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

**FORM 10-Q**

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

For the quarterly period ended March 31, 2016

OR

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_  
Commission File Number: 333-88480

**OHR PHARMACEUTICAL, INC.**

(Exact name of registrant as specified in its charter)

**Delaware**

(State or other jurisdiction of incorporation or  
organization)

**46-5622433**

(I.R.S. Employer Identification No.)

**800 Third Avenue, 11th Floor  
New York, NY 10022**

(Address of principal executive offices)  
**(212) 682-8452**

(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  No

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this Chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files).

Yes  No

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	<input type="checkbox"/>	Accelerated filer	<input checked="" type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>

Do not check if smaller reporting company

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act).

Yes  No

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date: 31,496,869 shares of common stock, par value \$0.0001 per share, outstanding as of May 8, 2016.

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OHR PHARMACEUTICAL, INC.  
TABLE OF CONTENTS

	Page
<u>PART I</u>	
<u>Item 1</u>	3
<u>Item 2</u>	3
<u>Item 3</u>	11
<u>Item 4</u>	18
<u>PART II</u>	
<u>Item 1</u>	18
<u>Item 1A</u>	19
<u>Item 2</u>	19
<u>Item 3</u>	33
<u>Item 4</u>	34
<u>Item 5</u>	34
<u>Item 6</u>	34

Part 1 FINANCIAL INFORMATION

Item 1. Financial Statements.

TABLE OF CONTENTS

PAGE

<a href="#"><u>Unaudited Consolidated Balance Sheets as of March 31, 2016 and September 30, 2015</u></a>	4
<a href="#"><u>Unaudited Consolidated Statements of Operations for the three and six months ended March 31, 2016 and 2015</u></a>	5
<a href="#"><u>Unaudited Consolidated Statements of Cash Flows for the three and six months ended March 31, 2016 and 2015</u></a>	6
<a href="#"><u>Notes to Unaudited Consolidated Financial Statements</u></a>	7

**OHR PHARMACEUTICAL, INC.**  
**Consolidated Balance Sheets**  
**(Unaudited)**

	<u>March 31,</u> <u>2016</u>	<u>September 30,</u> <u>2015</u>
<u>ASSETS</u>		
<b>CURRENT ASSETS</b>		
Cash	\$ 21,881,370	\$ 28,697,323
Prepaid expenses and other current assets	456,514	338,713
<b>Total Current Assets</b>	<u>22,337,884</u>	<u>29,036,036</u>
<b>EQUIPMENT, net</b>	<u>229,363</u>	<u>248,753</u>
<b>OTHER ASSETS</b>		
Security deposit	12,243	12,243
Intangible assets, net	15,770,541	16,332,863
Goodwill	740,912	740,912
<b>TOTAL ASSETS</b>	<u>\$ 39,090,943</u>	<u>\$ 46,370,807</u>
<u>LIABILITIES AND STOCKHOLDERS' EQUITY</u>		
<b>CURRENT LIABILITIES</b>		
Accounts payable and accrued expenses	\$ 1,869,265	\$ 1,592,348
Notes payable	215,810	48,063
Contingent consideration	1,430,393	2,239,603
<b>Total Current Liabilities</b>	<u>3,515,468</u>	<u>3,880,014</u>
<b>TOTAL LIABILITIES</b>	<u>3,515,468</u>	<u>3,880,014</u>
<b>STOCKHOLDERS' EQUITY</b>		
Preferred stock, Series B; 6,000,000 shares authorized, \$0.0001 par value, 0 shares issued and outstanding, respectively	—	—
Common stock; 180,000,000 shares authorized, \$0.0001 par value, 31,496,869 and 30,331,309 shares issued and outstanding, respectively	3,150	3,033
Additional paid-in capital	105,514,059	100,999,173
Accumulated deficit	(69,941,734)	(58,511,413)
<b>Total Stockholders' Equity</b>	<u>35,575,475</u>	<u>42,490,793</u>
<b>TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY</b>	<u>\$ 39,090,943</u>	<u>\$ 46,370,807</u>

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

**OHR PHARMACEUTICAL, INC.**  
**Consolidated Statements of Operations**  
(Unaudited)

	For the Three Months Ended March 31,		For the Six Months Ended March 31,	
	2016	2015	2016	2015
<b>OPERATING (INCOME) EXPENSES</b>				
General and administrative	\$ 2,966,363	\$ 3,334,971	\$ 4,184,492	\$ 4,151,834
Research and development	4,043,859	3,175,855	6,120,139	5,919,977
Depreciation and amortization	296,077	295,262	593,816	600,884
Gain on settlement of accounts payable	(710,264)	—	(710,264)	—
<b>OPERATING LOSS</b>	<b>6,596,035</b>	<b>6,806,088</b>	<b>10,188,183</b>	<b>10,672,695</b>
<b>OTHER INCOME (EXPENSE)</b>				
Change in fair value of contingent consideration	1,305,623	3,786,193	(1,251,926)	3,102,807
Share in losses on investment in joint venture	—	(41,211)	—	(67,861)
Other income	—	—	3,419	35,813
Interest income (expense), net	5,553	(536)	6,369	(663)
Impairment of intangibles	—	(338,906)	—	(338,906)
<b>Total Other Income (Expense)</b>	<b>1,311,176</b>	<b>3,405,540</b>	<b>(1,242,138)</b>	<b>2,731,190</b>
<b>LOSS FROM OPERATIONS BEFORE</b>				
<b>INCOME TAXES</b>	<b>(5,284,859)</b>	<b>(3,400,548)</b>	<b>(11,430,321)</b>	<b>(7,941,505)</b>
<b>PROVISION FOR INCOME TAXES</b>	<b>—</b>	<b>—</b>	<b>—</b>	<b>—</b>
<b>NET LOSS</b>	<b>\$ (5,284,859)</b>	<b>\$ (3,400,548)</b>	<b>\$ (11,430,321)</b>	<b>\$ (7,941,505)</b>
<b>BASIC AND DILUTED LOSS PER SHARE</b>	<b>\$ (0.17)</b>	<b>\$ (0.12)</b>	<b>\$ (0.37)</b>	<b>\$ (0.30)</b>
<b>WEIGHTED AVERAGE NUMBER OF SHARES</b>				
<b>OUTSTANDING:</b>				
<b>BASIC AND DILUTED</b>	<b>31,344,424</b>	<b>27,713,410</b>	<b>30,906,114</b>	<b>26,479,538</b>

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

**OHR PHARMACEUTICAL, INC.**  
**Consolidated Statements of Cash Flows**  
**(Unaudited)**

	<b>For the Six Months Ended March 31,</b>	
	<b>2016</b>	<b>2015</b>
<b>OPERATING ACTIVITIES</b>		
Net loss	\$ (11,430,321)	\$ (7,941,505)
Adjustments to reconcile net loss to net cash used by operating activities:		
Common stock issued for services	715,952	353,876
Warrants issued for services	—	8,559
Stock option expense	1,724,375	2,269,785
Change in fair value of contingent consideration	1,251,926	(3,102,807)
Share in losses on investment in joint venture	—	67,861
Depreciation	31,494	24,866
Amortization of intangible assets	562,322	576,018
Impairment of Intangibles	—	338,906
Gain on settlement of accounts payable	(710,264)	—
Changes in operating assets and liabilities		
Prepaid expenses and deposits	98,009	(419,991)
Accounts payable and accrued expenses	987,180	1,018,156
	<u>(6,769,327)</u>	<u>(6,806,276)</u>
Net Cash Used in Operating Activities	(6,769,327)	(6,806,276)
<b>INVESTING ACTIVITIES</b>		
Investment in joint venture	—	(100,000)
Purchase of property and equipment	(12,103)	(86,891)
Net Cash Used in Investing Activities	<u>(12,103)</u>	<u>(186,891)</u>
<b>FINANCING ACTIVITIES</b>		
Proceeds for issuance of common stock for cash	—	26,582,998
Proceeds from warrants exercised for cash	13,540	80,005
Repayments of short-term notes payable	(48,063)	(67,068)
Net Cash Provided by (Used in) Financing Activities	<u>(34,523)</u>	<u>26,595,935</u>
NET CHANGE IN CASH	(6,815,953)	19,602,768
CASH AT BEGINNING OF PERIOD	<u>28,697,323</u>	<u>13,220,494</u>
CASH AT END OF PERIOD	<u>\$ 21,881,370</u>	<u>\$ 32,823,262</u>
<b>SUPPLEMENTAL DISCLOSURES OF CASH FLOW INFORMATION CASH PAID FOR:</b>		
Interest	\$ —	\$ 1,533
Income Taxes	—	—
<b>NON CASH FINANCING ACTIVITIES:</b>		
Settlement of contingent consideration	\$ 2,061,136	\$ —
Financing of insurance premiums through issuance of short term notes	215,810	212,400
Common stock issued to settle accounts payable	—	50,000

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

**OHR PHARMACEUTICAL, INC.**  
**Notes to Unaudited Consolidated Financial Statements**  
**March 31, 2016**

**NOTE 1 - BASIS OF PRESENTATION**

The accompanying unaudited condensed consolidated financial statements include the accounts of Ohr Pharmaceutical, Inc. and its subsidiaries (the "Company"). The accompanying unaudited consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States ("GAAP") for interim financial information and with the instructions to Form 10-Q and Rule 10-01 of Regulation S-X related to interim period financial statements. Accordingly, these condensed consolidated financial statements do not include certain information and footnotes required by GAAP for complete financial statements. However, in the opinion of management, all adjustments (which include only normal recurring adjustments) necessary to present fairly the financial position, results of operations, and cash flows at March 31, 2016, and for all periods presented herein, have been made.

It is suggested that these unaudited consolidated financial statements be read in conjunction with the consolidated financial statements and notes thereto included in the Company's Annual Report on Form 10-K for the fiscal year ended September 30, 2015. The results of operations for the quarterly periods ended March 31, 2016 and 2015 are not necessarily indicative of the operating results for the full years. Reclassification has been made to conform to current period presentation which included moving applicable stock option expense from general and administrative expense to research and development expense.

**NOTE 2 - SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES**

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities at the date of the financial statements and the reported amounts of revenue and expenses during the reporting period. Actual results could differ from those estimates. Estimates subject to change in the near term include impairment (if any) of long-lived assets and fair value of derivative liabilities.

Fair Value of Financial Instruments

In accordance with ASC 820, the carrying value of cash and cash equivalents, accounts receivable, accounts payable and notes payable approximates fair value due to the short-term maturity of these instruments. ASC 820 clarifies the definition of fair value, prescribes methods for measuring fair value, and establishes a fair value hierarchy to classify the inputs used in measuring fair value as follows:

Level 1 - Inputs are unadjusted quoted prices in active markets for identical assets or liabilities available at the measurement date.

Level 2 - Inputs are unadjusted quoted prices for similar assets and liabilities in active markets, quoted prices for identical or similar assets and liabilities in markets that are not active, inputs other than quoted prices that are observable, and inputs derived from or corroborated by observable market data.

Level 3 - Unobservable inputs, where there is little or no market activity for the asset or liability. These inputs reflect the reporting entity's own beliefs about the assumptions that market participants would use in pricing the asset or liability, based on the best information available in the circumstances.

The following table presents assets and liabilities that are measured and recognized at fair value as of March 31, 2016 and September 30, 2015, on a recurring basis:

Assets and liabilities measured at fair value on a recurring basis at March 31, 2016	Level 1	Level 2	Level 3	Total Carrying Value
Contingent stock consideration	\$ —	\$ —	\$ 1,430,393	\$ 1,430,393
	\$ —	\$ —	\$ 1,430,393	\$ 1,430,393

Assets and liabilities measured at fair value on a recurring basis at September 30, 2015	Level 1	Level 2	Level 3	Total Carrying Value
Contingent stock consideration	\$ —	\$ —	2,239,603	\$ 2,239,603
	\$ —	\$ —	2,239,603	\$ 2,239,603

The following table provides a summary of the changes in fair value, including net transfers in and/or out, of the financial instruments, measured at fair value on a recurring basis using significant unobservable inputs:

	Contingent Stock Consideration
Level 3 Reconciliation:	
Level 3 assets and liabilities at September 30, 2015	\$ 2,239,603
Purchases, sales, issuances and settlements (net)	(2,061,136)
Mark to market adjustments	1,251,926
Total Level 3 assets and liabilities at March 31, 2016	<u>\$ 1,430,393</u>

#### Recent Accounting Pronouncements

Management has considered all recent accounting pronouncements issued since the last audit of the Company's financial statements. The Company's management believes that these recent pronouncements will not have a material effect on the Company's financial statements.

#### **NOTE 3 - INTANGIBLE ASSETS**

Intangible assets at March 31, 2016 and September 30, 2015:

	<b>March 31, 2016</b>	<b>September 30, 2015</b>
License Rights	\$ 17,712,991	\$ 17,712,991
Patent Costs	200,000	200,000
	17,912,991	17,912,991
Accumulated Amortization	(2,142,450)	(1,580,128)
Total Intangible Assets	<u>\$ 15,770,541</u>	<u>\$ 16,332,863</u>

During the three and six month periods ended March 31, 2016, the Company recognized \$279,625 and \$562,322, respectively, in amortization expense on the patents and license rights. The amortization expense has been included in research and development expense.

#### **NOTE 4 – ACCOUNTS PAYABLE AND ACCRUED EXPENSES**

During the three and six month periods ended March 31, 2016, the Company realized a gain of \$710,264 related to the settlement of an accounts payable balance.

#### **NOTE 5 - NOTES PAYABLE**

On February 28, 2016, the Company entered into a premium financing arrangement for its directors and officers insurance in the amount of \$215,810. The financing arrangement bears interest at 7% per annum. As of March 31, 2016, the Company has not made any payments toward the loan.

