of other product candidates will require substantial additional cash to fund expenses. For some of our product candidates, we may decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates. For example, we intend to seek to commercialize Fovista through a variety of types of collaboration arrangements outside the United States.

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

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

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

We have relied on third-party clinical research organizations, or CROs, in conducting our completed Phase 2b clinical trial of Fovista and our completed Phase 1/2a clinical trial of ARC1905. We expect to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions and clinical investigators, in conducting our clinical trials for Fovista, including the clinical trials in our Phase 3 clinical program, and expect to rely on these third parties to conduct clinical trials of any other product candidate that we develop. We or these

third parties may terminate their engagements with us at any time for a variety of reasons, including a failure to perform by the third parties. If we need to enter into alternative arrangements, that would delay our product development activities.

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

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If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors.

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

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

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

We currently rely exclusively on one third-party manufacturer to supply us with Fovista drug substance on a purchase order basis. We also rely on another third-party manufacturer to conduct fill-finish services on a purchase order basis. We do not currently have any contractual commitments for commercial supply of bulk drug substance for Fovista or for fill-finish services. We also do not currently have arrangements in place for redundant supply or a second source for bulk drug substance for Fovista or for fill-finish services. The prices at which we are able to obtain supplies of Fovista drug substance and fill-finish services may vary substantially over time and adversely affect our financial results. Furthermore, we currently rely on sole-source suppliers of certain raw materials and other specialized components of production used in the manufacture and fill-finish of Fovista.

We currently rely exclusively on Nektar to supply us with a proprietary polyethylene glycol, or PEG, reagent under a supply agreement with Nektar. PEG reagent is a chemical we use to modify the chemically synthesized aptamer in Fovista. The PEG reagent made by Nektar is proprietary to Nektar and, to our knowledge, is not currently available from any other third party.

If our third-party manufacturer for Fovista drug substance fails to fulfill our purchase orders, if Nektar breaches its obligations to us under our supply agreement or if either of these manufacturers should become unavailable to us for any reason, we believe that there are a limited number of potential replacement manufacturers, and we likely would incur added costs and delays in identifying or qualifying such replacements. We could also incur additional costs and delays in identifying or qualifying a replacement manufacturer for fill-finish services if our existing third-party manufacturer should become unavailable for any reason. We may be unable to establish any agreements with such replacement manufacturers or to do so on acceptable terms.

Under the supply agreement with Nektar, we must purchase our entire requirements for PEG reagent exclusively from Nektar at an agreed price. In the event Nektar breaches its supply obligations as specified in the agreement, Nektar has agreed to enable a third-party manufacturer, if one is available, to supply us with PEG reagent until Nektar demonstrates that Nektar has the ability to supply all of our requirements for PEG reagent. The agreement of Nektar to enable a third-party manufacturer may be difficult to enforce in the context of a breach by Nektar of its supply obligations. We may not be able to reach an agreement with any third-party manufacturer to take on the supply of PEG reagent under such circumstances because, to our knowledge, no third party currently manufactures the PEG reagent we currently use in making the Fovista drug substance. Furthermore, the third party s right to supply us with PEG reagent would be subject to termination at any

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time once Nektar demonstrates that Nektar has the ability to supply all of our requirements for PEG reagent, which may limit the interest of potential third-party manufacturers in undertaking such an engagement. In addition, the process of transferring any necessary technology or process to a third-party manufacturer would entail significant delay in or disruption to the supply of PEG reagent and, as a result, a significant delay in or disruption to the manufacture of Fovista. Furthermore, the FDA or other regulatory authorities might require additional studies to demonstrate equivalence between the Fovista drug substance made using the Nektar PEG reagent and the Fovista drug substance made using any replacement PEG reagent we propose to use or between the Nektar PEG reagent itself and any replacement PEG reagent we propose to use to make Fovista. We ultimately may be unable to demonstrate such equivalence.

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

- Fovista and any other product that we develop may compete with other product candidates and products for access to a limited number of suitable manufacturing facilities that operate under current good manufacturing practices, or cGMP, regulations;

- reliance on the third party for regulatory compliance and quality assurance;

- the possible breach of the manufacturing agreement by the third party;

- the possible misappropriation of our proprietary information, including our trade secrets and know-how; and

- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products and harm our business and results of operations.

We depend on licenses and sublicenses for development and commercialization rights to our products, product candidates and technologies. Termination of these rights or the failure to comply with obligations under these or other agreements under which we obtain such rights could materially harm our business and prevent us from developing or commercializing our products and product candidates.

We are party to various agreements, including an acquisition agreement with OSI Pharmaceuticals and license agreements with Archemix and Nektar that we depend on for rights to Fovista and other product candidates and technology. These agreements impose, and we may enter into

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

We also have diligence and development obligations under our acquisition agreement with OSI Pharmaceuticals and our license agreements with Archemix and Nektar. Generally, these diligence obligations require us to use commercially reasonable efforts to develop, seek regulatory approval for and commercialize our products in the United States, the European Union and, in some cases, certain other specified countries. If we fail to comply with our obligations under current or future acquisition, license and funding agreements, or otherwise breach an acquisition or license agreement, 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 failed 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 reinstated agreements with less favorable terms, seek alternative sources of financing or cause us to lose our rights

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under these agreements, including our rights to Fovista and other important intellectual property or technology. Any of the foregoing could prevent us from commercializing Fovista or our other product candidates, which could have a material adverse effect on our operating results and overall financial condition.

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

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

- Under the terms of the amended license, manufacturing and supply agreement with Nektar, pursuant to which we obtained, among other licenses, an exclusive, worldwide license to make, develop, use, import, offer for sale and sell certain products that incorporate a specified PEG reagent linked with the active ingredient in Fovista, if we fail to use commercially reasonable efforts to achieve the first commercial sale of Fovista in the United States or one of a specified group of other countries by December 31, 2017, which date Nektar and we may agree in good faith to extend in specified circumstances, Nektar may either terminate our license or convert our license for such country to a non-exclusive license. In addition, if we fail to use commercially reasonable efforts to develop Fovista and file and seek approval of NDAs on a schedule permitting us to make first commercial sales of Fovista in specified countries by December 31, 2017, do not make such first commercial sales of Fovista by such date, or thereafter fail to use commercially reasonable efforts to continue to commercialize and market Fovista in such countries, we will be in material breach of the agreement and Nektar will have the right to terminate the agreement.

In addition to the above risks, certain of our intellectual property rights are sublicenses under intellectual property owned by third parties, in some cases through multiple tiers. The actions of our licensors may therefore affect our rights to use our sublicensed intellectual property, even if we are in compliance with all of the obligations under our license agreements. For example, the licenses from Archemix include sublicenses to us of rights to specified technology, which we refer to as the SELEX technology, licensed by University License Equity Holdings, Inc. to Gilead Sciences, Inc., or Gilead, and sublicensed by Gilead to Archemix, as well as other technology owned by Gilead and licensed to Archemix. In addition, the licenses we have obtained from Nektar include sublicenses of certain rights. Should our licensors or any of the upstream licensors fail to comply with their obligations under the agreements pursuant to which they obtain the rights that are sublicensed to us, or should such agreements be terminated or amended, our ability to develop and commercialize Fovista, ARC1905 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 upstream licensors, which may not be forthcoming. Our business could be adversely affected if we are unable to prosecute, maintain and enforce our licensed and sublicensed intellectual property effectively.

Risks Related to Our Intellectual Property

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

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

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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. 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 right to develop and commercialize any of our products that are the subject of such licensed rights could be adversely affected.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign jurisdictions may not protect our rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions. Moreover, the United States Patent and Trademark Office might require that the term of a patent issuing from a pending patent application be disclaimed and limited to the term of another patent that is commonly owned or names a common inventor. As a result, the issuance, scope, validity, term, enforceability and commercial value of our patent rights are highly uncertain.