#### **NOTE 6 - CAPITAL STOCK**

Below is a table summarizing the capital stock activity during the six months ended March 31, 2016 and the shares outstanding as of March 31, 2016:

	<b>Number Outstanding</b>	<b>Weighted- Average Exercise/Stock Price</b>
Outstanding at September 30, 2015	30,331,309	\$ 3.33
Exercise of Warrants	6,944	1.95
Milestone 1 – SKS Acquisition	497,859	4.14
Restricted Stock Issued	660,757	4.12
Outstanding at March 31, 2016	<u>31,496,869</u>	<u>\$ 3.35</u>

The Board approved the achievement of Milestone 1 in connection with the ophthalmology assets acquisition by the Company of the privately held SKS Ocular LLC and its affiliate, SKS Ocular 1 LLC ("SKS"). As a result, on December 11, 2015, the Company issued 497,859 shares of its common stock to SKS. The value of the stock issued was \$2,061,136, as determined by the \$4.14 per share closing sale price of the Company's common stock on the date of Board approval.

On December 11, 2015, pursuant to a consulting agreement, the Board granted 120,000 shares of the Company's restricted stock under the Company's 2014 Stock Incentive Plan to Dr. Irach Taraporewala, the Company's former Chief Technology Officer. The restricted stock will vest in four equal, semiannual installments, beginning on July 16, 2016, subject to Dr. Taraporewala continuously providing consulting services to the Company from the grant date until each such vesting date.

On December 29, 2015, the Company issued 6,944 shares of common stock pursuant to an exercise of warrants at \$1.95 per share, for gross proceeds of \$13,540.

On January 9, 2016, the Board granted 130,000 and 350,358 shares of the Company's restricted stock to Sam Backenroth, Chief Financial Officer and Dr. Jason Slakter, Chief Executive Officer, respectively, 50% of these shares vest on the January 9, 2017 and the remaining 50% vest on January 9, 2018.

On January 22, 2016 and March 17, 2016, the Board approved the issuance of 25,399 and 35,000 shares, respectively, of the Company's restricted stock to a consultant of the Company for services rendered.

During the six months ended March 31, 2016, the Company recognized compensation expense of \$715,952 for shares issued for services during the current and prior periods.

#### NOTE 7 - COMMON STOCK WARRANTS

Below is a table summarizing the warrants issued and outstanding as of March 31, 2016:

	<b>Number Outstanding</b>	<b>Weighted- Average Exercise Price</b>
Outstanding at September 30, 2015	746,869	\$ 4.75
Exercised	(6,944)	1.95
Forfeited/ Expired	(43,334)	4.89
Outstanding at March 31, 2016	<u>696,591</u>	<u>\$ 4.67</u>

As of March 31, 2016, the outstanding warrants have an intrinsic value of \$184,312.

#### NOTE 8 - COMMON STOCK OPTIONS

During the three and six months ended March 31, 2016, the Company recognized \$1,407,556 and 1,724,375 of expense related to options granted, respectively. Unamortized option expense as of March 31, 2016 for all options outstanding amounted to approximately \$3,764,535.

Below is a table summarizing the options issued and outstanding as of March 31, 2016:

	<b>Number Outstanding</b>	<b>Weighted - Average Exercise Price</b>
Outstanding at September 30, 2015	2,761,001	\$ 7.27
Granted	669,275	4.11
Forfeited/ Expired	(558,333)	6.60
Outstanding at March 31, 2016	<u>2,871,943</u>	<u>\$ 6.66</u>
Exercisable at March 31, 2016	<u>1,681,920</u>	<u>\$ 6.45</u>

As of March 31, 2016, the outstanding options have an intrinsic value of \$500,001.

The fair value of options granted during the six months ended March 31, 2016 are estimated at the date of grant using the Black-Scholes option-pricing model and the following assumptions: stock price at valuation ranging from of \$3.23 to \$5.14, expected term ranging from of 2.75 to 3.25 years, exercise price ranging from \$3.23 to \$5.14, a risk free interest rate ranging from 1.04 to 1.31 percent, a dividend yield of 0 percent and volatility ranging from 103 to 108 percent. The average vesting period of options issued during the six month period ended March 31, 2016 is 10.9 months with 36.3 percent of the options vesting at the date of the grant.

#### NOTE 9 - COMMITMENTS AND CONTINGENCIES

##### Legal Proceedings

The Company may become involved in certain legal proceedings and claims which arise in the normal course of business. If an unfavorable ruling were to occur, there exists the possibility of a material adverse impact on the Company's results of operations, prospects, cash flows, financial position and brand. To the best knowledge of the Company's management, at March 31, 2016, there are no legal proceedings which the Company believes will have a material adverse effect on its business, results of operations, cash flows or financial condition.

#### Contingent Stock Consideration

On May 30, 2014, the Company completed the acquisition of certain assets of SKS, including licenses, patents and contracts relating to micro-fabrication polymer-based sustained delivery platforms related to ocular therapeutics and dry age-related macular degeneration animal models, together with biomarkers to support such models.

The purchase price consisted of: (a) cash in the amount of \$3,500,000; (b) 1,194,862 shares of the Company's common stock (valued at \$10,180,224 based on the closing sale price of the common stock of the Company on May 30, 2014) and (c) an additional 1,493,577 shares that will be issued contingent to achievement of certain milestones. This contingent consideration has been recorded as a liability of the Company and is reviewed by management for probability and likelihood of the milestones being achieved at each reporting period. The liability is adjusted according to management's assessment.

On December 11, 2015, the Board approved the achievement of Milestone 1 and consequently, the Company issued 497,859 shares of its common stock to SKS as partial settlement of the contingent consideration. The value of the stock issued was \$2,061,136, as determined by the \$4.14 per share closing sale price of the Company's common stock on the date of Board approval.

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

Our discussion and analysis of the business and subsequent discussion of financial conditions may contain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Statements that are not historical in nature, including statements about beliefs and expectations, are forward-looking statements. Words such as “may,” “will,” “should,” “estimates,” “predicts,” “believes,” “anticipates,” “plans,” “expects,” “intends” and similar expressions are intended to identify these forward-looking statements, but are not the exclusive means of identifying such statements. Such statements are based on currently available operating, financial and competitive information and are subject to various risks and uncertainties as described in greater detail in Item 1A, Part II, our “Risk Factors” on page 19 of this Report. You are cautioned that these forward-looking statements reflect management’s estimates only as of the date hereof, and we assume no obligation to update these statements, even if new information becomes available or other events occur in the future, except as required by law. Actual future results, events and trends may differ materially from those expressed in or implied by such statements depending on a variety of factors, including, but not limited to those set forth in our filings with the Securities and Exchange Commission (“SEC”). Specifically, and not in limitation of these factors, we may alter our plans, strategies, objectives or business.

### **Company Overview**

Ohr Pharmaceutical, Inc. (“we,” “us,” “our,” “Ohr” or the “Company”) is a pharmaceutical company focused on the development of novel therapeutics and delivery technologies for the treatment of ocular disease. Our development pipeline consists of multiple programs and indications at various stages of development. Our lead clinical program, squalamine lactate ophthalmic solution, 0.2% (“Squalamine”, also known as OHR-102), is a novel therapeutic product which could provide a non-invasive therapy to improve vision outcomes without requiring multiple injections per office visit. We are evaluating Squalamine, given in combination with Lucentis injections, for the treatment of retinal diseases including wet-AMD, retinal vein occlusion and proliferative diabetic retinopathy. Data from our Phase II clinical trial in wet-AMD demonstrated a positive and clinically meaningful treatment effect of Squalamine combination therapy in those patients classified as having classic containing choroidal neovascular lesions (“classic CNV”) as well as in the larger population of patients classified as having occult neovascularization (“occult CNV”) less than 10mm<sup>2</sup>.

Our preclinical stage pipeline is focused on the development of sustained release therapeutics utilizing the platform technology we acquired in May 2014. There are several active programs evaluating molecules and approaches for the treatment of glaucoma, steroid induced glaucoma, ocular allergy, and retinal disease. These programs have been identified by Ohr as large markets with unmet medical needs or where sustained release therapeutics can greatly improve upon the way ocular disease is currently being treated, including increasing compliance rates and reducing treatment burden.

### **Corporate and Historical Information**

The Company is a Delaware corporation that was organized on August 4, 2009, as successor to BBM Holdings, Inc. (formerly Prime Resource, Inc., which was organized March 29, 2002) pursuant to a reincorporation merger. On August 4, 2009, the Company reincorporated in Delaware as Ohr Pharmaceutical, Inc.

On May 30, 2014, the Company completed the ophthalmology assets acquisition (the “SKS Acquisition”) of the privately held SKS Ocular LLC and its affiliate, SKS Ocular 1 LLC (“SKS”). Under the terms of the acquisition agreement, in exchange for substantially all the assets of SKS, Ohr made an upfront payment of \$3.5 million in cash and issued 1,194,862 shares of Ohr common stock to SKS. In addition, SKS is eligible to receive up to an aggregate of 1,493,577 additional shares of Ohr common stock in three contingent milestone payments, each milestone resulting in the issuance of 497,859 shares of Ohr common stock. Milestone 1 required Ohr to demonstrate a consistent long-term release of a therapeutic agent above threshold therapeutic levels in the targeted ocular tissues of an animal model. Ohr met this milestone in December 2015. Milestone 2 requires the completion of a pharmacodynamic study in an animal model showing clinically relevant efficacy from a drug substance released from SKS Microparticles within 24 months of the date of the closing of the SKS Acquisition. Milestone 3 requires, among other things, the approval of an Investigational New Drug Application (“IND”) within three years of the date of the closing of the SKS Acquisition. An IND is required to be submitted by us (or one of our subsidiaries) to the U.S. Food and Drug Administration (the “FDA”) within four months of the date of the successful completion of an IND enabling toxicity study and other IND enabling processes (the “IND Ready Date”); however if an IND is not submitted to the FDA within four months of the IND Ready Date, then Milestone 3 shall mean the approval of an IND at any time (regardless of whether the date of such approval is earlier or later than the date that is three years of the date of the closing of the SKS Acquisition; provided that if we (or one of our subsidiaries) fail to submit such IND to the FDA within nine months of the IND Ready Date, then Milestone 3 will be considered to have been achieved.

The transaction provided Ohr with a proprietary, patent protected, sustained release technology platform under development as well as a pipeline of pre-clinical sustained release drug product candidates that address ocular indications including glaucoma, ocular allergy, retinal disease and other ophthalmic indications. As part of the SKS Acquisition, Ohr retained the SKS employees and continued the SKS operations at the SKS research laboratory facility in San Diego, CA.

Simultaneous with the SKS Acquisition, Ohr completed a holding company reorganization in which Ohr merged with a wholly-owned subsidiary and a new parent corporation succeeded Ohr as a public holding company under the same name. The business operations of Ohr did not change as a result of the reorganization. The new holding company retains the name “Ohr Pharmaceutical, Inc.” Outstanding shares of the capital stock of the former Ohr Pharmaceutical, Inc. were automatically converted, on a share for share basis, into identical shares of common stock of the new holding company.

## Recent Events

In March 2016, the Company reached an agreement on a Special Protocol Assessment with the United States Food and Drug Administration on the design of the Phase III trial for its lead drug candidate, squalamine lactate ophthalmic solution, 0.2% (“Squalamine,” also known as OHR-102). Based on the agreed upon SPA, Ohr initiated the first of two planned Phase III global clinical studies evaluating the efficacy and safety of Squalamine, given in combination with Lucentis<sup>®</sup>, for the treatment of neovascular age-related macular degeneration (wet AMD). The first patient in the phase III study was enrolled in April 2016.

## PRODUCT PIPELINE

### (a) SQUALAMINE LACTATE OPHTHALMIC SOLUTION 0.2%

#### Squalamine Lactate Ophthalmic Solution 0.2% (“Squalamine”, also known as OHR-102)

Squalamine lactate is a small molecule anti-angiogenic drug with a novel intracellular mechanism of action. The drug acts against the development of aberrant neovascularization by inhibiting multiple protein growth factors of angiogenesis, including vascular endothelial growth factor (“VEGF”), platelet-derived growth factor (“PDGF”) and basic fibroblast growth factor (“bFGF”). Recent clinical evidence has shown PDGF to be an additional target for the treatment of Wet-AMD and bFGF levels have been shown to be elevated in retinal vein occlusion and wet-AMD patients as well.

Ohr formulated squalamine lactate as a topical solution (Squalamine lactate ophthalmic solution 0.2% or OHR-102) for ophthalmic indications and optimized the formulation for enhanced uptake into the back of the eye, and to potentially provide increased comfort in an elderly patient population. The Company is advancing its clinical wet-AMD program with this topical formulation. Unlike other combination therapy approaches being evaluated in clinical studies, Squalamine does not require direct injection into the eye.

We believe that Squalamine used in combination with an anti-VEGF agent may provide several potential advantages over other combination therapy approaches currently being investigated in clinical studies including:

- Daily topical therapy compared to additional monthly intravitreal injections.
- Potential use in combination with as-needed (PRN) anti-VEGF injections or treat-and-extend regimens as well as a monthly/bi-monthly anti-VEGF injection regimen.
- Adaptable for use in combination with longer acting anti-VEGF agents.
- Inhibition of multiple growth factor pathways.
- Cost efficiency of manufacturing a small molecule when compared to large molecule proteins and antibodies.

The Company has conducted a preclinical program which consisted of pharmacology, pharmacokinetic, and toxicology studies which support the ongoing clinical development of Squalamine.