Our 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 ability to generate additional preclinical or clinical data that support the patentability of our proposed claims. We 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 patents or narrow the scope of our patent protection.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art, may affect patent litigation and switch the U.S. patent system from a first-to-invent system to a first-to-file system. Under a first-to-file system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to the patent on an invention regardless of whether another inventor had made the invention earlier. The U.S. Patent and Trademark Office recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first-to-file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

Moreover, we may be subject to a third-party preissuance submission of prior art to the U.S. Patent and Trademark Office, or become involved in opposition, derivation, reexamination, *inter partes* review, post-grant review, interference proceedings or other patent office proceedings or litigation, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights; allow third parties to commercialize our technology or products and compete directly with us, without payment to us; or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is

threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

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If we are unable to obtain and maintain patent protection for our technology and products during the period of their commercialization, or if the scope of the patent protection is not sufficiently broad, our competitors could develop and commercialize technology and products similar or identical to ours, and our ability to successfully commercialize our technology and products may be adversely affected.

The last to expire of the U.S. patent rights covering the composition of matter of Fovista is expected to expire in 2017. Such expiration date is not long after the date by which we expect Fovista to be commercialized in the United States if we obtain marketing approval and may even be prior to such date. We own an issued U.S. patent covering a method of treating wet AMD with Fovista in combination with Avastin or Lucentis, which is expected to expire in 2024. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, permits a patent restoration term of up to five years as compensation for patent term effectively lost during product development and the FDA regulatory review process occurring after the issuance of a patent. We may be able to obtain a patent term extension for one of these U.S. patents. However, we may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration or the term or scope of any such extension is less than we request, any period during which we have the right to exclusively market our product will be shorter than we would otherwise expect, and our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced, possibly materially.

The European patent rights covering the composition of matter of Fovista are expected to expire in 2018. Such expiration date is shortly after the date by which we expect Fovista to be commercialized in Europe, and may even be prior to such date. We own a granted European patent covering a combination of Fovista and Lucentis or Avastin for use in a method for treating wet AMD. This European patent is expected to expire in 2024.

We also have filed in the United States patent applications covering a method of treating wet AMD in patients with Fovista in combination with Eylea and in Europe a patent application covering a combination of Fovista and Eylea for use in a method for treating wet AMD. These patent applications are in the early stages of prosecution and may not result in patents being issued which protect the use of Fovista in combination with Eylea for treating wet AMD or effectively prevent others from commercializing competitive technologies and products. If a patent is granted following prosecution of any such application, that patent would be expected to expire in 2030.

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

The Hatch-Waxman act also permits the manufacture, use, offer for sale, sale or importation of a patented invention other than a new animal drug or veterinary biological product, if the manufacture, use, offer for sale, sale or importation is solely for uses that are reasonably related to development of information that could be submitted to the FDA. For this reason, our competitors might be able under certain circumstances to perform activities within the scope of the U.S. patents that we own or under which we are licensed without infringing such patents. This might enable our competitors to develop during the lifetime of these patents drugs that compete with Fovista or ARC1905, if approved.

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The U.S. patent rights covering ARC1905 as a composition of matter are expected to expire in 2025. Such expiration date may be prior to the date by which we would be able to commercialize ARC1905 in the United States if we seek and obtain marketing approval. The U.S. patent rights covering methods of treating certain complement protein mediated disorders with ARC1905 are expected to expire in 2026. As a result, if we obtain marketing approval for ARC1905, we may not be able to exclude competitors from commercializing products similar or identical to ours if such competitors do not use our claimed methods of treatment. Depending on potential delays in the regulatory review process for ARC1905, we may be able to obtain a patent term extension for one of these patents in the United States, but we can provide no assurances that such an extension will be obtained.

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

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

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

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

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

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and products and use our proprietary technologies without infringing or otherwise violating the intellectual

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property and other proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference, derivation, re-examination, post-grant review, opposition or similar proceedings before the U.S. Patent and Trademark Office or its foreign counterparts. The risks of being involved in such litigation and proceedings may also increase as our product candidates near commercialization and as we gain the greater visibility associated with being a public company. Third parties may assert infringement claims against us based on existing or future intellectual property rights. We may not be aware of all such intellectual property rights potentially relating to our product candidates and their uses. Thus, we do not know with certainty that Fovista or any other product candidate, or our intended commercialization thereof, does not and will not infringe or otherwise violate any third party s intellectual property.