#### Completed Phase II Trial in wet-AMD: the IMPACT Study (formerly OHR-002)

We commenced a clinical study, Study OHR-002 (or IMPACT Study), which began enrolling patients in late 2012. The IMPACT Study was a multi-center, randomized, double masked, placebo controlled Phase II study to evaluate the efficacy and safety of Squalamine combination therapy for the treatment of wet-AMD. The study enrolled treatment naïve wet-AMD patients at more than 20 clinical sites in the U.S. who were randomly assigned to treatment with Squalamine lactate ophthalmic solution 0.2%, or placebo eye drops for a nine month period, along with Lucentis<sup>®</sup> injections, as necessary, following an initial baseline Lucentis injection. Full enrollment was completed in April 2014. In March 2015, we completed the IMPACT Study and announced topline results. The final data from the IMPACT Study was presented at multiple scientific conferences and forums in 2015 and 2016, with additional data presentations expected at scientific conferences in calendar 2016.

In a prespecified analysis, data from the IMPACT study demonstrated that, in the intent-to-treat (ITT-LOCF) population with lesions containing classic choroidal neovascularization (“classic CNV”) (Squalamine combination treatment n=38, Lucentis® monotherapy n=32), 42% of the patients receiving Squalamine achieved a  $\geq 3$  line gain at nine months, as compared to 28% in the Lucentis monotherapy group. In patients with classic CNV (ITT-LOCF), mean gains in visual acuity were +10.5 letters for the Squalamine combination arm and +5.4 letters with Lucentis monotherapy, a clinically meaningful benefit of +5.1 letters. The positive effect on visual acuity in classic CNV was seen early in the course of treatment and continued to increase through the end of the study. Less of a visual acuity benefit was seen in the overall population (classic CNV and occult only CNV lesions). The mean number of injections between the treatment arms, the primary endpoint of the study, was not meaningfully different.

Further analyses were conducted to determine the patient population most likely to benefit from combination treatment. Patients with classic CNV are a heterogeneous population and, within the enrollment criteria of our study, could have encompassed small classic lesions with no occult component as well as lesions up to 12 disc areas ( $\sim 30\text{mm}^2$ ) in size made up almost entirely of occult CNV. These diverse lesions would both fall under the same category of “classic containing lesion” even though they would be expected to respond differently to treatment. Correlation analyses determined that the occult CNV size at baseline, regardless of whether there was a classic CNV component present, directly correlated with improved visual acuity outcomes in the Squalamine combination group ( $p < 0.0001$ ), which was not seen in the Lucentis monotherapy group. This suggests that the occult CNV size was a more important predictor of success for combination therapy than the presence of classic CNV, and a cutoff less than  $10\text{mm}^2$  of occult size at baseline was determined to be the optimal size to include in future clinical studies. In those patients with occult CNV less than  $10\text{mm}^2$  in area (n=94 of 128 completing the phase II study), 40% of those treated with Squalamine combination therapy achieved a gain of 3 or more lines of vision, compared with 26% of patients in the Lucentis monotherapy arm, a 54% additional benefit. In addition, mean gains in visual acuity compared to baseline were +11.0 letters for the Squalamine combination arm and +5.7 letters with Lucentis monotherapy, a clinically meaningful benefit of +5.3 letters. Importantly, this group of patients represents a larger proportion of the subjects enrolled in the IMPACT study than the classic containing group. We are enrolling this optimized patient population in our Phase III clinical program.

#### Regulatory (FDA) Status of Squalamine Program in Wet-AMD

In March 2016, the Company reached an agreement on a Special Protocol Assessment (SPA) with the United States Food and Drug Administration (US FDA) on the design of the Phase III trial. The FDA awarded Fast Track Designation to the Squalamine eye drop program for the potential treatment of wet-AMD in 2012.

#### Phase III Trials in Wet-AMD

The Phase III program will evaluate the efficacy and safety of Squalamine given in combination with Lucentis for newly diagnosed, treatment naive patients with wet-AMD. The comprehensive clinical program will be comprised of double-masked, placebo-controlled, multicenter, international studies of Squalamine administered twice a day in patients with newly diagnosed wet AMD, in combination with Lucentis injections. The primary endpoint will be a measurement of visual acuity gains at nine months, with patients followed to two years for safety. We intend to enroll a patient population that we believe is the most likely to benefit from Squalamine combination therapy based on our full analysis of the IMPACT study. During the first year of the study, patients will be randomized 1:1 to receive monthly Lucentis plus Squalamine twice a day or Lucentis plus placebo eye drops twice a day. During the second year they will receive Lucentis PRN (as needed) plus Squalamine or placebo eye drops twice a day. In March 2016, the Company initiated the first phase III study and enrolled the first patient in April 2016.

#### OHR-1501 Study

OHR-1501 is an exploratory, double-masked, randomized, placebo-controlled study designed to assess safety and efficacy of treatment with squalamine lactate ophthalmic solution in combination with monthly Lucentis injections in patients with neovascular AMD. Approximately 20 subjects will participate for a six-month duration. Efficacy parameters will include functional visual acuity (BCVA), and retinal imaging modalities. The primary endpoints of the study are the outcome measures at Week 12. We expect data from this study in calendar 2016.

#### Completed Trial in Proliferative Diabetic Retinopathy (“PDR”) - Study 003

Study 003 was an open-label monotherapy investigator sponsored trial (“IST”) evaluating Squalamine in five patients with PDR. Patients enrolled in the study received Squalamine for a six month treatment period and were then followed for an additional two months. The endpoints included regression of neovascularization, anatomical measurements, visual acuity, and safety parameters. The principal investigator of Study 003 presented a case report from the first patient to complete the protocol in February 2014. In this case report, the oral presentation discussed the case of a treatment naive patient diagnosed with PDR. The data demonstrated that topical application of Squalamine in a monotherapy regimen, twice daily and then four times daily, was associated with regression of retinal neovascularization within two months. The retinal neovascularization remained regressed throughout the six months of four times daily Squalamine therapy. One month after cessation of treatment, the abnormal blood vessels returned in this patient’s retina in the absence of Squalamine treatment, and continued to grow through the second month, the latest time point measured. We expect the final data will be disseminated by the investigator in a scientific publication.

## Completed Trial in Branch and Central Retinal Vein Occlusion -Study 004

Study 004 was an IST evaluating squalamine lactate ophthalmic solution, 0.2%, in 20 patients with branch and central retinal vein occlusion. All patients in the study received Squalamine for 10 weeks, with injections of Lucentis at week two and six, and a data readout at week 10. At week 10, the patients entered into the extension phase and were randomized 1:1 to either continue or discontinue taking Squalamine through week 38 (“extension phase”). During the extension phase, the patients received Lucentis injections on a PRN basis as determined by fluid based OCT criteria. The principal investigator presented the 10 week data from the study in August 2014. The data demonstrated that, at week 10 (1) the mean gain in visual acuity was 20.3 letters for all 20 patients using the combination therapy, (2) the mean visual acuity for all 20 patients at was 20/32, (3) the average central foveal thickness for all 20 patients was reduced to 270u, and (4) only one of 20 patients qualified for an injection of Lucentis, indicating dryness of the retina and a 95% macular deturgescence rate.

In July 2015, final data was presented demonstrating that at week 38, (1) the mean gain in visual acuity from baseline for patients randomized (at week 10) to treatment with Squalamine + Lucentis PRN was +27.8 letters compared with +23.3 for patients randomized to treatment with Lucentis plus PRN alone (control group), a clinically meaningful difference of +4.5 letters, (2) 80% of patients in the Squalamine + Lucentis treated group had a gain in visual acuity, compared with 50% of patients treated with Lucentis alone, and (3) none of the patients in the Squalamine + Lucentis treated group lost any vision as compared to 50% of the patients receiving Lucentis alone. After the initial combination therapy phase, the mean gain in visual acuity from week 10 to week 38 was +7.4 letters for patients who continued treatment with Squalamine + Lucentis PRN compared with +3.1 letters in those receiving Lucentis PRN alone. Patients treated with Squalamine + Lucentis PRN required a mean of 2.0 Lucentis injections between weeks 10 and 38, compared with a mean of 3.3 Lucentis injections for the monotherapy group over the same time period.

### (b) SKS Sustained Release Ocular Drug Delivery Platform Technology

The SKS sustained release technology employs a hydrogel template approach to prepare nano or microparticles of predefined size and shape and with homogeneous size distribution. The size of the particles can be adjusted, providing flexibility in controlling the size and release rate in drug delivery formulations. The drug loading capacity is much higher than that achieved by conventional methods (50% or higher), with a controlled initial burst release of drug that is minimal. Simplicity in processing makes the hydrogel template method useful for scale-up manufacturing of particles. We believe the technology has significant advantages over currently available microparticle drug delivery systems prepared by emulsion and other methods. This technology platform is adaptable to multiple routes of ocular delivery and amenable to multiple different polymers.

The SKS sustained release technology was designed to develop best-in-class drug formulations for ocular disease. The technology employs micro fabrication techniques to create nano and microparticle drug formulations that can provide sustained and predictable release of a therapeutic drug over a 3 – 6 month period. The versatility of this delivery technology makes it well suited to deliver hydrophilic or hydrophobic small molecules, as well as proteins with complex structures. Ohr’s preclinical pipeline of sustained release programs include sustained release formulations of small molecule and protein therapeutics for the treatment of ocular diseases, including glaucoma, steroid induced glaucoma, allergies, and retinal disease. Ohr has several molecules under development for these indications and anticipates expanding the pipeline during calendar year 2016 to include additional molecules and indications in ocular disease.

In December 2015, we announced the achievement of Milestone 1, demonstrating in vivo proof of sustained release in one of our internal programs, and data from this study was presented at the Association for Research in Vision and Ophthalmology (ARVO) in May 2016. In this study we dosed rabbits with a single intravitreal dose of our SKS sustained release formulation of a novel small molecule anti-angiogenic compound, and then followed the rabbits over a six week period. The study showed that sustained supratherapeutic levels of active drug could be achieved in the retina and choroid, which are the target ocular tissues for back of the eye disease. These observations were made at all time points in the study which demonstrates a prolonged pharmacokinetic profile. Furthermore, vitreous concentrations remained at stable, high levels throughout the six week study indicating that the true duration of effect is likely much longer. This study serves as an important validation for our SKS sustained release technology which we believe holds the promise of improving the standard of care in a number of ocular conditions by allowing for physician administration of drugs at convenient treatment intervals. We anticipate presenting additional in-vivo proof of concept data on our internal programs in calendar 2016 and potentially filing an investigational new drug application with the FDA on one sustained release program in the first half of calendar 2017.

### (c) Animal Model for Dry-AMD

As part of the SKS Acquisition, we acquired the exclusive rights to an animal model for dry-AMD whereby mice are immunized with a carboxyethylpyrrole (“CEP”) which is bound to mouse serum albumin (“MSA”). CEP is produced following the oxidation of docosahexaenoic acid, which is abundant in the photoreceptor outer segments that are phagocytosed by the retinal pigment epithelium (“RPE”). A number of CEP-adducted proteins have been identified in proteomic studies examining the composition of drusen and other subretinal deposits found in the eyes of patients with dry-AMD. Studies have shown that immunization of CEP-MSA can lead to an ophthalmic phenotype very similar to dry-AMD, including deposition of complement in the RPE, thickening of the Bruch’s membrane, upregulation of inflammatory cytokines, and immune cell influx into the eye. Ohr licenses the intellectual property rights to this model, and EyeCRO, a development partner of the Company, has obtained exclusive rights to provide contracted screening services in the CEP model. EyeCRO is a contract research organization specializing in preclinical services to the ophthalmology industry. As part of the EyeCRO arrangement, we have optimized the induction parameters to create disease pathology within 60 days. Upon immunization with CEP, a marked decrease in contrast sensitivity which precedes a loss of visual acuity, was observed, similar to what occurs in many patients with dry AMD. Under the terms of the license agreement, we may receive royalties from EyeCRO during fiscal 2016.

(d) Non-Ophthalmology Assets

Ohr also owns various other compounds in earlier stages of development, including the PTP1b inhibitor Trodusquemine and related analogs. See “Corporate Strategy” concerning the Trodusquemine joint venture. During fiscal 2015, the Company ceased all development of OHR/AVR 118 and recognized an impairment on the patent portfolio in the amount of \$338,906.

## Competitive Factors

### *Competition in General*

Competition in the area of biomedical and pharmaceutical research and development is intense and significantly depends on scientific and technological factors. These factors include the availability of patent and other protection for technology and products, the ability to commercialize technological developments and the ability to obtain regulatory approval for testing, manufacturing and marketing. Our competitors include major pharmaceutical and specialized biotechnology companies, many of which have financial, technical and marketing resources significantly greater than ours. In addition, many biotechnology companies have formed collaborations with large, established companies to support research, development and commercialization of products that may be competitive with ours. Academic institutions, governmental agencies and other public and private research organizations are also conducting research activities and seeking patent protection and may commercialize products on their own or through joint ventures. We are aware of certain other products manufactured or under development by competitors that are used for the treatment of the health conditions that we have targeted for product development. We can provide no assurance that developments by others will not render our technology obsolete or noncompetitive, that we will be able to keep pace with new technological developments or that our technology will be able to supplant established products and methodologies in the therapeutic areas that are targeted by us. The foregoing factors could have a material adverse effect on our business, prospects, financial condition and results of operations. These companies, as well as academic institutions, governmental agencies and private research organizations, also compete with us in recruiting and retaining highly qualified scientific personnel and consultants.