If we are found to infringe or otherwise violate a third party s intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us and could require us to make substantial licensing and royalty payments. We 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 from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could expose us to similar liabilities and have a similar negative impact on our business.

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

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

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact conceives or develops intellectual property that we regard as our own. Moreover, because we acquired rights to Fovista from Eyetech, Archemix and Nektar, we must rely on these parties practices, and those of their predecessors, with regard to the assignment of intellectual property therein. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

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

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

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

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financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

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

In addition to seeking patents for some of our technology and products, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. We cannot guarantee that we have executed such agreements with each party that may have or have had access to our trade secrets. Moreover, because we acquired certain rights to Fovista from Eyetech, Archemix and Nektar, we must rely on these parties practices, and those of their predecessors, with regard to the protection of Fovista-related trade secrets before we acquired them. Any party with whom we or they have executed a non-disclosure and confidentiality agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Our proprietary information may also be obtained by third parties by other means, such as breaches of our physical or computer security systems.

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

Risks Related to Regulatory Approval and Other Legal Compliance Matters

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

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

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

The FDA or other regulatory authorities may determine that Fovista or any other product candidate that we develop is not effective, is only moderately effective or has undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use. The FDA or other regulatory authority may limit the approval of Fovista to use with only specified anti-VEGF drugs rather than with all anti-VEGF drugs. Such limitation could limit sales of Fovista.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and

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require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

Marketing approval of novel product candidates such as Fovista and ARC1905 manufactured using novel manufacturing processes can be more expensive and take longer than for other, more well-known or extensively studied pharmaceutical or biopharmaceutical products, due to regulatory agencies lack of experience with them. We believe that the FDA has only granted marketing approval for one aptamer product to date. This lack of experience may lengthen the regulatory review process, require us to conduct additional studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post-approval limitations or restrictions.

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

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

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

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

In order to market and sell Fovista and any other product candidate that we develop in the European Union and many other jurisdictions, we or our third-party collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We or these third parties 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 may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market.

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

Any product candidate, including Fovista, for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to manufacturing, quality control, quality assurance, complaints and corresponding maintenance of records and documents,

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requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or may be subject to significant conditions of approval.

The FDA may also impose requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the product, including the adoption and implementation of risk evaluation and mitigation strategies. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling and regulatory requirements. The FDA imposes stringent restrictions on manufacturers communications regarding off-label use and if we do not restrict the marketing of our products only to their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws.

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

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions and warnings in the labeling and marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;

- fines, restitution or disgorgement of profits or revenue;

- suspension or withdrawal of marketing approvals;

- refusal to permit the import or export of our products;

- product seizure; or

- injunctions or the imposition of civil or criminal penalties.

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

Our 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 to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of any product candidates, including Fovista, for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us 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 market, sell and distribute any products for which we 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

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return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;

- the federal False Claims Act imposes criminal and civil penalties, including civil whistleblower or *qui tam* actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

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

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

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

- the federal transparency requirements under the Health Care Reform Law and analogous state laws require manufacturers of drugs, devices, biologics and medical supplies to report information related to payments and other transfers of value to physicians and teaching hospitals and physician ownership and investment interests; and

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

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

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we 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 operations. If any of the physicians or other providers or entities with whom we expect

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

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

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of Fovista or any other product candidate that we develop, restrict or regulate post-approval activities and affect our ability to generate revenue from, sell profitably or commercialize any product candidates, including Fovista, for which we obtain marketing approval or products that we may develop or in-license. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or MMA, changed the way Medicare covers and pays for pharmaceutical products and could decrease the coverage and price that we receive for any approved products. While the MMA applies only to drug benefits for Medicare beneficiaries, private payors

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often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors.

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

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices;
- extension of manufacturers Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements to report financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and

- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since PPACA was enacted. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding.