Competition within our sector is increasing, so we will encounter competition from existing firms that offer competitive solutions in ocular diseases. These competitive companies could develop products that are superior to, or have greater market acceptance, than the products being developed by us. We will have to compete against other biotechnology and pharmaceutical companies with greater market recognition and greater financial, marketing and other resources.

### *Competition in Wet AMD*

Lucentis® (Genentech/Roche) and Eylea® (Regeneron) are currently approved by the FDA and are the market leaders for the treatment of wet-AMD. Ophthotech is developing a combination therapy (Fovista®) used with an additional intravitreal agent to improve vision outcomes. There is no assurance that we can get FDA approval for Squalamine for the treatment of wet-AMD, and if we get it, there is no assurance we will be able to displace the market leaders as a treatment in a significant percentage of patients. In addition, there are various other companies with drugs in Phase I, II, and III trials for the treatment of wet-AMD. We cannot assure that none of them will get to market before us or that Squalamine will be a better treatment. See “Competitive Landscape in Wet-AMD” below.

### *Wet-AMD Market*

Age-related macular degeneration (“AMD”) is a medical condition which usually affects older adults and generally results in a loss of vision. AMD occurs in “dry” (non-exudative) and “wet” (exudative) forms. Wet-AMD is the advanced form of macular degeneration that involves the formation of abnormal and leaky blood vessels in the back of the eye behind the retina, through a process known as choroidal neovascularization (“CNV”). The wet form accounts for approximately 15 percent of all AMD cases, yet is responsible for 90 percent of severe vision loss associated with AMD. According to the National Eye Institute (NEI), the prevalence of wet-AMD among adults 40 years or older in the U.S. alone is estimated at 1.75 million people. In addition, more than 200,000 new cases are diagnosed yearly in the U.S.

### *Competitive Landscape in Wet-AMD*

The current FDA approved market leaders for the treatment of wet AMD are VEGF inhibitors, including Lucentis®, Eylea® and (off-label) Avastin®. In 2015, annual revenue (worldwide) was more than \$3 billion for Lucentis, despite significant cannibalization by the off-label use of Avastin (estimated to be 45-60% of the overall market). Eylea®, was approved for use in wet-AMD in the U.S. in November 2011 and achieved 2015 revenues of approximately \$4 billion. Both Lucentis and Eylea are administered via frequent intravitreal injections directly into the eye. We believe our primary competition is Fovista®, a PDGF targeting aptamer being developed by Ophthotech and Novartis, which is currently enrolling the last of three Phase III clinical studies to evaluate Fovista in combination with anti-VEGF agents, including Lucentis®, Eylea®, and Avastin®. To date, Fovista and Squalamine are the only combination therapy approaches we are aware of that have demonstrated a visual acuity benefit when used in combination with an anti-VEGF intravitreal injection. The Fovista clinical trials are designed for patients to receive two intravitreal injections per month. Other programs that are currently in Phase II or Phase III trials include Abicipar Pegol, a VEGF targeting DARPIn molecule being developed by Allergan, RTH258, an anti-VEGF agent being developed by Alcon/Novartis, X-82, an oral tyrosine kinase inhibitor being developed by Tyrogenex, ALG-1001, an integrin targeting peptide being developed by Allegro Ophthalmics, and REG-2176, a combination injection of anti-VEGF and PDGF agents being developed by Regeneron. All of these products in clinical development, with the exception of X-82, use an intravitreal route of administration much like the current standards of care.

### ***Competitive Landscape in Sustained Release Drug Delivery***

There are a number of companies developing various forms of sustained release drug delivery platforms for ophthalmic applications. These include GreyBug with a biodegradable polymer microsphere/nanoparticle matrix system, Envisia Therapeutics with the PRINT<sup>®</sup> technology system for microparticle and nanoparticle formulations, Kala Pharmaceuticals with a mucus-penetrating particle (MPP) technology, and Ocular Therapeutix with a proprietary hydrogel technology ophthalmic sustained drug delivery. All of these programs are in the preclinical or early clinical development stages. Each of these may prove to be effective means to deliver drugs in a sustained manner and we cannot assure you that none of them will get to market before us or that the SKS technology will be a better drug delivery approach.

### **Corporate Strategy**

The Company is currently actively developing its pipeline products for applications in ophthalmology. Beginning in fiscal 2014, we transitioned Ohr to a core focus on ophthalmology indications and building an ophthalmology-focused pipeline, and we expect to continue to see growth in our pipeline and ophthalmology initiatives.

After the presentations of the final results from the Phase II IMPACT Study with Squalamine, we began an initiative to seek and implement strategic alternatives with respect to our products, including licenses, business collaborations and other business combinations or transactions with other pharmaceutical and biotechnology companies. Several third parties with whom we have been in discussions have expressed interest in a potential licensing or partnering transaction for the Squalamine program. We continue to make progress in these discussions; however, we currently do not have any agreement to enter into such a transaction, and there is no assurance that the Company will enter into or complete such a transaction.

As part of its core strategy, on February 26, 2014, the Company entered into a Joint Venture Agreement and related agreements with Cold Spring Harbor Laboratory (“CSHL”) pursuant to which a joint venture, DepYmed Inc. (“DepYmed”), was formed to further preclinical and clinical development of Ohr’s Trodusquemine and analogues as PTP1B inhibitors for oncology indications. DepYmed licenses research from CSHL and intellectual property from the Company. In December 2014, DepYmed hired a full time CEO to run the operations of DepYmed and intends to seek private investment to fund the ongoing operations of DepYmed, leaving Ohr as a passive joint venturer.

### **Liquidity and Sources of Capital**

The Company has limited working capital reserves with which to continue development of its pharmaceutical products and continuing operations. The Company is reliant, at present, upon its capital reserves for ongoing operations and has no revenues.

Net working capital reserves decreased from the beginning of the first quarter of fiscal 2016 to the end of the second quarter of fiscal 2016 by \$6,333,606 (to \$18,822,416 from \$25,156,022) primarily due to increased cost of developing our product portfolio. Our quarterly cash burn had been approximately \$2 to \$3.5 million per quarter in fiscal 2015 and for the first two quarters of fiscal 2016 and we expect our cash burn to significantly increase in the remainder of fiscal 2016 in connection with the enrollment of patients in Phase III program and the ongoing development of our sustained release platform technology. The Company does not have a bank line of credit or other fixed source of capital reserves. Should it need additional capital in the future, the Company will be primarily reliant upon private or public placement of its equity or debt securities, or a transaction with a pharmaceutical partner, for which there can be no warranty or assurance that the Company may be successful in such efforts. Management believes the Company has sufficient capital to meet its planned operating needs through December 2016.

## Results of Operations

### Three Months Ended March 31, 2016

Three months ended March 31, 2016 (“2016”) compared to the three months ended March 31, 2015 (“2015”). Results of operations for the three months ended March 31, 2016 reflect the following changes from the prior period.

	<u>2016</u>	<u>2015</u>	<u>Change</u>
General and administrative	\$ 2,966,363	\$ 3,334,971	\$ (368,608)
Research and development	4,043,859	3,175,855	868,004
Depreciation and amortization	296,077	295,262	815
Gain on settlement of accounts payable	(710,264)	—	(710,264)
Total Operating (Income) Expenses	<u>6,596,035</u>	<u>6,806,088</u>	<u>(210,053)</u>
Operating Loss	(6,596,035)	(6,806,088)	210,053
Change in fair value of contingent consideration	1,305,623	3,786,193	(2,480,570)
Share in losses on investment in joint venture	—	(41,211)	41,211
Impairment of intangibles	—	(338,906)	338,906
Other income and expenses, net	5,553	(536)	6,089
Net Loss	<u>\$ (5,284,859)</u>	<u>\$ (3,400,548)</u>	<u>\$ (1,884,311)</u>

The Company had no net revenues from operations in 2015 or 2016. Accordingly, the Company had no cost of revenue from operations in 2015 or 2016.

General and administrative expenses from operations decreased from \$3,334,971 in 2015 to \$2,966,363 in 2016. The decrease is a result of a change in stock price that affects stock compensation expense.

The Company incurred \$4,043,859 in research and development expenses in 2016 compared to \$3,175,855 in 2015. The increase is a result of the ongoing clinical trials in ophthalmic indications and increased costs from the development of our sustained release platform technology. The Company expects research and development expenses to continue to rise as development of its products continues.

Depreciation and amortization expense remained relatively stable with \$295,262 in 2015 and \$296,077 in 2016.

In 2016, the Company had a gain on settlement of \$710,264 compared to no gain on settlement in 2015.

In 2016, the Company reported an increase in the change in fair value of contingent consideration of \$1,305,623 compared to an increase of \$3,786,193 in 2015. The decrease is due to a change in fair value of the contingent consideration from the SKS Acquisition and the achievement of Milestone 1.

In 2016, the Company had no impairment of intangibles compared to \$338,906 in 2015.

In 2016, the Company had no losses on investment in joint venture compared to \$41,211 in 2015.

For the three months ended March 31, 2016, the Company recognized a net loss of \$5,284,859 compared to a net loss of \$3,400,548 for the same period in 2015. Until the Company is able to generate revenues, management expects to continue to incur net losses.

### Six Months Ended March 31, 2016

Six months ended March 31, 2016 (“2016”) compared to the six months ended March 31, 2015 (“2015”). Results of operations for the six months ended March 31, 2016 reflect the following changes from the prior period.

	<u>2016</u>	<u>2015</u>	<u>Change</u>
General and administrative	\$ 4,184,492	\$ 4,151,834	\$ 32,658
Research and development	6,120,139	5,919,977	200,162
Depreciation and amortization	593,816	600,884	(7,068)
Gain on settlement of accounts payable	(710,264)	—	(710,264)
Total Operating (Income) Expenses	<u>10,188,183</u>	<u>10,672,695</u>	<u>(484,512)</u>
Operating Loss	(10,188,183)	(10,672,695)	484,512
Change in fair value of contingent consideration	(1,251,926)	3,102,807	(4,354,733)
Share in losses on investment in joint venture	—	(67,861)	67,861
Impairment of intangibles	—	(338,906)	338,906
Other income and expenses, net	9,788	35,150	(25,362)

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Net Loss

\$ (11,430,321) \$ (7,941,505) \$ (3,488,816)

The Company had no net revenues from operations in 2015 or 2016. Accordingly, the Company also had no cost of revenue from operations in 2015 or 2016.

General and administrative expenses from operations remained relatively flat, with a \$32,658 increase when comparing 2016 to 2015.

The Company incurred \$6,120,139 in research and development expenses in 2016 compared to \$5,919,977 in 2015. The increase is a result of the ongoing clinical trials in ophthalmic indications and increased costs from the development of our sustained release platform technology. The Company expects research and development expenses to continue to rise as development of its products continues.

Depreciation and amortization expense remained relatively flat with \$600,884 expensed in 2015 and \$593,816 in 2016.

In 2016, the Company had a gain on settlement of \$710,264 compared to no gain on settlement in 2015.

In 2016, the Company had a decrease in the fair value of contingent consideration in 2016 of \$1,251,926 compared to an increase of \$3,102,807 in 2015. The decrease is due to a change in fair value of the contingent consideration from the SKS Acquisition and the achievement of Milestone 1.

In 2016, the Company had no impairment of intangibles compared to \$338,906 in 2015.

In 2016, the Company had no losses on investment in joint venture compared to \$67,861 in 2015.

For the six months ended March 31, 2015, the Company recognized a net loss of \$11,430,321 compared to net loss of \$7,941,505 for the same period in 2015. Until the Company is able to generate revenues, management expects to continue to incur such net losses.

### **Item 3. Quantitative and Qualitative Risk**

Market risk represents the risk of loss arising from adverse changes in interest rates and foreign exchange rates. The Company does not have any material exposure to interest rate or exchange rate risk.

### **Item 4. Controls and Procedures**

#### **Evaluation of Disclosure Controls and Procedures**

The Company, under the supervision and with the participation of its management, including the Chief Executive Officer and the Chief Financial Officer, evaluated the effectiveness of the design and operation of the Company's "disclosure controls and procedures" (as defined in Rule 13a-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act")) as of the end of the period covered by this Report. Based on that evaluation, the Chief Executive Officer and the Chief Financial Officer concluded the Company's disclosure controls were effective. In designing and evaluating the disclosure controls and procedures, our management, including the Chief Executive Officer and the Chief Financial Officer, recognized that any controls and procedures, no matter how well designed and operated, can only provide reasonable assurance of achieving the desired control objectives, and management was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our disclosure controls and procedures are designed to provide a reasonable level of assurance of reaching our desired disclosure controls objectives.

#### **Changes in Internal Control Over Financial Reporting**

During the period covered by this Report there has been no change in our internal control over financial reporting that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

## **PART II OTHER INFORMATION**

### **Item 1. Legal Proceedings**

None.

### **Item 1A. Risk Factors**

*You should carefully consider the following factors which may affect future results of operations. If any of the adverse events described below actually occur, our business, financial condition and operating results could be materially adversely affected and you may lose part or all of the value of your investment. If you choose to invest in our securities, you should be able to bear a complete loss of your investment.*

#### **Risks Related to Our Business and Industry**

##### **We currently do not have, and may never have, any products that generate significant revenues.**

We are a development stage pharmaceutical company and currently do not have, and may never have, any products that generate revenues. Investment in pharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and become commercially viable. To date, we have not generated any product revenues from our product candidates currently in development. We cannot guarantee that any of our product candidates currently in development will ever become marketable products.

We must demonstrate that our drug candidates satisfy rigorous standards of safety and efficacy for their intended uses before the FDA, and other regulatory authorities in the United States, the European Union and elsewhere. Significant additional research, preclinical testing and clinical testing is required before we can file applications with the FDA or other regulatory authorities for premarket approval of our drug candidates. In addition, to compete effectively, our drugs must be easy to administer, cost-effective and economical to manufacture on a commercial scale. We may not achieve any of these objectives. We recently completed a Phase II clinical trial for Squalamine, our most advanced drug candidate. The results of the Phase II clinical trial continue to support conducting Phase III clinical trials for Squalamine for a targeted population, with enrollment criteria based on the complete analysis of the Phase II clinical trial. The Company reached an agreement on a Special Protocol Assessment with the United States Food and Drug Administration on the design of the Phase III trial in March 2016, and we initiated the Phase III clinical program and began enrolling patients in April 2016. We cannot be certain that the clinical development of this or any other drug candidates in preclinical testing or clinical development will be successful, that we will receive the regulatory approvals required to commercialize them or that any of our other research and drug discovery programs will yield a drug candidate suitable for investigation through clinical trials. Our commercial revenues from our product candidates currently in development, if any, will be derived from sales of drugs that will not become marketable for several years, if at all.

##### **We have incurred significant losses and anticipate that we will incur additional losses. We might never achieve or sustain revenues.**

We have experienced significant net losses since our inception. As of March 31, 2016, we had an accumulated deficit of approximately \$70 million. We expect to incur net losses over the next several years as we advance our programs and incur significant clinical development costs. We have no products approved for commercial sale and have not generated any revenue from product sales to date, and we continue to incur significant research and development and other expenses related to our ongoing operations. We do not expect to receive, for at least the next several years, any revenues from the commercialization of our product candidates.

##### **There is no guarantee that our future Phase III clinical trials for Squalamine in wet-AMD will be completed in the anticipated timeframe or that they will be successful.**

We commenced a clinical study, Study OHR-002 (or IMPACT Study), which began enrolling patients in late 2012. The IMPACT Study was a randomized, double masked, placebo controlled Phase II study to evaluate the efficacy and safety of Squalamine for the treatment of wet-AMD. The study enrolled treatment naïve wet-AMD patients at 23 clinical sites in the U.S., who were treated with Squalamine eye drops or placebo eye drops for a nine month period. Full enrollment was completed in April 2014, and final results were released in March 2015. The results of the Phase II clinical trial continue to support conducting Phase III clinical trials for Squalamine with enrollment criteria for a targeted population, based on the complete analysis of the Phase II clinical trial. The Company reached an agreement on a Special Protocol Assessment with the FDA on the design of the Phase III trial in March 2016, and we initiated the Phase III clinical program and began enrolling patients in April 2016. There can be no assurance that the Phase III clinical trials will be completed in the anticipated timeframe or that they will be successful.

**The historical rate of failures for product candidates in clinical development and late-stage clinical trials is high.**

The Phase III trials for Squalamine are designed to measure the efficacy of combination therapy with Squalamine plus Lucentis injections compared with Lucentis monotherapy in treatment naïve patients with wet-AMD. All patients will be followed for safety for two years.

During the first year of the study, patients will be randomized 1:1 to receive monthly Lucentis plus Squalamine (Squalamine lactate ophthalmic solution, 0.2%) twice a day or Lucentis plus placebo. During the second year they will receive Lucentis PRN (as needed) plus Squalamine or placebo twice a day. The primary endpoint will be an improvement in a visual acuity parameter, as measured by a standard ETDRS visual acuity chart. The Company reached an agreement on a Special Protocol Assessment with the FDA on the design of the Phase III trial in March 2016, and we initiated the Phase III clinical program and began enrolling patients in April 2016.

There can be no assurance that we will meet the goals of the Phase III clinical trials or that we will have the same level of success in these the Phase III clinical trials as we have in our prior clinical trials, or be successful at all. We believe that Squalamine may also have clinical utility in indications other than wet-AMD. We have completed ISTs in ophthalmic indications where a molecule that possesses anti-angiogenic properties may provide therapeutic benefit. These indications include branch retinal vein occlusion, central retinal vein occlusion, and proliferative diabetic retinopathy.

If we do not successfully complete clinical development of Squalamine, we will be unable to market and sell products derived from it and to generate product revenues. Even if we do successfully complete clinical trials for Squalamine in patients with wet-AMD, those results are not necessarily predictive of results of future pivotal trials that may be needed before we may submit a New Drug Application, or NDA, to the FDA for the initial or other future indications. Of the vast number of drugs in development, only a small percentage result in the submission of an NDA to the FDA, and even fewer result in the NDA ultimately being approved by the FDA for commercialization.

**We will need to raise substantial additional capital to further our drug and delivery platform development programs as well as future trials, including our planned Phase III clinical trials for Squalamine in wet-AMD, and may not be able to raise additional capital on favorable terms, if at all. If additional capital is not available, we may have to delay, reduce or cease operations.**

We will need substantial additional capital to further our drug and delivery platform development programs as well as future trials. Specifically, we will require significant additional funds to complete our planned Phase III clinical trials for Squalamine in wet-AMD. In our capital-raising efforts, we may seek to sell additional equity or debt securities or obtain a bank credit facility. The sale of additional equity or debt securities, if convertible, could result in dilution to our stockholders. The incurrence of indebtedness would result in increased fixed obligations and could also result in covenants that would restrict our operations. However, we may not be able to raise additional funds on acceptable terms, or at all. If we are unable to secure sufficient capital to fund our research and development activities, we may have to delay, reduce or cease operations.

Our long-term strategy with respect to Squalamine in wet-AMD is to seek a strategic commercial partner, or partners, such as large pharmaceutical companies, with extensive experience in the development, commercialization, and marketing of our products. Several third parties with whom we have been in discussions have expressed interest in a potential licensing or partnering transaction for the Squalamine program. We continue to make progress in these discussions. However, we currently do not have any agreement to enter into such a transaction, and there is no assurance that the Company will enter into or complete such a transaction.

**Results from early clinical trials for Squalamine in wet-AMD are not necessarily predictive of the results of later clinical trials for Squalamine in wet-AMD. If we cannot replicate the results from our earlier clinical trials for Squalamine in wet-AMD in our later clinical trials, we may be unable to successfully develop, obtain regulatory approval for and commercialize Squalamine in wet-AMD.**

Results from our Phase II clinical trial for Squalamine in wet-AMD may not necessarily be predictive of the results from required later clinical trials. We may not be able to complete our planned Phase III clinical trials for Squalamine in wet-AMD. Similarly, even if we are able to complete our planned Phase III clinical trials for Squalamine in wet-AMD according to our current development timeline, the results from our Phase II clinical trial for Squalamine in wet-AMD may not be replicated in our Phase III clinical trial results. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development, and we cannot be certain that we will not face similar setbacks. These setbacks have been caused by, among other things, pre-clinical findings made while clinical trials were underway or safety or efficacy observations made in clinical trials, including previously unreported adverse events. Moreover, pre-clinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that believed their product candidates performed satisfactorily in pre-clinical studies and clinical trials nonetheless failed to obtain FDA approval. If we fail to produce positive results in our planned Phase III clinical trials for Squalamine in wet-AMD, the development timeline and regulatory approval and commercialization prospects for our leading product candidate, and, correspondingly, our business and financial prospects, would be materially adversely affected.

**Delays, suspensions and terminations in our clinical trials could result in increased costs to us, delay our ability to generate product revenues and therefore may have a material adverse effect on our business, results of operations and future growth prospects.**

The commencement of clinical trials can be delayed for a variety of reasons, including: delays in demonstrating sufficient safety and efficacy to obtain regulatory approval to commence a clinical trial; reaching agreement on acceptable terms with prospective contract research organizations and clinical trial sites; manufacturing sufficient quantities of a product candidate; obtaining clearance from the FDA to commence clinical trials pursuant to an Investigational New Drug application, or IND; financial or strategic considerations; obtaining institutional review board approval to conduct a clinical trial at a prospective clinical trial site; and patient enrollment, which is a function of many factors, including the size of the patient population, the nature of the protocol, the proximity of patients to clinical trial sites, the availability of effective treatments for the relevant disease and the eligibility criteria for the clinical trial.

Once a clinical trial has begun, it may be delayed, suspended or terminated due to a number of factors, including: ongoing discussions with regulatory authorities regarding the scope or design of our clinical trials or requests by them for supplemental information with respect to our clinical trial results; failure to conduct clinical trials in accordance with regulatory requirements; lower than anticipated screening or retention rates of patients in clinical trials; serious adverse events or side effects experienced by participants; financial or strategic considerations; and insufficient supply or deficient quality of product candidates or other materials necessary for the conduct of our clinical trials.

Many of these factors may also ultimately lead to denial of regulatory approval of a current or potential product candidate. If we experience delays, suspensions or terminations in a clinical trial, the commercial prospects for the related product candidate will be harmed, and our ability to generate product revenues will be delayed.

**Even if we successfully complete the clinical trials of one or more of our product candidates, the product candidates may fail for other reasons.**

Even if we successfully complete the clinical trials for one or more of our product candidates, the product candidates may fail for other reasons, including the possibility that the product candidates will:

- fail to receive the regulatory approvals required to market them as drugs;
- be subject to proprietary rights held by others requiring the negotiation of a license agreement prior to marketing;
- be difficult or expensive to manufacture on a commercial scale;
- have adverse side effects that make their use less desirable; or
- fail to compete with product candidates or other treatments commercialized by our competitors.

In addition, our clinical trials may involve a specific patient population. Because of the small sample size, the results of these early clinical trials may not be indicative of future results. Adverse or inconclusive results may cause us to abandon a drug candidate and may delay development of other drug candidates. Any delay in, or termination of, our clinical and preclinical studies will delay the filing of our NDAs with the FDA and, ultimately, significantly impair our ability to commercialize our drug candidates and generate product revenues which would have a material adverse effect on our business and results of operations. If we are unable to receive the required regulatory approvals, secure our intellectual property rights, minimize the incidence of any adverse side effects or fail to compete with our competitors' products, our business, financial condition, and results of operations could be materially and adversely affected.

**If we find it difficult to enroll patients in our clinical trials, it will cause significant delays in the completion of such trials and may cause us to abandon one or more clinical trials.**

For the diseases or disorders that our product candidates are intended to treat, we expect only a subset of the patients with these diseases to be eligible for our clinical trials. Given that each of our product candidates is in preclinical or clinical development, we may not be able to initiate or continue clinical trials for each or all of our product candidates if we are unable to locate a sufficient number of eligible subjects to participate in the clinical trials required by the FDA or other foreign regulatory authorities. The requirements of our clinical testing mandate that a patient cannot be involved in another clinical trial for the same indication. We are aware that our competitors have ongoing clinical trials for products that are competitive with our product candidates and subjects who would otherwise be eligible for our clinical trials may be involved in such testing, rendering them unavailable for testing of our product candidates. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether, which would have a material adverse effect on our business.

**We rely, and expect that we will continue to rely, on third parties to conduct any future clinical trials for us, including our planned Phase III clinical trial for Squalamine in wet-AMD. If such third parties do not successfully carry out their duties or if we lose our relationships with such third parties, our drug development efforts could be delayed.**

We are dependent on contract research organizations, third-party vendors and independent investigators for preclinical testing, and clinical trials related to our drug discovery and development efforts, and we will likely continue to depend on them to assist in our future discovery and development efforts. These parties are not our employees, and we cannot control the amount or timing of resources that they devote to our programs. If they fail to devote sufficient time and resources to our drug development programs or if their performance is substandard, it will delay the development and commercialization of our product candidates. The parties with which we contract for execution of our clinical trials play a significant role in the conduct of the trials and the subsequent collection and analysis of data. Their failure to achieve their research goals or otherwise meet their obligations on a timely basis could adversely affect clinical development of our product candidates.

Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience regulatory compliance issues;
- undergo changes in priorities or become financially distressed; or
- form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our clinical trials and may subject us to unexpected cost increases that are beyond our control. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on contract research organizations does not relieve us of our regulatory responsibilities. We and our contract research organizations are required to comply with current Good Clinical Practices, or cGCPs, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for any products in clinical development. The FDA enforces these cGCP regulations through periodic inspections of clinical trial sponsors, principal investigators and trial sites. If we or our contract research organizations fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, the FDA will determine that any of our clinical trials comply with cGCPs. In addition, our clinical trials must be conducted with product produced under current Good Manufacturing Practices, or cGMPs, regulations and will require a large number of test subjects. Our failure or the failure of our contract research organizations to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process and could also subject us to enforcement action up to and including civil and criminal penalties.

Although we do design our clinical trials for Squalamine in wet-AMD and other drug candidates, contract research organizations conduct all of the clinical trials. As a result, many important aspects of our drug development programs are outside of our direct control. In addition, the contract research organizations may not perform all of their obligations under arrangements with us or in compliance with regulatory requirements, but we remain responsible and are subject to enforcement action that may include civil penalties up to and including criminal prosecution for any violations of FDA laws and regulations during the conduct of our clinical trials. If the contract research organizations do not perform clinical trials in a satisfactory manner, breach their obligations to us or fail to comply with regulatory requirements, the development and commercialization of Squalamine in wet-AMD and other drug candidates may be delayed or our development program materially and irreversibly harmed. We cannot control the amount and timing of resources these contract research organizations devote to our program. If we are unable to rely on clinical data collected by our contract research organizations, we could be required to repeat, extend the duration of, or increase the size of our clinical trials and this could significantly delay commercialization and require significantly greater expenditures.