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

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

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

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

We and our third-party manufacturers are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and produce hazardous waste products. We cannot eliminate the risk of

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

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

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

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

Risks Related to Employee Matters and Managing Growth

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

We are highly dependent on David R. Guyer, M.D., our Chief Executive Officer, Samir Patel, M.D., our President, and Bruce Peacock, our Chief Financial and Business Officer, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into employment agreements with our executive officers, each of them may terminate their employment with us at any time. We do not maintain key person insurance for any of our executives or other employees.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain marketing approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms, if at all, given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

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

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of clinical development, regulatory affairs and sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to manage effectively the expansion of our operations or recruit and train additional qualified personnel. The expansion

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of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Risks Related to Our Common Stock

Our executive officers, directors and principal stockholders have the ability to control all matters submitted to stockholders for approval.

As of November 8, 2013, our executive officers, directors and principal stockholders and their affiliates, in the aggregate, beneficially owned shares representing approximately 65.8% of our capital stock. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire.

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.

An active trading market for our common stock may not be sustained.

Our shares of common stock began trading on the NASDAQ Global Select Market on September 25, 2013. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price of our common stock and thereby affect the ability of our stockholders to sell their shares.

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

Our stock price may be volatile. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their common stock at or above the price at which they purchased it. The market price for our common stock may be influenced by many factors, including:

- the success of competitive products or technologies;
- results of clinical trials of Fovista and any other product candidate that we develop;
- results of clinical trials of product candidates of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to in-license or acquire the rights to other products, product candidates and technologies for the treatment of ophthalmic diseases, the costs of commercializing any such products and the costs of development of any such product candidates or technologies;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;

- variations in our financial results or those of companies that are perceived to be similar to us;

- changes in the structure of healthcare payment systems;

- market conditions in the pharmaceutical and biotechnology sectors;

- general economic, industry and market conditions; and

- the other factors described in this Risk Factors section.

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

A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.

While a significant portion of our total outstanding shares are restricted from immediate resale, they may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. As of November 8, 2013, we had outstanding 31,373,489 shares of common stock. Of these shares, 22,532,241 shares are restricted securities under Rule 144 under the Securities Act and substantially all of which are subject to lock-up agreements entered into in connection with our initial public offering but will be able to be sold after the expiration of the applicable lock-up period, subject to volume, notice and manner of sale restrictions in the case of our affiliates. The remaining shares, including shares sold in our initial public offering, may be resold in the public market

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without restriction unless purchased by our affiliates. Moreover, holders of an aggregate of 20,631,329 shares of our common stock, including shares issuable pursuant to outstanding warrants, have rights, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders, subject to waiver or expiration of the applicable lock-up agreements. In October 2013, we registered all shares of common stock that we may issue under our equity compensation plans. These shares can be freely sold in the public market upon issuance, subject to volume, notice and manner of sale limitations applicable to affiliates and the applicable lock-up agreements entered into in connection with our initial public offering.

We are an emerging growth company, and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company for up to five years. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced Management s Discussion and Analysis of Financial Condition and Results of Operations disclosure;

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;

- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor s report providing additional information about the audit and the financial statements;

- reduced disclosure obligations regarding executive compensation; and

- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

We expect to continue to take advantage of some or all of the available exemptions. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act also provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have elected to delay such adoption of new or revised accounting standards, and, as a result, we may not comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for public companies that are not emerging growth companies. As a result of such election, our financial statements may not be comparable to the financial statements of other public companies.

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

As a public company, and particularly after we are no longer an emerging growth company, we incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The NASDAQ Global Select Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations have increased our legal and financial compliance costs and will make some activities more time-consuming and costly. We currently estimate that we will incur incremental annual costs, including costs for additional personnel, of approximately $2.0 million associated with operating as a public company, although it is possible that our actual incremental annual costs will be higher than we currently estimate.

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For as long as we remain an emerging growth company, we may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies as described in the preceding risk factor. We may remain an emerging growth company until the end of the 2018 fiscal year, although if the market value of our common stock that is held by non-affiliates exceeds $700 million as of any June 30 before that time or if we have annual gross revenues of $1 billion or more in any fiscal year, we would cease to be an emerging growth company as of December 31 of the applicable year. We also would cease to be an emerging growth company if we issue more than $1 billion of non-convertible debt over a three-year period.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we will be required to furnish a report by our management on our internal control over financial reporting. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe, or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

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

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

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

Recent Sales of Unregistered Securities

Set forth below is information regarding securities sold or issued by us during the three months ended September 30, 2013, that were not registered under the Securities Act of 1933, as amended, or the Securities Act. Also included is the consideration, if any, received by us for the securities and information relating to the section of the Securities Act, or rule of the Securities and Exchange Commission, or SEC, under which exemption from registration was claimed.