**If our contract research organizations do not successfully carry out their duties or if we lose our relationships with contract research organizations, our drug development efforts could be delayed.**

We are dependent on contract research organizations, third-party vendors and independent investigators for preclinical testing and clinical trials related to our drug discovery and development efforts, and we will likely continue to depend on them to assist in our future discovery and development efforts. These parties are not our employees, and we cannot control the amount or timing of resources that they devote to our programs. If they fail to devote sufficient time and resources to our drug development programs or if their performance is substandard, it will delay the development and commercialization of our product candidates. The parties with which we contract for execution of our clinical trials play a significant role in the conduct of the trials and the subsequent collection and analysis of data. Their failure to achieve their research goals or otherwise meet their obligations on a timely basis could adversely affect clinical development of our product candidates.

If we lose our relationship with any one or more of these parties, we could experience a significant delay in both identifying another comparable provider and then contracting for its services. We may be unable to retain an alternative provider on reasonable terms, if at all. Even if we locate an alternative provider, it is likely that this provider may need additional time to respond to our needs and may not provide the same type or level of service as the original provider. In addition, any provider that we retain will be subject to current Good Laboratory Practices, and similar foreign standards, and we do not have control over compliance with these regulations by these providers. Consequently, if these practices and standards are not adhered to by these providers, the development and commercialization of our product candidates could be delayed.

**Our product candidates may not gain acceptance among physicians, patients, or the medical community, thereby limiting our potential to generate revenues, which will undermine our future growth prospects.**

Even if our product candidates are approved for commercial sale by the FDA or other regulatory authorities, the degree of market acceptance of any approved product candidate by physicians, healthcare professionals and third-party payors, and our profitability and growth will depend on a number of factors, including:

- the ability to provide acceptable evidence of safety and efficacy;
- pricing and cost effectiveness, which may be subject to regulatory control;
- our ability to obtain sufficient third-party insurance coverage or reimbursement;
- effectiveness of our or our collaborators' sales and marketing strategy;
- relative convenience and ease of administration;
- the prevalence and severity of any adverse side effects; and
- availability of alternative treatments.

If any product candidate that we develop does not provide a treatment regimen that is at least as beneficial as the current standard of care or otherwise does not provide some additional patient benefit over the current standard of care, that product will not achieve market acceptance and we will not generate sufficient revenues to achieve profitability.

**We may not be able to continue or fully exploit our partnerships with outside scientific and clinical advisors, which could impair the progress of our clinical trials and our research and development efforts.**

We work with scientific and clinical advisors at academic and other institutions who are experts in the field of ocular disorders. They advise us with respect to our clinical trials. These advisors are not our employees and may have other commitments that would limit their future availability to us. If a conflict of interest arises between their work for us and their work for another entity, we may lose their services, which may impair our reputation in the industry and delay the development or commercialization of our product candidates.

**We rely completely on third-party manufacturers which may result in delays in our clinical trials, regulatory approvals and product introductions.**

We have no manufacturing facilities and do not have extensive experience in the manufacturing of drugs or in designing drug-manufacturing processes. We have contracted with third-party manufacturers to produce, in collaboration with us, our product candidates, including Squalamine, for clinical trials. If any of our product candidates are approved by the FDA or other regulatory agencies for commercial sale, we may need to amend our contract with our current manufacturer or contract with another third party to manufacture them in larger quantities. If we need to enter into alternative arrangements, it could delay our product development activities. Our reliance on these third parties for development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study and trial protocols. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study and trial plans and protocols, or if there are disagreements between us and these third parties, we will not be able to complete, or may be delayed in completing, the clinical trials required to support future approval of our product candidates. In some such cases we may need to locate an appropriate replacement third-party relationship, which may not be readily available or with acceptable terms, which would cause additional delay with respect to the approval of our product candidates and would thereby have a material adverse effect on our business, financial condition, results of operations and prospects.

We have not entered into long-term agreements with our current third-party manufacturers or with any alternate suppliers. Although we intend to do so prior to any commercial launch of a product that is approved by the FDA in order to ensure that we maintain adequate supplies of commercial drug product, we may be unable to enter into such agreements or do so on commercially reasonable terms, which could delay a product launch or subject our commercialization efforts to significant supply risk. In addition, reliance on third-party manufacturers entails risks to which we would not be subject to if we manufactured the product candidates ourselves, including:

- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- reduced control as a result of using third-party manufacturers for all aspects of manufacturing activities;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier.

Any of these events could lead to clinical trial delays or failure to obtain regulatory approval, or impact our ability to successfully commercialize future product candidates. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of product manufacture.

**Our contract manufacturers are subject to significant regulatory oversight with respect to manufacturing our products. The manufacturing facilities on which we rely may not continue to meet regulatory requirements and may have limited capacity.**

The manufacturers of our product candidates are obliged to operate in accordance with FDA-mandated cGMPs. In addition, the facilities used by our contract manufacturers or other third party manufacturers to manufacture our product candidates must be approved by the FDA pursuant to inspections that will be conducted after we request regulatory approval from the FDA. A failure of any of our current or future contract manufacturers to establish and follow cGMPs and to document their adherence to such practices may lead to significant delays in clinical trials or in obtaining regulatory approval of product candidates or the ultimate launch of products based on our product candidates into the market. Furthermore, all of our contract manufacturers are engaged with other companies to supply and/or manufacture materials or products for such companies, which exposes our manufacturers to regulatory risks for the production of such materials and products. As a result, failure to satisfy the regulatory requirements for the production of those materials and products may affect the regulatory clearance of our contract manufacturers' facilities generally. Failure by our current or future third-party manufacturers or us to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, failure of the government to grant pre-market approval of drugs, delays, suspension or withdrawal of approvals, seizures or recalls of products, operating restrictions, and criminal prosecutions. Many aspects of the clinical trial and manufacturing process are outside of our control. In addition, the third-party manufacturers may not perform all of their obligations under arrangements with us or in compliance with regulatory requirements. If a third-party manufacturer breaches their obligations to us or fails to comply with regulatory requirements, the commercialization of Squalamine in wet-AMD and other drug candidates may be delayed or irreversibly harmed.

The facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

The regulatory authorities also may, at any time following approval of a product for sale, audit our manufacturing facilities or those of our third-party manufacturers. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or our third-party manufacturers to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a manufacturing facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

**We are highly dependent upon our ability to enter into agreements with collaborative partners to develop, commercialize, and market our products.**

Our strategy is to seek a strategic commercial partner, or partners, such as large pharmaceutical companies, with extensive experience in the development, commercialization, and marketing of ophthalmic products. We anticipate such partner or partners would be responsible for, or substantially support, late stage clinical trials (Phase III) and sales and marketing of our products. Such planned strategic partnership, or partnerships, may provide a marketing and sales infrastructure for our products as well as financial and operational support for global clinical trials, post marketing studies, label expansions and other regulatory requirements concerning future clinical development in the United States.

While our strategy is to partner with an appropriate party, no assurance can be given that any third party would be interested in partnering with us. We currently lack the resources to manufacture any of our product candidates on a large scale and we have no sales, marketing or distribution capabilities. In the event we are not able to enter into a collaborative agreement with a partner or partners, on commercially reasonable terms, or at all, we may be unable to commercialize our products, which would have a material adverse effect upon our business, prospects, financial condition, and results of operations.

**If we are ever in a position to commercialize our product candidates, of which there can be no assurance, we have no experience selling, marketing or distributing products and no internal capability to do so.**

We currently have no sales, marketing or distribution capabilities and no experience in building a sales force and distribution capabilities. If we are ever in a position to commercialize our product candidates, of which there can be no assurance, we must develop internal sales, marketing and distribution capabilities, which will be expensive and time consuming, or make arrangements with third parties to perform these services. If we decide to market any of our products directly, we must commit significant financial and managerial resources to develop a marketing and sales force with technical expertise and with supporting distribution capabilities. Building an in-house marketing and sales force with technical expertise and distribution capabilities will require significant expenditures, management resources and time. Factors that may inhibit our efforts to commercialize our products directly and without strategic partners 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 sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating and sustaining an independent sales and marketing organization.

We may not be successful in recruiting the sales and marketing personnel necessary to sell our products and even if we do build a sales force, they may not be successful in marketing our products, which would have a material adverse effect on our business and results of operations.

**Developments by competitors may render our products or technologies obsolete or non-competitive which would have a material adverse effect on our business and results of operations.**

We compete against fully integrated pharmaceutical companies and smaller companies that are collaborating with larger pharmaceutical companies, academic institutions, government agencies and other public and private research organizations. Our drug candidates will have to compete with existing therapies and therapies under development by our competitors. In addition, our commercial opportunities may be reduced or eliminated if our competitors develop and market products that are less expensive, more effective or safer than our drug products. Other companies have drug candidates in various stages of preclinical or clinical development to treat diseases for which we are also seeking to develop drug products. Some of these potential competing drugs are further advanced in development than our drug candidates and may be commercialized earlier. Even if we are successful in developing effective drugs, our products may not compete successfully with products produced by our competitors. Most of our competitors, either alone or together with their collaborative partners, operate larger research and development programs, have larger staffing and facilities, and have substantially greater financial resources than we do, as well as significantly greater experience in:

- developing drugs;
- undertaking preclinical testing and human clinical trials;
- obtaining FDA and other regulatory approvals of drugs;
- formulating and manufacturing drugs; and
- launching, marketing and selling drugs.

These organizations also compete with us to attract qualified personnel, acquisitions and joint ventures candidates and for other collaborations. Activities of our competitors may impose unanticipated costs on our business which would have a material adverse effect on our business and results of operations.

**We depend upon key officers and consultants in a competitive market for skilled personnel. If we are unable to attract and retain key personnel, it could adversely affect our ability to develop and market our products.**

We are highly dependent upon the principal members of our management team, especially our Chief Executive Officer, Dr. Jason Slakter, and Vice President of Business Development and Chief Financial Officer, Sam Backenroth, as well as our directors and key consultants. A loss of any of these personnel may have a material adverse effect on aspects of our business and clinical development and regulatory programs.

We also depend in part on obtaining the service of scientific personnel and our ability to identify, hire and retain additional personnel. We experience intense competition for qualified personnel, and the existence of non-competition agreements between prospective employees and their former employers may prevent us from hiring those individuals or subject us to suit from their former employers. While we attempt to provide competitive compensation packages to attract and retain key personnel, many of our competitors are likely to have greater resources and more experience than we have, making it difficult for us to compete successfully for key personnel.

**Any future acquisitions we make of companies or technologies may result in disruption to our business or distraction of our management, due to difficulties in assimilating acquired personnel and operations.**

We may acquire or make investments in complementary businesses, technologies, services or products which complement our biotech operations if appropriate opportunities arise. From time to time we engage in discussions and negotiations with companies regarding our acquiring or investing in such companies' businesses, products, services or technologies, in the ordinary course of our business. We cannot be assured that we will be able to identify future suitable acquisition or investment candidates, or if we do identify suitable candidates, that we will be able to make such acquisitions or investments on commercially acceptable terms or at all. If we acquire or invest in another company, we could have difficulty in assimilating that company's personnel, operations, technology and software. In addition, the key personnel of the acquired company may decide not to work for us. If we make other types of acquisitions, we could have difficulty in integrating the acquired products, services or technologies into our operations. These difficulties could disrupt our ongoing business, distract our management and employees, increase our expenses and adversely affect our results of operations. Furthermore, we may incur indebtedness or issue equity securities to pay for any future acquisitions. The issuance of equity securities would be dilutive to our existing stockholders. However, we currently do not have any agreement to enter into any material investment or acquisition transaction.

**We may be unsuccessful in monetizing existing assets, acquiring additional assets or entering into joint development programs.**

We will continue to seek to acquire or make investments in complementary businesses, technologies, services or products and plan to seek development partners for our existing products. Several third parties with whom we have been in discussions have expressed interest in a potential licensing or partnering transaction for the Squalamine program. We continue to make progress in these discussions; however, we currently do not have any agreement to enter into such a transaction, and there is no assurance that the Company will enter into or complete such a transaction.

**Security breaches and other disruptions could compromise our information and expose us to liability, which would cause our business and reputation to suffer.**

We store sensitive data, including intellectual property, our proprietary business information and personally identifiable information of our employees, in our data centers and on our networks. The secure maintenance of this information is critical to our operations. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. Any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, regulatory penalties, and damage our reputation.

### **Risks Related to FDA Regulation**

**We face heavy government regulation, and FDA regulatory approval of our products is uncertain.**

The research, testing, manufacturing and marketing of drug products such as those that we are developing are subject to extensive regulation by federal, state and local government authorities, including the FDA. To obtain regulatory approval of a product, we must demonstrate to the satisfaction of the applicable regulatory agency that, among other things, the product is safe and effective for its intended use. In addition, we must show that the manufacturing facilities used to produce the products are in compliance with current Good Manufacturing Practices regulations (cGMP).

The process of obtaining FDA and other required regulatory approvals and clearances will require us to expend substantial time and capital. Despite the time and expense expended, regulatory approval is never guaranteed. The number of preclinical and clinical trials that will be required for FDA approval varies depending on the drug candidate, the disease or condition for which the drug candidate is in development, and the requirements applicable to that particular drug candidate. The FDA can delay, limit or deny approval of a drug candidate for many reasons, including that:

- a drug candidate may not be shown to be safe or effective;
- the FDA may not approve our manufacturing process;
- the FDA may interpret data from preclinical and clinical trials in different ways than we do; and
- the FDA may not meet, or may extend, the Prescription Drug User Fee Act date with respect to a particular New Drug Application (“NDA”).

For example, if certain of our methods for analyzing our trial data are not accepted by the FDA, we may fail to obtain regulatory approval for our product candidates. Moreover, if and when our products do obtain marketing approval, the marketing, distribution and manufacture of such products would remain subject to extensive ongoing regulatory requirements. Failure to comply with applicable regulatory requirements could result in:

- warning letters;
- fines;
- civil penalties;
- injunctions;
- recall or seizure of products;
- total or partial suspension of production;
- refusal of the government to grant future approvals;
- withdrawal of approvals; and
- criminal prosecution.

Any delay or failure by us to obtain regulatory approvals for our product candidates could diminish competitive advantages that we may attain and would adversely affect the marketing of our products. We have not received regulatory approval to market any of our product candidates in any jurisdiction.

**Following regulatory approval of any of our drug candidates, we will be subject to ongoing regulatory obligations and restrictions, which may result in significant expense and limit our ability to commercialize our potential products.**

With regard to our drug candidates, if any, approved by the FDA or by another regulatory authority, we are held to extensive regulatory requirements over product manufacturing, labeling, packaging, adverse event reporting, storage, advertising, promotion and record keeping. Regulatory approvals may also be subject to significant limitations on the indicated uses or marketing of the drug candidates. Potentially costly follow-up or post-marketing clinical studies may be required as a condition of approval to further substantiate safety or efficacy, or to investigate specific issues of interest to the regulatory authority. Previously unknown problems with the drug candidate, including adverse events of unanticipated severity or frequency, may result in restrictions on the marketing of the drug, and could include withdrawal of the drug from the market.

In addition, the law or regulatory policies governing pharmaceuticals may change. New statutory requirements may be enacted or additional regulations may be enacted that could prevent or delay regulatory approval of our drug candidates. We cannot predict the likelihood, nature or extent of adverse government regulation that may arise from future legislation or administrative action, either in the United States or elsewhere. If we are not able to maintain regulatory compliance, we might not be permitted to market our drugs and our business could suffer.

**Healthcare policy changes, including pending legislation recently adopted and further proposals still pending to reform the U.S. healthcare system, may harm our future business.**

Healthcare costs have risen significantly over the past decade. There have been and continue to be proposals by legislators, regulators and third-party payors to keep these costs down. Certain proposals, if passed, would impose limitations on the prices we will be able to charge for the products that we are developing, or the amounts of reimbursement available for these products from governmental agencies or third-party payors. These limitations could in turn reduce the amount of revenues that we will be able to generate in the future from sales of our products and licenses of our technology.

In March 2010, the U.S. Congress enacted and President Obama signed into law healthcare reform legislation that may significantly impact the pharmaceutical industry. In addition to requiring most individuals to have health insurance and establishing new regulations on health plans, this legislation will require discounts under the Medicare drug benefit program and increased rebates on drugs covered by Medicaid. In addition, the legislation imposes an annual fee, which will increase annually, on sales by branded pharmaceutical manufacturers starting in 2011. The financial impact of these discounts, increased rebates and fees and the other provisions of the legislation on our business is unclear and there can be no assurance that our business will not be materially adversely affected. In addition, these and other ongoing initiatives in the United States have increased and will continue to increase pressure on drug pricing. The announcement or adoption of any such initiative could have an adverse effect on potential revenues from any product that we may successfully develop.

Various healthcare reform proposals have also emerged at the state level. We cannot predict what healthcare initiatives, if any, will be implemented at the federal or state level, or the effect any future legislation or regulation will have on us. However, an expansion in government's role in the U.S. healthcare industry may lower the future revenues for the products we are developing and adversely affect our future business, possibly materially.

### **Risks Related to Our Intellectual Property**

#### **Our ability to compete may be undermined if we do not adequately protect our proprietary rights.**

Our commercial success depends on obtaining and maintaining proprietary rights to our product candidates and technologies and their uses, as well as successfully defending these rights against third-party challenges. We will be able to most effectively protect our product candidates, technologies, and their uses from unauthorized use by third parties to the extent that valid and enforceable patents, or effectively protected trade secrets, cover them. For example, we have rights under patents and patent applications US 7981876, 8716270, 6262283, 7728157, 6962909, and 20130281420 to cover the Squalamine formulations, composition of matter, methods of manufacture and synthesis and uses. Nonetheless, the issued patents and patent applications covering our primary technology programs remain subject to uncertainty due to a number of factors, including:

- we may not have been the first to make one or more of the inventions covered by our pending patent applications or issued patents;
- we may not have been the first to file patent applications for one or more of our product candidates or the technologies we rely upon;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- our disclosures in a particular patent application may be determined to be insufficient to meet the statutory requirements for patentability;
- one or more of our pending patent applications may not result in issued patents;
- we may not seek or obtain patent protection in all countries that will eventually provide a significant business opportunity;
- one or more patents issued to us or to our collaborators may not provide a basis for commercially viable products, may not provide us with any competitive advantages or may be challenged by third parties;
- we may fail to file for patent protection in all of the countries where patent protection will ultimately be necessary or fail to comply with other procedural, documentary, fee payment or other provisions during the patent process in any such country, and we may be precluded from filing at a later date or may lose some or all patent rights in the relevant jurisdiction;
- one or more of our technologies may not be patentable;
- others may design around one or more of our patent claims to produce competitive products which fall outside of the scope of our patents;
- others may identify prior art which could invalidate our patents; or
- changes to patent laws may limit the exclusivity rights of patent holders.

Even if we have or obtain patents covering our product candidates or technologies, we may still be barred from making, using and selling one or more of our product candidates or technologies because of the patent rights of others. Others have or may have filed, and in the future are likely to file, patent applications covering compounds, assays, therapeutic products and delivery systems, including sustained release delivery, that are similar or identical to ours. Numerous U.S. and foreign issued patents and pending patent applications owned by others exist in the area of ocular disorders. These could materially affect our ability to develop our product candidates or sell our products. Because patent applications can take years to issue, there may be currently pending applications, unknown to us, that may later result in issued patents that our product candidates or technologies may infringe. These patent applications may have priority over one or more patent applications filed by us.

If our competitors prepare and file patent applications in the United States that claim technology we also claim, we may have to participate in interference proceedings required by the United States Patent and Trademark Office to determine priority of invention, which could result in substantial costs, even if we ultimately prevail. Results of interference proceedings are highly unpredictable and may result in us having to try to obtain licenses in order to continue to develop or market certain of our drug products.

**Disputes may arise regarding the ownership or inventorship of our inventions. It is difficult to determine how such disputes would be resolved. Others may challenge the validity of our patents. If one or more of our patents are found to be invalid, we will lose the ability to exclude others from making, using or selling the inventions claimed therein.**

Some of our research collaborators and scientific advisors have rights to publish data and information to which we have rights. Additionally, employees whose positions may be eliminated in connection with restructurings may seek future employment with our competitors. Although each of our employees is required to sign a confidentiality agreement with us at the time of hire, we cannot guarantee that the confidential nature of our proprietary information will be maintained in the course of such future employment. In addition, technology that we may license in may become important to some aspects of our business. We generally will not control all of the patent prosecution, maintenance or enforcement of in-licensed technology.

**We rely on confidentiality agreements that could be breached and may be difficult to enforce which could have a material adverse effect on our business and competitive position.**

Our policy is to enter agreements relating to the non-disclosure of confidential information with third parties, including our contractors, consultants, advisors and research collaborators, as well as agreements that purport to require the disclosure and assignment to us of the rights to the ideas, developments, discoveries and inventions of our employees and consultants while we employ them. However, these agreements can be difficult and costly to enforce. Moreover, to the extent that our contractors, consultants, advisors and research collaborators apply or independently develop intellectual property in connection with any of our projects, disputes may arise as to the proprietary rights to this type of information. If a dispute arises, a court may determine that the right belongs to a third party, and enforcement of our rights can be costly and unpredictable. In addition, we rely on trade secrets and proprietary know-how that we will seek to protect in part by confidentiality agreements with our employees, contractors, consultants, advisors or others. In addition, courts outside the United States may be less willing to protect trade secrets. Despite the protective measures we employ, we still face the risk that:

- these agreements may be breached;
- these agreements may not provide adequate remedies for the applicable type of breach; or
- our trade secrets or proprietary know-how will otherwise become known.

Any breach of our confidentiality agreements or our failure to effectively enforce such agreements would have a material adverse effect on our business and competitive position.

**If we infringe the rights of third parties we could be prevented from selling products, forced to pay damages and required to defend against litigation which could result in substantial costs and may have a material adverse effect on our business and results of operations.**

We have not received to date any claims of infringement by any third parties. However, as our product candidates progress into clinical trials and commercialization, if at all, our public profile and that of our product candidates may be raised and generate such claims. Defending against such claims, and occurrence of a judgment adverse to us, could result in unanticipated costs and may have a material adverse effect on our business and competitive position. If our products, methods, processes and other technologies infringe the proprietary rights of other parties, we could incur substantial costs and we may have to:

- obtain licenses, which may not be available on commercially reasonable terms, if at all;
- redesign our products or processes to avoid infringement;
- stop using the subject matter claimed in the patents held by others, which could cause us to lose the use of one or more of our drug candidates;
- defend litigation or administrative proceedings that may be costly whether we win or lose, and which could result in a substantial diversion of management resources; or
- pay damages.

Any costs incurred in connection with such events or the inability to sell our products may have a material adverse effect on our business and results of operations.

**Intellectual property litigation is increasingly common and increasingly expensive and may result in restrictions on our business and substantial costs, even if we prevail.**

Patent and other intellectual property litigation is becoming more common in the pharmaceutical industry. Litigation is sometimes necessary to defend against or assert claims of infringement, to enforce our patent rights, including those we have licensed from others, to protect trade secrets or to determine the scope and validity of proprietary rights of third parties. Currently, no third party is asserting that we are infringing upon their patent rights or other intellectual property, nor are we aware or believe that we are infringing upon any third party's patent rights or other intellectual property. We may, however, be infringing upon a third party's patent rights or other intellectual property, and litigation asserting such claims might be initiated in which we would not prevail, or we would not be able to obtain the necessary licenses on reasonable terms, if at all. All such litigation, whether meritorious or not, as well as litigation initiated by us against third parties, is time-consuming and very expensive to defend or prosecute and to resolve. In addition, if we infringe the intellectual property rights of others, we could lose our right to develop, manufacture or sell our products or could be required to pay monetary damages or royalties to license proprietary rights from third parties. An adverse determination in a judicial or administrative proceeding or a failure to obtain necessary licenses could prevent us from manufacturing or selling our products, which could harm our business, financial condition and prospects.

**A dispute concerning the infringement or misappropriation of our proprietary rights or the proprietary rights of others could be time consuming and costly, and an unfavorable outcome could harm our business.**

There is significant litigation in our industry regarding patent and other intellectual property rights. While we are not currently subject to any pending intellectual property litigation, and are not aware of any such threatened litigation, we may be exposed to future litigation by third parties based on claims that our product candidates, technologies or activities infringe the intellectual property rights of others. If our drug development activities are found to infringe any such patents, we may have to pay significant damages or seek licenses to such patents. If our products are found to infringe any such patents, we may have to pay significant damages or seek licenses to such patents. A patentee could prevent us from making, using or selling the patented compounds. We may need to resort to litigation to enforce a patent issued to us, protect our trade secrets or determine the scope and validity of third-party proprietary rights. From time to time, we may hire scientific personnel formerly employed by other companies involved in one or more areas similar to the activities conducted by us. Either we or these individuals may be subject to allegations of trade secret misappropriation or other similar claims as a result of their prior affiliations. If we become involved in litigation, it could consume a substantial portion of our managerial and financial resources, regardless of whether we win or lose. We also may not be able to afford the costs of litigation.

**The patent applications of pharmaceutical and biotechnology companies involve highly complex legal and factual questions, which, if determined adversely to us, could negatively impact our patent position.**

The patent positions of pharmaceutical and biotechnology companies can be highly uncertain and involve complex legal and factual questions. No consistent policy regarding the breadth of claims allowed in biotechnology patents has emerged to date. The U.S. Patent and Trademark Office's standards are uncertain and could change in the future. Consequently, the issuance and scope of patents cannot be predicted with certainty. Patents, if issued, may be challenged, invalidated or circumvented. U.S. patents and patent applications may also be subject to interference or derivation proceedings, and U.S. patents may be subject to ex parte review and reexamination proceedings in the U.S. Patent and Trademark Office (and foreign patents may be subject to opposition or comparable proceedings in the corresponding foreign patent office), which proceedings could result in either loss of the patent or denial of the patent application or loss or reduction in the scope of one or more of the claims of the patent or patent application. Similarly, opposition or invalidity proceedings could result in loss of rights or reduction in the scope of one or more claims of a patent in foreign jurisdictions. Such interference, ex parte review, reexamination and opposition proceedings may be costly. Accordingly, rights under any issued patents may not provide us with sufficient protection against competitive products or processes.

Changes in or different interpretations of patent laws in the United States and foreign countries may permit others to use our discoveries or to develop and commercialize our technology and products without providing any compensation to us or may limit the number of patents or claims we can obtain. In particular, there have been proposals to shorten the exclusivity periods available under U.S. patent law that, if adopted, could substantially harm our business. The product candidates that we are developing are protected by intellectual property rights, including patents and patent applications. If any of our product candidates becomes a marketable product, we will rely on our exclusivity under patents to sell the compound and recoup our investments in the research and development of the compound. If the exclusivity period for patents is shortened, then our ability to generate revenues without competition will be reduced and our business could be materially adversely impacted. The laws of some countries do not protect intellectual property rights to the same extent as U.S. laws, and those countries may lack adequate rules and procedures for defending our intellectual property rights. For example, some countries, including many in Europe, do not grant patent claims directed to methods of treating humans and, in these countries, patent protection may not be available at all to protect our product candidates. In addition, U.S. patent laws may change, which could prevent or limit us from filing patent applications or patent claims to protect our products or technologies or limit the exclusivity periods that are available to patent holders. For example, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was recently signed into law and includes a number of significant changes to U.S. patent law. These include changes to transition from a "first-to-invent" system to a "first-to-file" system and to the way issued patents are challenged. These changes may favor larger and more established companies that have more resources to devote to patent application filing and prosecution. The U.S. Patent and Trademark Office has been in the process of implementing regulations and procedures to administer the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act may affect our ability to obtain, enforce or defend our patents. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will ultimately have on the cost of prosecuting our patent applications, our ability to obtain patents based on our discoveries and our ability to enforce or defend our issued patents.