Issuances of securities

In August 2013, we issued and sold an aggregate of 13,333,333 shares of our series C preferred stock, at a price per share of $2.50, for an aggregate purchase price of $33.3 million. No underwriters were involved in this issuance of securities, which were issued to accredited investors in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(2) under the Securities Act, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. The recipients of securities in the this issuance represented that they were accredited investors and were acquiring the securities for their own account for investment purposes only and not with a view to, or for sale in connection with, any distribution thereof and that they could bear the risks of the investment and could hold the securities for an indefinite period of time and appropriate legends were affixed to the instruments representing such securities issued in such transactions.

Stock options and other equity awards

During the three months ended September 30, 2013, we issued to certain employees, directors and consultants options to purchase an aggregate of 472,024 shares of our common stock at a weighted-average exercise price of $13.42 per share. During the three months ended September 30, 2013, options to purchase an aggregate of 2,542 shares of our common stock were exercised. The stock options and shares of restricted stock issued upon the exercise of stock options described above were issued pursuant to written compensatory plans or arrangements with our employees, directors and consultants, in reliance on the exemption from the registration requirements of the Securities Act provided by Rule 701 promulgated under the Securities Act or the exemption set forth in Section 4(2) under the Securities Act and Regulation D promulgated

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thereunder relative to transactions by an issuer not involving any public offering. All recipients either received adequate information about us or had access, through employment or other relationships, to such information.

Purchase of Equity Securities

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

Use of Proceeds from Registered Securities

On September 30, 2013, we closed our initial public offering of 8,740,000 shares of our common stock, including 1,140,000 shares of our common stock pursuant to the exercise by the underwriters of an over-allotment option, at a public offering price of $22.00 per share for an aggregate offering price of approximately $192.3 million. The offer and sale of all of the shares in the offering were registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-190643), which was declared effective by the SEC on September 24, 2013. Morgan Stanley & Co. LLC and J.P. Morgan Securities LLC acted as joint book-running managers for the offering and as representatives of the underwriters. Leerink Swann LLC and Stifel, Nicolaus & Company, Incorporated acted as the co-managers for the offering. The offering commenced on September 24, 2013 and did not terminate until the sale of all of the shares offered.

We received aggregate net proceeds from the offering of $175.6 million, after deducting underwriting discounts and commissions and other offering expenses payable by us. None of the underwriting discounts and commissions or other offering expenses were incurred or paid to directors or officers of ours or their associates or to persons owning 10 percent or more of our common stock or to any affiliates of ours.

We have invested the net proceeds from the offering in a variety of capital preservation investments, including short-term, investment grade, interest bearing instruments and U.S. government securities. There has been no material change in our planned use of the net proceeds from the offering as described in our final prospectus filed with the SEC pursuant to Rule 424(b) under the Securities Act.

Item 6. Exhibits.

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

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SIGNATURES

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

OPHTHOTECH CORPORATION

Date: November 13, 2013

By: /s/ Bruce A. Peacock
Bruce A. Peacock
Chief Financial and Business Officer
(Principal Financial and Accounting Officer)

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EXHIBIT INDEX

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

* Submitted electronically herewith.

Attached as Exhibit 101 to this report are the following formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheet at December 31, 2012 and September 30, 2013 (unaudited), (ii) Statement of Operations (unaudited) for the three month period ended September 30, 2013 and 2012, the nine month period ended September 30, 2013 and 2012 and for the period from inception (January 5, 2007) through September 30, 2013, (iii) Statement of Cash Flows (unaudited) for the nine month period ended September 30, 2013 and 2012 and for the period from inception (January 5, 2007) through September 30, 2013 and (iv) Notes to Financial Statements (unaudited).

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