If we fail to obtain and maintain patent protection and trade secret protection of our product candidates, proprietary technologies and their uses, we could lose our competitive advantage and competition we face would increase, reducing our potential revenues and adversely affecting our ability to attain or maintain profitability.

### **Risks Related to our Common Stock**

**The market price and volume of our common stock fluctuate significantly and could result in substantial losses for individual investors.**

The stock market from time to time experiences significant price and volume fluctuations that are unrelated to the operating performance of particular companies. These broad market fluctuations may cause the market price and volume of our common stock to decrease. In addition, the market price and volume of our common stock is highly volatile.

Factors that may cause the market price and volume of our common stock to decrease include:

- adverse results or delays in our clinical trials;
- fluctuations in our results of operations, timing and announcements of our bio-technological innovations or new products or those of our competitors;
- developments concerning any strategic alliances or acquisitions we may enter into;
- announcements of FDA non-approval of our drug products, or delays in the FDA or other foreign regulatory review process or actions;
- adverse actions taken by regulatory agencies with respect to our drug products, clinical trials, manufacturing processes or sales and marketing activities;
- any lawsuit involving us or our drug products;
- developments with respect to our patents and proprietary rights;
- announcements of technological innovations or new products by our competitors;
- public concern as to the safety of products developed by us or others;
- regulatory developments in the United States and in foreign countries;
- changes in stock market analyst recommendations regarding our common stock or lack of analyst coverage;
- the pharmaceutical industry conditions generally and general market conditions;
- failure of our results of operations to meet the expectations of stock market analysts and investors;
- sales of our common stock by our executive officers, directors and five percent stockholders or sales of substantial amounts of our common stock;
- changes in accounting principles; and
- loss of any of our key scientific or management personnel.

**The market for our common stock is illiquid. Our stockholders may not be able to resell their shares at or above the purchase price paid by such stockholders, or at all.**

Our common stock is quoted on the NASDAQ Capital Market. The market for our securities is illiquid. This illiquidity may be caused by a variety of factors including:

- lower trading volume; and
- market conditions.

There is limited trading in our common stock and our security holders may experience wide fluctuations in the market price of our securities. Such price and volume fluctuations have particularly affected the trading prices of equity securities of many pharmaceutical and biotechnology companies. These price and volume fluctuations often have been unrelated to the operating performance of the affected companies. These fluctuations may have an extremely negative effect on the market price of our securities and may prevent a stockholder from obtaining a market price equal to the purchase price such stockholder paid when the stockholder attempts to sell our securities in the open market. In these situations, the stockholder may be required either to sell our securities at a market price which is lower than the purchase price the stockholder paid, or to hold our securities for a longer period of time than planned. An inactive market may also impair our ability to raise capital by selling shares of capital stock or to recruit and retain managers with equity-based incentive plans.

**If we do not raise additional funds, we will not be able to continue operations or complete the necessary clinical and preclinical trials to complete development of Squalamine and our sustained release ophthalmological platform or our other products and will not be able to sell them anywhere.**

We will not be able to sell Squalamine and our sustained release ophthalmological platform or our other products in the United States unless we submit, and the FDA approves, an NDA for each such product. We must conduct clinical trials of each of our products in humans before we submit an NDA. We currently do not have sufficient capital to complete the necessary trials to complete the development of Squalamine and our sustained release ophthalmological platform or any of our other therapeutic drug products.

It is possible that the results of clinical and preclinical studies of Squalamine and our sustained release ophthalmological platform or our other products will not prove that they are safe and effective. It is also possible that the FDA will not approve the sale of any of our products in the United States if we submit an NDA for such product. Even if the data show that any of our products are safe and effective, obtaining approval of the NDA could take years and require financing of amounts not presently available to us.

Conducting the clinical and preclinical studies of each of our products will require significant cash expenditures and we do not have the funds necessary to complete all phases of clinical trials for Squalamine and our sustained release ophthalmological platform or any other products. Our products may never be approved for commercial distribution by any country. Because our research and development expenses and clinical and preclinical study expenses will be charged against earnings for financial reporting purposes, we expect that losses from operations will continue to be incurred for the near future. We currently do not have sufficient funds to complete all phases of clinical and preclinical testing of any of our products which are required to permit the commercial sale of such products.

**We will not pay cash dividends and investors may have to sell their shares in order to realize their investment.**

We have not paid any cash dividends on our common stock and do not intend to pay cash dividends in the foreseeable future. We intend to use our cash for reinvestment in the development and marketing of our products and services. As a result, investors may have to sell their shares of common stock to realize their investment.

**Our internal controls over financial reporting may not be effective, and our independent auditors may not be able to certify as to their effectiveness, which could have a significant and adverse effect on our business and reputation.**

We are subject to the requirements of Section 404 of the Sarbanes-Oxley Act of 2002 and the rules and regulations of the SEC thereunder ("Section 404"). Section 404 requires us to report on the design and effectiveness of our internal controls over financial reporting. In the past, our management has identified certain "material weaknesses" in our internal controls over financial reporting which we believe have been remediated. However, any failure to maintain effective controls could result in significant deficiencies or material weaknesses, and cause us to fail to meet our periodic reporting obligations, or result in material misstatements in our financial statements. We may also be required to incur costs to improve our internal control system and hire additional personnel. This could negatively impact our results of operations.

Section 404 also requires an independent registered public accounting firm to test our internal controls over financial reporting and report on the effectiveness of such controls. For future reporting periods, there can be no assurance that our auditors will issue an unqualified report attesting to our internal controls over financial reporting at that time. As a result, there could be a negative reaction in the financial markets due to a loss of confidence in the reliability of our financial statements or our financial statements could change.

**Compliance with changing regulation of corporate governance and public disclosure may result in additional expenses and divert management's attention from operating our business, which could have a material adverse effect on our business.**

There have been other changing laws, regulations and standards relating to corporate governance and public disclosure in addition to the Sarbanes-Oxley Act, as well as new regulations promulgated by the Commission and rules promulgated by the national securities exchanges. These new or changed laws, regulations and standards are subject to varying interpretations in many cases due to their lack of specificity, and as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies, which could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices. As a result, our efforts to comply with evolving laws, regulations and standards are likely to continue to result in increased general and administrative expenses and a diversion of management time and attention from revenue-generating activities to compliance activities. Our board members, Chief Executive Officer and Chief Financial Officer could face an increased risk of personal liability in connection with the performance of their duties. As a result, we may have difficulty attracting and retaining qualified board members and executive officers, which could have a material adverse effect on our business. If our efforts to comply with new or changed laws, regulations and standards differ from the activities intended by regulatory or governing bodies, we may incur additional expenses to comply with standards set by regulatory authorities or governing bodies which would have a material adverse effect on our business and results of operations.

**Delaware law could discourage a change in control, or an acquisition of the Company by a third party, even if the acquisition would be favorable to stockholders.**

The Delaware General Corporation Law contains provisions that may have the effect of making more difficult or delaying attempts by others to obtain control of the Company, even when these attempts may be in the best interests of stockholders. Delaware law imposes conditions on certain business combination transactions with "interested stockholders." These provisions and others that could be adopted in the future could deter unsolicited takeovers or delay or prevent changes in our control or management, including transactions in which stockholders might otherwise receive a premium for their shares of common stock over then current market prices. These provisions may also limit the ability of stockholders to approve transactions that they may deem to be in their best interests.

**Our Board of Directors has the authority to issue Serial Preferred Stock, which could affect the rights of holders of our common stock and may delay or prevent a takeover that could be in the best interests of our stockholders.**

The Board of Directors has the authority to issue up to 9,416,664 shares of Serial Preferred Stock, \$.0001 par value per share (the "Serial Preferred Stock") (after giving effect to the conversion and cancellation of a previous issue of 5,583,336 shares of Series B Preferred), in one or more series and to fix the number of shares constituting any such series, the voting powers, designation, preferences and relative participation, optional or other special rights and qualifications, limitations or restrictions thereof, including the dividend rights and dividend rate, terms of redemption (including sinking fund provisions), redemption price or prices, conversion rights and liquidation preferences of the shares constituting any series, without any further vote or action by the stockholders. 6,000,000 shares of the Serial Preferred Stock, designated the Series B Preferred, have been authorized, 5,583,336 were issued and, as of the date of this filing, all such shares have been converted and no Series B Preferred shares remain issued and outstanding. The issuance of additional Serial Preferred Stock could affect the rights of the holders of Common Stock. For example, such issuance could result in a class of securities outstanding that would have preferential voting, dividend, and liquidation rights over the Common Stock, and could (upon conversion or otherwise) enjoy all of the rights appurtenant to the shares of Common Stock. The authority possessed by the Board of Directors to issue Serial Preferred Stock could potentially be used to discourage attempts by others to obtain control of the Company through merger, tender offer, proxy contest or otherwise by making such attempts more difficult or costly to achieve. The Board of Directors may issue the Serial Preferred Stock without stockholder approval and with voting and conversion rights which could adversely affect the voting power of holders of Common Stock. There are no agreements or understandings for the issuance of Serial Preferred Stock and the Board of Directors has no present intention to issue any Serial Preferred Stock.

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

On January 7, 2016, the Company issued options to purchase 240,000 shares of common stock to directors of the Company. One third of the options vested immediately, and one third of the options will vest on each of January 7, 2017 and January 7, 2018. The options have an exercise price of \$5.14 and term of five years.

On January 14, 2016, the Company issued options to purchase 149,275 shares of common stock to employees of the Company. One third of the options vested immediately, and one third of the options will vest on each of January 14, 2017 and January 14, 2018. The options have an exercise price of \$3.85 and term of five years.

On January 22, 2016, the Company issued 480,358 shares of restricted stock to employees of the Company. One half of the shares will vest on each of January 7, 2017, and January 7, 2018, subject to accelerated vesting under certain conditions.

On January 22, 2016, the Company issued options to purchase 80,000 shares of common stock to a director. One third of the options vested immediately, and one third of the options will vest on each of January 22, 2017 and January 22, 2018. The options have an exercise price of \$3.62 and term of five years.

On January 22, 2016 and March 17, 2016 the Board approved the issuance of 25,399 and 35,000 shares, respectively, of the Company's restricted stock to a consultant of the Company for services rendered.

The issuances of the shares were made in reliance on the exemption from registration provided under Section 4(2) of the Securities Act of 1933, as amended.

**Item 3. Defaults Upon Senior Securities.**

None.

**Item 4. Mine Safety Disclosures.**

Not applicable

**Item 5. Other Information**

None

**Item 6. Exhibits**

<u>Exhibit</u>	<u>Description</u>
31.1	<a href="#"><u>Certification of Chief Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002</u></a>
31.2	<a href="#"><u>Certification of Chief Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002</u></a>
32.1	<a href="#"><u>Certification of Chief Executive Officer Pursuant to 18 U.S.C Section 1350, As Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002</u></a>
32.2	<a href="#"><u>Certification of Chief Financial Officer Pursuant to 18 U.S.C Section 1350, As Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002</u></a>
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

**SIGNATURES**

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

Date: May 10, 2016

**OHR PHARMACEUTICAL, INC.**  
(Registrant)

By: /s/ Jason S. Slakter

Jason S. Slakter

Chief Executive Officer (Principal Executive Officer)

By: /s/ Sam Backenroth

Sam Backenroth

Chief Financial Officer (Principal Financial and Chief Accounting Officer)

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Certification of Principal Executive Officer  
Pursuant to Section 302 of the  
Sarbanes-Oxley Act of 2002

I, Jason Slakter, certify that:

1. I have reviewed this report on Form 10-Q of Ohr Pharmaceutical, Inc;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:
  - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: May 10, 2016

/s/ Jason Slakter

Jason Slakter

Chief Executive Officer (Principal Executive Officer)

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Certification of Principal Financial Officer  
Pursuant to Section 302 of the  
Sarbanes-Oxley Act of 2002

I, Sam Backenroth, certify that:

1. I have reviewed this report on Form 10-Q of Ohr Pharmaceutical, Inc
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrants other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:
  - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant 's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant 's ability to record, process, summarize and report financial information; and
  - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: May 10, 2016

/s/ Sam Backenroth

Sam Backenroth

Chief Financial Officer (Principal Financial Officer)

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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

Not Filed Pursuant to the Securities Exchange Act of 1934

In connection with the Quarterly Report of Ohr Pharmaceutical, Inc. (the "*Company*") on Form 10-Q for the period ending March 31, 2016 as filed with the Securities and Exchange Commission on the date hereof (the "*Report*"), I, Jason Slakter, Chief Executive Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: May 10, 2016

/s/ Jason Slakter

Name: Jason Slakter

Title: Chief Executive Officer (Principal Executive Officer)

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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

Not Filed Pursuant to the Securities Exchange Act of 1934

In connection with the Quarterly Report of Ohr Pharmaceutical, Inc. (the "*Company*") on Form 10-Q for the period ending March 31, 2016, as filed with the Securities and Exchange Commission on the date hereof (the "*Report*"), I, Sam Backenroth, Chief Financial Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: May 10, 2016

/s/ Sam Backenroth

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Name: Sam Backenroth

Title: Chief Financial Officer (Principal Financial Officer)

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